PARLIAMENT OF THE COMMONWEALTH OF AUSTRALIA

The New Frontier - Delivering better health for all Australians

Inquiry into approval processes for new drugs and novel medical technologies in Australia

House of Representatives Standing Committee on Health, Aged Care and Sport

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CANBERRA

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Chair's Foreword

Over the last 18 months many Australians have observed with awe and admiration the incredible work of medical scientists in finding vaccines and new treatments to a virus that has taken the lives or impacted the health of millions around the world.

Many of the innovations and medical understandings developed during the COVID-19 pandemic will have long-term benefits for health treatments for other conditions beyond COVID-19.

These innovations reflect the new frontier of medicine which is giving many hope for better treatments and technologies for conditions ranging from cancers to rare diseases. At its forefront is the development of personalised or precision medicine which is being delivered as our understanding of fields like genomics grows.

This report examines the opportunities to deliver better health care for Australians through our regulatory and health technology assessment process for both medicines and technologies.

At its heart are the needs of patients - Australians who are born with or who acquire conditions, many of which have so far eluded highly effective treatments. Everything in this report is about providing better options and hope for Australians with medical conditions.

Australia has long prided itself on having one of the world's best health systems. By any measure we do. Our success in protecting Australians during a global pandemic is the latest evidence of both the strengths of our health care system and the quality and dedication of all those who work in health care.

However, no nation and no health system can rest on its laurels. With innovation happening at a fast pace, governments at both the state and federal level have a duty to ensure that Australians continue to have access quickly to medicines and medical technology and that our health systems facilitate that outcome rather than

hinder it. Australians can also benefit by being at the forefront of innovation through clinical trials and a strong domestic research, development and manufacturing capacity.

Medical innovation has grown exponentially in recent years and pharmaceutical and Medtech companies are eager to bring new medicines and devices to market as efficiently as possible. The Committee also heard from clinical experts and patient groups and their families who urged us to support a more flexible system to provide for timely access to the latest medicines, devices and treatments.

One of the challenges facing the existing system is the trend towards delivering precision medicine to patients. Precision medicine is an emerging approach for disease treatment and prevention that takes into account individual variabilities in genes, environment and lifestyle for each person. This offers great hope for patients from a broad spectrum of conditions and diseases, including patients with rare diseases. However, these developments were not envisaged when the current regulatory and reimbursement system was designed and legislated.

The Committee recommends the creation of a Centre for Precision Medicine and Rare Disease within the Department of Health, to provide advice on research priorities, education and training for clinicians and patients, and the development of a comprehensive horizon scanning unit for new medicines and novel medical technologies. The Committee also recommends that a new pathway for cell and gene therapy be established to simplify the Health Technology Assessment (HTA) processes.

The Committee heard from patients and their families about the need for more patient involvement in the approvals decision-making process for new drugs and novel medical technologies. Patients have a crucial perspective on what treatments work best for them, including important lifestyle benefits, but this has traditionally not been given enough attention within the regulatory and reimbursement system. The Committee recommends reforms that will strengthen the central role of patients in the assessment system.

Many submitters to the inquiry suggested that there is little measurement and publication of how well the regulatory and reimbursement system is performing. The Committee believes this should be more transparent and recommends the Department of Health annually publish data on HTA processing times and benchmark these against other nations with advanced HTA processes.

The Committee heard from patients and clinicians who were frustrated that some medicines and technologies are available overseas and not in Australia, with companies seemingly deciding not to sell their products in Australia for commercial reasons. This is a particular issue that arises for orphan drugs and

drugs for rare diseases. The Committee recommends changes to encourage companies to enter the Australian market with their products and technologies. This includes changes to the fee structure for applications to the TGA and HTA processes – particularly for orphan drugs and smaller companies, including Australian start-ups.

The Committee also recommends the creation of an annually capped fund with clear and transparent eligibility rules to provide funding for applications by patients, clinicians and non-profits, where there is no realistic prospect of a company serving as a sponsor.

The approval processes for new medicines and novel medical technologies are very complex, and this report discusses different ways to streamline them to provide better and faster patient access to treatments. While it is often difficult to achieve this without compromising on patient safety, efficacy or cost effectiveness, the Committee believes there are areas where major changes are necessary and possible. One example of this is the Life Saving Drugs Program (LSDP) for treatments for very rare diseases, which despite the urgent patient need, currently requires a lengthy two-step application process. The Committee recommends that this process be streamlined into a one step process to establish a new pathway to the LSDP Expert Panel or to establish an alternate pathway by adjusting the Pharmaceutical Benefits Scheme section 100 program.

Another cause of complexity in the approvals system for medicines and medical technologies is the interaction between the Commonwealth and the states and territories. The Committee found that there are several areas where the Australian Government can work better with the states and territories. An important example of this is newborn screening, which has the potential to ensure early intervention and more accurate diagnosis. The Committee recommends that the Australian Government lead efforts to complete the standardisation of this screening across the country, based on new understandings of genomic testing, and to review the newborn screening program every two years to keep pace with new medical developments.

Clinical trials are another area where Australia has considerable strong comparative advantages. Ensuring Australia remains a top-tier country for trials not only develops our own research capacity but, more importantly, can ensure early access to life changing drugs and technologies.

The Committee has recommended changes to streamline the system and ensure Australia is an even more attractive location for clinical trials. These include the immediate harmonisation of ethics and governance approvals into one online platform and the establishment of a national clinical trials register.

The research and development (R&D) of new medicines and medical technologies attracted considerable attention during this inquiry, and the Committee makes a number of recommendations to support stronger and more collaborative R&D. Patient groups advocated strongly for the repurposing of existing medicines to treat alternate disease or conditions. The Committee recognises this is an area that requires a more flexible vision for the future and recommends the establishment of a new pathway that incentivises the repurposing of drugs for all diseases.

This report is being delivered in an ever-changing environment. The Australian Government is reviewing the National Medicines Policy (NMP) and a further major review of HTA processes has been announced. It is our hope that many of the recommendations in this report can be implemented in the short-term and not await the outcome of these further reviews. We have also identified medium term issues that should be central to the HTA review.

It was clear to the Committee that there was a great deal of momentum behind the push to improve the regulatory and reimbursement system — not just a general desire for change, but a wealth of ideas for reform and a willingness to make the efforts and compromises necessary to implement them. The Committee hopes that this report captures those ideas, and paves the way for the improvements needed to provide Australians with the best possible health care now and into the future. Indeed, the Committee inquiry has already triggered change as government agencies have heard and considered the evidence we received.

I want to thank everyone who took the time to give evidence to this inquiry.

We were moved by the testimony of patients and their families and inspired by the work of our researchers and medical scientists. We were impressed by the professionalism of those working in the medicines and technology sectors and appreciative of the obvious dedication, co-operation and knowledge of those within the Department of Health who assisted our deliberations in public and private hearings and through their submissions.

I would also like of thank my fellow Committee members for their close engagement and their knowledgeable contributions that each member made to this inquiry. In particular, I wish to thank the Deputy Chair, Dr Mike Freelander MP, for his expertise, good judgement and good humour. In an area of such significance, the fact that we have emerged with a bipartisan and unanimously adopted report speaks to the commitment of all Committee members.

Finally, I want to thank our committee secretariat staff, particularly Kate Portus, Rebecca Gordon and Peter Richardson. This was the largest inquiry undertaken by the Committee during my five years as Chair and they have supported our work

with exceptional dedication and quality – and occasionally some patience and forbearance!

The new frontier of medicine and technology is an exciting one for the health care we provide as a nation. Acting now to build on our obvious strengths in health will have enduring benefits for all Australians.

Mr Trent Zimmerman MP

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Terms of Reference

Inquiry into approval processes for new drugs and novel medical technologies in Australia

The House of Representatives Standing Committee on Health, Aged Care and Sport will inquire into the approval processes for new drugs and novel medical technologies in Australia, with a particular focus on those for the treatment of rare diseases and conditions where there is high and unmet clinical need.

This inquiry will consider the following topics so that Australia continues to be well positioned to access new drugs and novel medical technologies in a timely manner and respond to emerging global trends:

1. The range of new drugs and emerging novel medical technologies in development in Australia and globally, including areas of innovation where there is an interface between drugs and novel therapies;
2. Incentives to research, develop and commercialise new drugs and novel medical technologies for conditions where there is an unmet need, in particular orphan, personalised drugs and off-patent that could be repurposed and used to treat new conditions;
3. Measures that could make Australia a more attractive location for clinical trials for new drugs and novel medical technologies; and
4. Without compromising the assessment of safety, quality, efficacy or cost-effectiveness, whether the approval process for new drugs and novel medical technologies, could be made more efficient, including through greater use of international approval processes, greater alignment of registration and reimbursement processes or post market assessment.

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Abbreviations

A&AA — Allergy and Anaphylaxis Australia

AAMRI — Australian Association of Medical Research Institutes

AAMRNet — Australian Antimicrobial Resistance Network

AANMS— Australasian Association of Nuclear Medicine Specialists

ACSQHC — Australian Commission on Safety and Quality in Health Care

ACTA — Australian Clinical Trials Alliance

ACvA — Australian Cardiovascular Alliance

AFAO — Australian Federation of AIDS Organisations

AHHA — Australian Healthcare and Hospitals Association

AIHW — Australian Institute of Health and Welfare

ALLG— Australasian Leukaemia and Lymphoma Group

ANZCHOG — Australian and New Zealand Children's Haematology/Oncology Group

ANZCTR — Australia New Zealand Clinical Trials Registry

ANZSNM— Australian and New Zealand Society of Nuclear Medicine

APAA — Australian Patient Advocacy Alliance

ARC — Australian Research Council

ARTG — Australian Register of Therapeutic Goods

ASCIA — Australasian Society of Clinical Immunology and Allergy

ATAGI — Australian Technical Advisory Group on Immunisation

AusPAR — Australian Public Assessment Report

BENeLuxA — Belgium Netherlands Luxembourg Austria Ireland

BMS — Bristol Myers Squibb

BTF — Biomedical Translation Fund

CFA — Cystic Fibrosis Australia

CHF — Consumers Health Forum of Australia

CMRI — Children's Medical Research Institute

COR — Comparable Overseas Regulator

COVID-19 — Coronavirus disease 2019

CRO — Clinical research organisation/contract research organisation

CTA — Clinical Trial Authorisation

CTAG — Clinical Trials Action Group

CTC — Clinical Trials Connect

CTN — Clinical Trial Network or Clinical Trial Notification

CTX— Clinical Trial Exemption

DTx — Digital therapeutics

EMA — European Medicines Agency

EoE — Eosinophilic Esophagitis

ESC — Economic Sub Committee (in the context of the PBAC) or Evaluation Sub Committee (in the context of the MSAC)

EU — European Union

EURORDIS — European Organisation for Rare Diseases

FDA — United States Food and Drugs Administration

GBMA — Generic and Biosimilar Medicines Association

GCP — Good Clinical Practice

GDP — Gross Domestic Product

GMO — Genetically Modified Organism

HealthPACT — Health Policy Advisory Committee on Technology

HECS — Higher Education Contribution Scheme

HIV — Human Immunodeficiency Virus

HPP — Health Products Portal

HREC — Human Research Ethics Committee

HTA — Health technology assessment

ICH — International Council for Harmonisation

ICTC — International Clinical Trial Collaborations

ICTRP — International Clinical Trials Registry Platform

IP — Intellectual property

IVD — In vitro diagnostic

KDB — Knowledge Development Box

LSDP — Life Saving Drugs Program

MA — Medicines Australia

MAP — Managed Access Program

MBS — Medicare Benefits Schedule

MDDA — Metabolic Dietary Disorders Association

MES — Managed Entry Scheme

MIME — Monash Institute for Medical Engineering

MOGA— Medical Oncology Group of Australia

MRFF — Medical Research Future Fund

MRI — Magnetic resonance imaging

MSAC — Medical Services Advisory Committee

MSD — Merck Sharp & Dohme Australia

MTAA — Medical Technology Association of Australia

MUCHE — Macquarie University Centre for the Health Economy

NACCHO — National Aboriginal Community-Controlled Health Organisation

NBA — National Blood Authority

NBS — Newborn Bloodspot Screening

NCAR — Australian National Congenital Anomalies Register

NDSS — National Diabetes Supply Scheme

NHMRC — National Health and Medical Research Centre

NHS — National Health Service

NHRA — National Health Reform Agreement

NICE —National Institute for Health and Care Excellence (United Kingdom)

NIP — National Immunisation Program

NMA — National Mutual Acceptance

NMP — National Medicines Policy

ODD — Orphan Drug Designation

OECD — Organisation for Economic Cooperation and Development

OGTR — Office of the Gene Technology Regulator

PASC — PICO Advisory Sub Committee

PBAC — Pharmaceutical Benefits Advisory Committee

PBPA — Pharmaceutical Benefits Pricing Authority

PBS — Pharmaceutical Benefits Scheme

PHA — Private Healthcare Australia

PICO — Population, Intervention, Comparator and Outcome

PL — Prostheses List

PLAC — Prostheses List Advisory Committee

POCT — Point of Care Testing

PREM — Patient Reported Experience Measure

PRISM — Psychedelic Research In Science and Medicine Inc.

PROM — Patient Reported Outcome Measure

PSA — Pharmaceutical Society of Australia

PTA — Pathology Technology Australia

R&D — Research and Development

RCRDUN — Rare Cancers, Rare Diseases and Unmet Need

RDIWG — Rare Disease Industry Working Group

RDTI — Research and Development Tax Incentive

RGO — Research Governance Office

RPBS — Repatriation Pharmaceutical Benefits Scheme

RRDA — Recordati Rare Diseases Australia

RVA — Rare Voices Australia

RWD — Real World Data

RWE — Real World Evidence

SAS — Special Access Scheme

SHARE — Scottish Health Research Registry

SOSDF — Save Our Sons Duchenne Foundation

SPHERE — Sydney Partnership for Health, Education, Research and Enterprise

STA — Specialised Therapeutics Australia

TGA —Therapeutic Goods Administration

WHO — World Health Organization

List of Recommendations

Recommendation 1

11.1 The Committee recommends the Australian Government establish a Centre for Precision Medicine and Rare Diseases within the Department of Health.

- The objective of the Centre should be to ensure that the capacity of the Department of Health is enhanced to provide Australians with timely access to new drugs and novel medical technologies, including for rare diseases, and that the HTA process and government research agenda aligns with this outcome.

- The Centre should provide advice to the Department of Health and the Australian Medical Research Advisory Board on research priorities.

- The Centre should provide education and training information including support for patients and a comprehensive horizon scanning unit for new medicines and novel medical technologies.

- The Centre should provide advice to governments on the establishment of a dedicated regulatory Health Technology Assessment pathway for cell and gene technologies, in consultation with state and territory governments, industry, patients and other relevant stakeholders. The Centre should regularly provide advice to government on the effectiveness of those pathways and areas for further reform.

Recommendation 2

11.2 The Committee recommends that, consistent with Recommendation 1 and the establishment of a Centre for Precision Medicine and Rare Diseases, the

Health Technology Assessment (HTA) process for cell and gene therapies be simplified to establish a clear and certain pathway for such therapies.

- This simplified process should be considered together with a new HTA pathway for cell and gene therapy.

- Building on the Medical Research Fund Genomics Mission, the Australian Government and state and territory governments should establish a jointly funded national genomics testing program to provide equitable access to genomic testing nationwide. As part of the program, governments should ensure the provision of genomics counselling for all patients.

- The Australian Government should prioritise and simplify the regulation of cell and gene therapy pathways for clinical trials in Australia.

Recommendation 3

11.3 The Committee recommends the Australian Government establish an Office of Clinical Evaluation within the Department of Health to assess the best and most effective care for patients in the context of new and emerging health technologies.

- The Office should enable evaluation of both pharmacological and non-pharmacological interventions, combination products and products with different sponsors. It should also establish a "living evidence" function to ensure Health Technology Assessment is based on the most up-to-date global health practices.

- The Office, in consultation with relevant stakeholders, should conduct a review of how the Department's Health Technology Assessment system assesses combination products, particularly combinations with different sponsors, with a focus on:

  - Value attribution between the different products
  - Challenges to cooperation between sponsors due to competition law
  - Disincentives for a sponsor with an already listed product to participate in its combination listing

- The Office should consider collaboration with the National Institute for Health and Care Excellence (NICE) in the United Kingdom to establish similar clinical evaluation processes in Australia that links in with Australian Health Technology Assessment processes.

- The Office should cooperate and share information with the state and territory governments to ensure that patients receive treatment where it is safest and most efficacious for them and that there are no gaps in continuity of care.

Recommendation 4

11.4 The Committee recommends that the assessment process for the Life Saving Drugs Program (LSDP) be streamlined and delays in access to treatments be reduced by ensuring that a sponsor only need lodge one application for one Health Technology Assessment pathway. The Committee recommends either:

- Providing sponsors with an immediate pathway to the LSDP Expert Panel (instead of waiting for a PBAC determination), or

- Providing a pathway by adjusting the Pharmaceutical Benefits Scheme section 100 program, with specific criteria, as with other section 100 programs.

The Committee believes it is critical that consideration be given to how the LSDP will integrate with an increasing number of precision medicine applications into the future.

Recommendation 5

11.5 The Committee recommends that the Australian Government develop a labour market and skills strategy to expand the number of health economists in Australia. This could include encouraging training within Australia as well as seeking expertise from overseas.

Recommendation 6

11.6 The Committee recommends that the Department of Health increase its efforts to educate and engage with patients, clinicians, industry and the

public and develop education campaigns on all aspects of the regulation and reimbursement system.

11.7 The Committee recommends that the Department of Health improve information available on the websites of the Therapeutic Goods Administration (TGA) and its Health Technology Assessment (HTA) bodies for all users including patients, clinicians, industry and the public. This would include:

- Using plain English language, infographics and videos to explain general processes and timelines
- Explanations on the TGA and all HTA's websites of how that entity fits into the overall regulation and reimbursement system, similar to the Medical Services Advisory Committee's *Australian Government HTA Processes* factsheet.
- The Department of Health expanding the Pharmaceutical Benefits Scheme Medicines Status website to include technologies funded through the Medicare Benefits Schedule or create an equivalent website for such technologies.

Recommendation 7

11.8 The Committee recommends that the Department of Health and the National Blood Authority, in consultation with state and territory governments, reform the Health Technology Assessment processes for blood products to provide better alignment with the Health Technology Assessment system, including:

- Publication of guidance documents for applicants
- Establishment of timelines for applications, and publication of an assessment cycle calendar
- Creation of a parallel Therapeutic Goods Administration and Health Technology Assessment process.

Recommendation 8

11.9 The Committee recommends that the Australian Government make the following changes to submission fees for the Therapeutic Goods Administration (TGA) and the Pharmaceutical Benefits Advisory Committee (PBAC) and where appropriate Medical Services Advisory Committee (MSAC) assessments in the following separate circumstances:

- Replace the current orphan drug fee waivers with a HECS-style fee waiver, in which orphan drug application fees are payable on successful application, only once the drug has earned the sponsor a certain amount of revenue. The Department of Health should determine this threshold value in consultation with industry

- To support smaller companies, HECS-style fee waivers for any sponsor company with revenue at or below $50 million per annum

- HECS-style fee waivers for Australian start-up companies with a specified amount of revenue in the Australian market to promote innovation.

The Committee also recommends introducing a sliding scale for fees for resubmissions, with fees being lower for resubmissions.

Recommendation 9

11.10 The Committee recommends that the Australian Government establish a fund to support patients, clinicians and non-profit organisations to sponsor registration and reimbursement applications where there is no realistic prospect of a company serving as sponsor, and where the Department of Health is otherwise supportive of the application.

- Such a fund should be targeted at treatments for conditions where low patient numbers in Australia serve as a market barrier and where there is a clinical demand and need. The fund should be available for applications to repurpose previously listed medicines and technologies.

- The fund should be annually capped with clear and transparent eligibility rules.

Recommendation 10

11.11 The Committee recommends that the Australian Government amend the *National Health Act 1953* (Cth) to give the Pharmaceutical Benefits Advisory Committee the power to authorise Managed Access Programs. The eligibility criteria for these Managed Accessed Programs should be aligned as far as possible with the eligibility criteria for the Therapeutic Goods Administration's provisional registration.

Recommendation 11

11.12 The Committee recommends that the Department of Health conduct a comprehensive consultation process with industry to establish a more flexible way forward for the repurposing of drugs in Australia. This should include:

- Establishing a new pathway that incentivises the repurposing of drugs for all diseases, not just rare disease.

Recommendation 12

11.13 The Committee recommends that the Therapeutic Goods Administration make the following changes to its Orphan Drugs Program:

- Provide automatic access to the Priority Review Pathway for all medicines granted an orphan drug designation

- Treat paediatric patient populations as separate to adult patient populations for the purposes of the eligibility criteria

- Better account for the extra costs incurred by a sponsor in expanding its medicine to paediatric indications, for the purposes of assessing commercial viability as part of the eligibility criteria

- Where the prevalence of a disease is unknown in Australia, accept evidence of prevalence in other comparable countries or, in diseases of extremely low prevalence, worldwide for the purposes of the eligibility criteria.

Recommendation 13

11.14 The Committee recommends that the Department of Health reform its regulatory and reimbursement processes to enable therapeutic goods to be registered and reimbursed by molecular indication in addition to by disease indication. This should include legislative change if necessary.

Recommendation 14

11.15 The Committee recommends that the Australian Government reconsider the current cost recovery funding model for the Therapeutic Goods Administration, paying attention to future staffing and IT infrastructure needs in an environment where demand on its services and systems are expected to increase in future years. The Committee recommends funding specifically for:

- IT systems upgrades, to modernise and match the IT capability of other overseas Tier 1 regulators.
- An expansion of its staffing capacity in areas of new medical and technological advances including for horizon scanning.
- The release of TGA Australian Public Assessment Reports at the same time as a prescription medicine is listed.
- The implementation of the HECS-style fee waivers outlined in Recommendation 8.

Recommendation 15

11.16 The Committee recommends that the Australian Government ensure the membership of the Pharmaceutical Benefits Advisory Committee and Medical Services Advisory Committee provides the appropriate expertise for all applications. This should include the possibilities of enhanced cross-membership between the two committees and the appointment of temporary members to consider individual applications.

- Recognising the nature of health challenges in Indigenous communities, membership should include representation from Aboriginal and Torres Strait Islander Peoples.

Recommendation 16

11.17 The Committee recommends that the Department of Health investigate further opportunities for the formation of an international Health Technology Assessment consortium similar to the Access Consortium to streamline the regulatory process for certain medicines and medical technologies. This investigation should include discussions with representatives of the Health Technology Assessment bodies of the United Kingdom, Canada and other countries with systems similar to Australia's.

- The Committee recommends that the Therapeutic Goods Administration work with the United States Food and Drug Administration and other overseas regulators to establish an equivalent of Project Orbis for non-cancer rare diseases, or to expand Project Orbis to include such diseases.

Recommendation 17

11.18 The Committee recommends that the Australian Government establish a scheme that supports the domestic medical technology sector, similar to the Food and Drug Administration's Breakthrough Devices Program in the United States.

Recommendation 18

11.19 Recognising the vital role that vaccines play in addressing many diseases, including its importance in providing protection against Covid-19, the Committee recommends that the Department of Health conduct a review of the National Immunisation Program. This review should focus on reforming existing approaches used to value vaccines to ensure early and rapid deployment of vaccines in Australia.

Recommendation 19

11.20 The Committee recommends that the Australian Government continue to address the following matters in its reforms to the Prostheses List:

- The lack of coverage for non-implantable devices under the current arrangements.

- Improving coordination between the Medical Services Advisory Committee and the Prostheses List Advisory Committee to provide faster access for patients.

Recommendation 20

11.21 The Committee recommends that the Australian Government establish a last resort mechanism for directly securing ongoing supply of medicines that meet a high clinical need and lack suitable alternatives that are at risk of being delisted from the Pharmaceutical Benefits Scheme.

Recommendation 21

11.22 The Committee recommends:

- The federal, state and territory health authorities complete the standardisation of newborn screening across Australia

- As part of that process, the Australian Government work with states and territories to expand the newborn screening program based on new understandings of genomic testing for conditions and international best practice

- That the Australian Government in collaboration with states and territories, conduct reviews every two years to determine whether the screening program should be further expanded based on new Australian and international scientific and medical knowledge.

While not in the terms of reference for this inquiry, the Committee recognises and supports the calls from rare disease patient groups for more funding for treatment pathways for actionable disorders across states and territories, where identified through newborn screening.

Recommendation 22

11.23 The Committee recommends that all levels of government prioritise and implement with urgency the harmonisation of Human Research Ethics Committee (HREC) and Site-Specific Assessment submissions into one Australian online platform and enable parallel review by HRECs and Research Governance Offices.

- The platform should be developed within the purview of the Australian Commission on Safety and Quality in Health Care.

- This work should be a continuation from the work prepared as part of the National Clinical Trials Governance Framework.

Recommendation 23

11.24 The Committee recommends that all levels of government jointly provide funding for the development of a national clinical trial register. It should include:

- Development of a sophisticated digital platform to collect and facilitate patient identification, patient recruitment, patient retention and completion rates for clinical trials.

- Linked data from existing national registers and consideration should be given to whether the register is best operated by a government agency or an existing Non-Government Organisation, or an academic body with appropriate experience.

Recommendation 24

11.25 The Committee recommends the Australian Government develop policies that encourage modernising digital technologies and practices to position Australia as the premier destination for international clinical trials. This would include developing national standards for the use of e-consent, e-signature, and electronic medical records to enable remote monitoring and participation in clinical trials across Australia.

- National standards should include standardising clinical costs and fees that are competitive with international fees.

Recommendation 25

11.26 The Committee recommends the Australian Government should develop a national standard approach, including nationally agreed systems and standard operating procedures to support and strengthen the capacity to conduct clinical tele-trials in rural, regional and remote areas.

- This approach should be developed in consultation with industry and allied health workers.

- This would include the need for education and training opportunities for General Practitioners and all allied health workers engaging in clinical trials using tele-trials and multi-centre trials.

Recommendation 26

11.27 The Committee recommends the Australian Government should continue to fund Clinical Trial Networks with a particular focus on developing seed funding for Indigenous Health Clinical Trial Networks.

Recommendation 27

11.28 The Committee recommends the Australian Government reform data exclusivity provisions in Australia with a view to extending data exclusivity for orphan drugs and vaccines to a period of up to 10 years. The Australian Government should:

- Develop additional reforms to data exclusivity timeframes to support research and development into new drugs and novel medical technologies in areas of unmet need.

- Consider future funding initiatives for novel drug discovery and support research and development partnerships in Australia. This would assist new drugs and novel medical technologies in early stage and pre-commercial development.

- In partnership with the states and territories, develop and implement a pilot scheme for value-based payments for new antimicrobial drugs. This pilot should apply the lessons learned from the Australian Government's pilot scheme for payment for Hepatitis C drugs, as well as from overseas antimicrobial drug schemes.

- Promote the recent research and development tax initiatives internationally as a way of encouraging industry to look to Australia for future investments in the healthcare sector.

- Conduct a full review of the patent box scheme every two years after implementation to ensure it is operating effectively and driving increased expenditure and innovation within Australia.

- Collaborate with the states and territories to review the funding of the research and development sector in health care to distribute funding in a methodical way that provides sufficient support throughout the research funding 'pipelines'.

  - Noting the work underway through the Modern Manufacturing Program, the Committee supports the development of an updated roadmap to facilitate the manufacturing and commercialisation of novel drugs and technologies in Australia.

Recommendation 28

11.29 The Committee recommends that:

- The Department of Health integrate the patient voice upfront into the Health Technology Assessment system. Earlier patient engagement with the Health Technology Assessment system would include:

  - Representation from peak patient bodies that is refreshed every three – five years
  - Representation of Aboriginal and Torres Strait Islander Peoples.

- The Department of Health implement a notification system for all HTA bodies and the TGA to advise relevant patient groups of the receipt of an application.

- The Department of Health provide patients and stakeholders with a concise sponsor's submission summary to help facilitate their own involvement in the Health Technology Assessment process.

- The Department of Health should consider making patient evidence compulsory for certain applications, and should consider the role of patient evidence in the decisions of the Therapeutic Goods Administration.

- The Department of Health should notify relevant patient groups of the outcome of the assessment process by all HTA bodies.

- The Department of Health be funded to implement these recommendations.

- The Australian Government provide funding for organisations to support participation in the HTA process, including for very rare disease patient groups that have limited capacity for fundraising or access to alternative funding.

Recommendation 29

11.30 The Committee recommends that:

- The Committee recommends that the Australian Government amend the *National Health Act 1953* (Cth) to formalise the role and powers of the Pharmaceutical Benefits Advisory Committee Executive. The scope of the Executive's role and powers should be determined by agreement between the Executive and the Department of Health.

- The Department of Health produce a pre-submission advice framework for submissions to the Therapeutic Goods Administration, Pharmaceutical Benefits Advisory Committee, Medical Services Advisory Committee and other Health Technology Assessment bodies, explaining the interaction between those bodies and their evidentiary and other requirements, to be provided to sponsors before they make their submissions.

- The independent Health Technology Assessment Review reassess relevant aspects of the Health Technology Assessment process to ensure there are future pathways for treatments and therapies that do not fit neatly into the current system such as rare cancers, antimicrobials, orphan drugs, and precision medicines.

  - It is imperative that appropriate clear pathways are considered for inclusion for paediatric medicines and technologies.
  - The Committee is of the clear view that precision medicine approval pathways will require a different application assessment than current approaches designed for treatments for common conditions, with large data sets and comparative evaluations.

- The Department of Health publish data on application processing times and positive recommendation rates for the Pharmaceutical Benefits Advisory Committee and other Health Technology Assessment bodies. In addition:

  - The Department of Health should publish Health Technology Assessment processing times annually, benchmarked against other nations with advanced HTA processes.

- The Australian Government, in collaboration with relevant stakeholders, develop a suite of clear and measurable benchmarks to track the Commonwealth's implementations of the recommendations made by the Committee and accepted by the Australian Government.

  - These agreed benchmarks along with measurable KPIs/metrics should be developed in such a way as to best facilitate the Department of Health, including its agencies and other relevant statutory bodies, in the tabling of an annual update to the Australian Parliament.

Recommendation 30

11.31 The Committee recommends that the Australian Government's independent Health Technology Assessment Review (which is scheduled to commerce in July 2022) consider and develop reforms in the following areas:

- Reducing the frequency and need for applications to HTA bodies to be resubmitted.

- Streamlining the interaction between hospitals and the Health Technology Assessment system

- Streamlining the interaction of the Therapeutic Goods Administration, the Pharmaceutical Benefits Advisory Committee, the Medical Services Advisory Committee and other Health Technology Assessment bodies

- Cooperation and harmonisation between Australian Health Technology Assessment bodies and equivalent bodies overseas

- Improving the measurement of the performance of the Pharmaceutical Benefits Advisory Committee and the publication of data on that performance

- Improving the mechanisms for communication between sponsors and the Pharmaceutical Benefits Advisory Committee during the submission process

- Increasing the use of Managed Access Programs to facilitate earlier access to innovative medicines

- Increasing the use of Real World Evidence in Health Technology Assessment

- Improving flexibility when choosing a comparator in Health Technology Assessment

- Introducing a scoping process that includes patients and clinicians at an early stage to agree on the framework that the submission will be considered. This process could draw on the approach taken by the United Kingdom's National Institute for Health and Care Excellence

- Improving the independent review process for HTA decisions, including the potential for this to be made available to groups of patients and clinicians in addition to sponsors.

Recommendation 31

11.32 The Committee recommends that:

- The Department of Health should consider, in consultation with state and territory governments, industry, patients and clinicians, the introduction of fees for Medical Services Advisory Committee applications on a cost recovery basis, if this is necessary to increase the speed and effectiveness of assessments. If fees are introduced they should have similar features to those recommended by the Committee for Pharmaceutical Benefits Advisory Committee fees (including those arrangements outlined at Recommendation 8).

- The Medical Services Advisory Committee increase the involvement of clinicians in its assessments of technologies with which its members lack relevant expertise.

- The Department of Health introduce an equivalent to the Managed Access Programs for medical devices. The details of this scheme including eligibility criteria and duration should be formulated in consultation with patient groups, clinicians and industry.

- The Therapeutic Goods Administration introduce parallel processing of applications with the Medical Services Advisory Committee.
- The Medical Services Advisory Committee increase opportunities for sponsors of particularly complex applications to present to it at its meetings and expand the opportunities for pre-submission meetings.
- The Medical Services Advisory Committee consider developing international collaboration for complex assessment proposals.
- The Department of Health expand the independent Health Technology Assessment Review in July 2022 to include Medical Service Advisory Committee processes.
- The Medical Services Advisory Committee publish a full calendar timeline of meeting agenda and outcomes, including dates when minutes and Public Summary Documents will be made public.
- The Medical Services Advisory Committee publish additional guidance for sponsors of digital health technologies.
- The Department of Health establish a benchmarking system for MSAC assessments, including benchmarking against comparable overseas organisations.

1. Introduction

1.1 Australia's regulatory system for bringing new medicines and devices to patients is regarded as being thorough and robust and is well respected internationally. Australians should be proud of our healthcare system. The Committee heard this from stakeholders, including the pharmaceutical industry, patient advocacy groups and clinicians throughout the inquiry. Many witnesses congratulated the staff working within the Department of Health, including the Therapeutic Goods Administration (TGA) for their professionalism and dedication working on the regulation and reimbursement systems.

1.2 Along with this praise came suggestions for improvements to make Australia's healthcare system even better. A significant challenge for Australia's regulatory system was to establish more flexible pathways to enable our system to keep pace with medical and technological advances, including precision medicine, that are available now.

1.3 Numerous stakeholders raised the issue of the length of time it takes for a new medicine to get approved and listed on the Pharmaceutical Benefits Scheme (PBS). The Australian system was compared with other international regulatory systems and the findings were variable depending upon which factors were included for comparison. It became clear to the Committee that international regulatory systems are all unique and complex.

1.4 Access to medicines and therapies for rare disease and precision medicine was discussed as a significant challenge that required solutions to enable more equity for patients. Some of the challenges for rare disease and precision medicine access raised issues relating to research and development, clinical trials and the status of using real world evidence.

1.5 The Committee launched this inquiry in August 2020 just months after the declaration that the world was living with the COVID-19 pandemic. At the time that the Committee was reviewing Australia's regulatory system, the Therapeutic Goods Administration and the Department of Health were fast tracking approval processes for certain drugs to assist with the treatment of COVID-19 patients in Australia. Many submissions noted this unprecedented collaboration with international and Australian regulators, pharmaceutical companies and clinical researchers. It was suggested that lessons could be learned from the pandemic and that our regulatory systems should be streamlined and adapted to cope with the flood of new healthcare innovations coming in the near future.

1.6 The Committee was mindful of the increasing globalisation of the pharmaceutical and medical devices industries and the rapid pace of innovation and change within the healthcare sector and how this impacted heavily on Australia's regulatory system. In addition, the Committee recognised that Australia's ageing population and growing burden of chronic diseases reinforced the importance of continued and ongoing investment in the timely access to new medicines and devices.

1.7 Stakeholders urged the Australia Government to consider the recommendations from this report together with the National Medicines Policy Review that recommenced in August 2021. These two reviews present an opportunity to continue this collaborative approach to reform and work towards a more streamlined system to access medicines and devices in Australia. This report lists the recommendations in the final chapter.

About the inquiry

Objectives and scope

1.8 On 13 August 2020, the Minister for Health, the Hon Greg Hunt MP, referred the *Inquiry into the approval processes for new drugs and novel medical technologies in Australia* (the inquiry) to the Standing Committee on Health, Aged Care and Sport (the Committee). The inquiry included a particular focus on approval processes and novel medical technologies for the treatment of rare diseases and conditions where there is high and unmet clinical need.

1.9 As part of the inquiry, the Committee examined the range of new drugs and emerging novel medical technologies that are in development and

progressing through the regulatory system in Australia and in other countries of the world.

1.10 Other focus areas included:

- Examining the approval processes of new drugs and medical technologies including whether these processes could be made more efficient without compromising safety, quality and efficacy
- Measures that could make Australia more attractive for clinical trials; and
- Incentives to research and commercialise new drugs and novel medical technologies.

1.11 The Committee appreciated receiving informative submissions from individuals, family members, patient advocacy groups, and peak bodies from small and large disease/patient groups who spoke of changes that were needed to make Australia's access to new drugs and medical devices more equitable and efficient. These submissions provided the Committee with insights into the importance of incorporating 'the patient voice' into the approval process.

1.12 The Committee thanks all stakeholders who were generous with their time and patience in bringing the Committee up to speed with Australia's regulatory and reimbursement system. This inquiry was complex and technical and required the Committee to have a comprehensive understanding of the system before it could consider making any recommendations to adjust it.

Inquiry conduct

1.13 On 18 August 2020, the Committee issued a media release announcing the inquiry and calling for submissions. The Committee invited submissions from government agencies, industry groups and pharmaceutical companies, research centres and universities, patient advocacy groups and healthcare providers, and the general public.

1.14 The inquiry received 207 submissions and an additional 30 supplementary submissions and one exhibit, which are listed in Appendix A and B.

1.15 The Committee held public hearings over 13 days, as outlined below. A list of witnesses and organisations who attended these public hearings is listed in Appendix C.

Table 1.1 Public hearings held

Date	Place
3 September 2020	Canberra ACT
5 February 2021	Canberra ACT
11 March 2021	Sydney NSW
12 March 2021	Sydney NSW
26 March 2021	Canberra ACT
22 April 2021	Melbourne VIC
23 April 2021	Melbourne VIC
7 May 2021	Sydney NSW
17 May 2021	Brisbane QLD
18 May 2021	Brisbane QLD
18 June 2021	Canberra ACT
24 June 2021	Canberra ACT
7 July 2021	Canberra ACT

Report structure

1.16 This report consists of eleven chapters. The final chapter is a list of recommendations:

- Chapter 2 provides a general overview of the recent reviews conducted, and the agreements entered into, by the Australian Government in relation to Australia's health programs and regulatory frameworks, which have had a bearing on the Committee's deliberations and subsequent recommendations in this report.

- Chapter 3 presents a high level overview of the regulatory and reimbursement frameworks, the general understanding of how these systems work, and where there are gaps in the system.

- Chapter 4 describes the concept of the 'patient voice', how it is currently drawn on in decision-making by Australia's Department of Health and in overseas models, and what further improvements to government engagement with the patient voice could look like.

- Chapter 5 provides an overview of the Therapeutic Goods Administration including the general themes to have emerged throughout the inquiry, including the regulation of medicines and medical devices, and the financial and technical aspects of its regulation.

- Chapter 6 outlines the Heath Technology Assessment (HTA) system. It discusses the processes of the Pharmaceutical Benefits Advisory Committee. Some of the main issues discussed include: the application process, length of time for review, fees, provisional access and international regulators.

- Chapter 7 explores the Medical Services Advisory Committee, another advisory committee in the HTA system that focuses on medical devices and services. Again, issues of flexibility, length of time for review, resourcing and application processes are discussed, as with its approach to real world evidence. The chapter also looks at the Prostheses List Advisory Committee and the future of the Prostheses List.

- Chapter 8 explores the important issue of rare disease, focussing on Government initiatives, potential HTA alternative pathways, the Life Saving Drugs Program, newborn screening and limitations on data, research and clinical trials.

- Chapter 9 looks at clinical trials in Australia including our regulations and challenges, why we have a competitive advantage, and discusses what is needed for Australia to be ready for a surge in demand for novel medicines and devices in the clinical trial sector.

- Chapter 10 discusses research and development in Australia and what the Australian Government is doing to fund initiatives, what research incentives are available, the need for further and greater horizon scanning, and the regulatory hurdles attached to the repurposing of drugs.

2. Recent reviews and agreements

The Therapeutic Goods Administration and updates to the Health Technology Assessment process

2.1 The Committee was aware that there has been a number of reviews and reforms of Australia's different health programs undertaken since 2013, including the *Expert Panel Review of Medicines and Medical Devices Regulation* (Sansom Review).

2.2 The Sansom Review was engaged to assess the current regulatory framework and make recommendations on options to improve the way in which therapeutic goods are regulated in Australia.[1]

2.3 In response to the Sansom Review, the Government provided $20.4 million over four years (including $9.5 million in capital funding) from 2016–17 to improve the regulation of therapeutic goods in Australia. The ongoing cost of the measure from 2017–18 is to be met by the TGA's cost recovery arrangements.[2]

2.4 The Department of Health (the Department) emphasised that Australia's regulatory and HTA processes continue to deliver good outcomes for Australians because they are subject to continuing review and improvement. Recent improvements to HTA processes include:

- greater collaboration across HTA committees and the Department to align regulatory and reimbursement processes

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1 Department of Health, Canberra, March 2015, Review of Medicines and Medical Devices Regulation, Report on the regulatory framework for medicines and medical devices, p. vii.

2 Australian Government, Budget Papers No. 2, B*udget Measures: Budget Paper No. 2: 2016–17*, p. 106.

- improved mechanisms for consumer involvement and engagement in HTA
- a Strategic Agreement with Medicines Australia that has streamlined medicines listing processes and reduced the time to listing by an average of 3.5 months
- the development of a Health Products Portal to reduce duplication and red tape through a digital solution for applicants engaging with both regulatory and reimbursement processes
- the 2020-25 National Health Reform Agreement which provides specific arrangements to ensure Australians with some of the rarest conditions have access to new, life-saving highly specialised therapies in public hospitals
- the use of Managed Access Programs to provide early access to clinically important medicines
- post-market reviews to inform optimal and sustainable use of listed medicines.[3]

National Medicines Policy Review

2.5 The Department describes Australia's National Medicines Policy (NMP) as a 'cooperative endeavour to bring about better health outcomes for all Australians, focusing especially on people's access to, and wise use of, medicines.'[4]

2.6 The NMP was published in 2000 and aims to deliver positive health outcomes for all Australians through their access to and appropriate use of medicines. It has four main pillars:

- timely access to the medicines that Australians need, at a cost that individuals and the community can afford
- medicines meeting appropriate standards of quality, safety and efficacy
- quality use of medicines
- maintaining a responsible and viable medicines industry.[5]

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3 Department of Health, Submission 15, pages 6-7.

4 Department of Health, Canberra, www1.health.gov.au/internet/main/publishing.nsf/Content/national-medicines-policy, viewed 21 September 2021.

5 Department of Health, Canberra, National Medicines Policy, p. 1, www1.health.gov.au/internet/main/publishing.nsf/Content/national-medicines-policy,

2.7 In recognition of the changing medicines landscape over the past 20 years, the Minister for Health made an election commitment in 2019 to review the NMP. The aim of the review is to identify any gaps in the policy's objectives, partnership approach and accountabilities.

2.8 The review of the NMP was delayed due to the COVID-19 pandemic.[6] The Department informed the Committee that the Review of the NMP will re-commence in August 2021.[7]

2.9 The Minister for Health has established an Expert Advisory Committee to lead the Review of the NMP for the Department. The Committee is chaired by Deputy Chief Medical Officer, Professor Michael Kidd AM. Its members include Professor Lloyd Sansom AO; Mrs Janette Donovan; Dr Sarah Dineen-Griffin and Mr David Herd.

2.10 This review will support a refresh of the NMP as a high-level policy framework, to ensure that the changes in the health system environment are addressed, and where applicable, the policy updated to take account of these changes.[8]

Post-market review of the Life Saving Drugs Program

2.11 The Australian Government's Life Saving Drugs Program (LSDP) provides subsidised access for eligible patients with rare and life-threatening diseases to essential and very expensive medicines. Persons with these rare diseases often require medicines that have a very high cost per patient. These medicines often fail to meet the comparative cost effectiveness criteria required for Pharmaceutical Benefits Scheme (PBS) funding. The LSDP provides eligible patients with access to these life-saving medicines at no expense to the patients or their families.[9]

2.12 In April 2014, the then Minister for Health announced the Post-market Review of the Life Saving Drugs Program (LSDP Review), providing an opportunity to review the program to ensure that Australians with very rare conditions continue to have subsidised access to much-needed, expensive

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6 Department of Health, Submission 15, p. 26.

7 Department of Health, Submission 15.6, p. 5.

8 Department of Health, Canberra, www.consultations.health.gov.au/technology-assessment-access-division/national-medicines-policy-review/ viewed 27 September 2021.

9 Australian Government response to the Post-market review of the Life Saving Drugs Program www.pbs.gov.au/reviews/lsdp-report/government-response-to-lsdp-review.pdf viewed 4 October 2021.

medicines. The LSDP Review examined important issues such as access and equity, value for money and the future administration of the program.[10]

2.13 A number of recommendations were made including that consideration be given to the value of medicines for rare diseases to consider matters beyond cost-effectiveness – 'these principles are already embedded in the approach used by the PBAC (Pharmaceutical Benefits Advisory Committee) in its decision making but this would benefit from being more transparent.' Further, 'consideration should be given to enhancing the medicines submission process for rare disease therapies by adopting a collaborative multi-stakeholder approach early in the assessment cycle, before the medicine submission is formally submitted for consideration by the PBAC.'[11]

2.14 In response to the LSDP Review, the Australian Government agreed to ensure that eligible patients retain ongoing access to medicines currently available through the LSDP; a pathway to consider new medicines which includes fit-for-purpose clinical effectiveness and cost effectiveness assessment; and the future integrity and sustainability of the program.[12]

Strategic Agreement 2022 – 2027 with Medicines Australia

2.15 In early September 2021, Medicines Australia signed a new, five-year Strategic Agreement with the Australian Government (MA Strategic Agreement) to deliver greater long-term policy certainty for patients, industry and the Government. The Committee was pleased to note that the MA Strategic Agreement will ensure that this report and the review into the NMP will play a role in improving the HTA processes.

2.16 Aims for the MA Strategic Agreement are as follows:

- Provide timely access to new medicines and vaccines.
- Ensure patients have greater involvement in decision making for medicines access.
- Modernise processes to keep pace with advancing science and innovative technologies.

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10 Department of Health, Canberra, www.pbs.gov.au/pbs/reviews/life-saving-drugs viewed 4 October 2021.

11 Department of Health, Canberra, Post-market review of the Life Saving Drugs Program, June 2014 – June 2015, https://www.pbs.gov.au/reviews/lsdp-report/lsdp-review-report.pdf viewed 4 October 2021.

12 Department of Health, Canberra, Australian Government response to the Post-market review of the Life Saving Drugs Program https://www.pbs.gov.au/reviews/lsdp-report/government-response-to-lsdp-review.pdf viewed 4 October 2021.

- Address the changing international policy environment on access.
- Keep Australia as a global priority for the launch of new and innovative medical treatments.[13]

2.17 Key measures for the MA Strategic Agreement include:

- An independent review of HTA processes will ensure Australia's HTA system evolves to keep pace with advancements in medical technologies. The Review will run from July 2022 – June 2023, with recommendations to be implemented by July 2024.
- The HTA Review will elevate the patient voice by including a patient representative on the Review Committee.
- An enhanced Patient Engagement Process will be created to incorporate patient views early in the PBAC system.
- The House Standing Committee on Health, Aged Care and Sport's inquiry and the review of the National Medicines Policy will play a role in improving Australia's HTA processes.
- Pricing reforms will provide clear purchasing and pricing arrangements with innovative medicines and vaccines manufacturers to ensure Australia has a viable supply of medicines.
- The New Medicines Funding Guarantee, agreed in 2020, will deliver $2.8 billion of PBS funding for new and amended listings over the forward estimates without the need for offsets.
- Medicines Australia will run an annual Horizon Scanning Forum from 2022 to identify major advances in healthcare over the next 3-5 years.
- Security of supply measures will help to reduce medicine shortages.
- Hospital price disclosure will support ongoing sustainability and supply.
- A pharmaceutical industry representative will be appointed to the Medical Services Advisory Committee.[14]

Strategic Agreement 2022 – 2027 with the Generic and Biosimilars Medicines Association

2.18 In early September 2021, the Australian Government and the Generic and Biosimilar Medicines Association (GBMA) signed off on a new five year strategic agreement (GBMA Strategic Agreement), brought forward by one

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13 Medicines Australia, *Strategic Agreement Factsheet for MPs*, Submission 141.2, pages 1-2.

14 Medicines Australia, *Strategic Agreement Factsheet for MPs*, Submission 141.2, pages 1-2.

year out of concern for patients who are struggling to access vital medicines during to the global pandemic disrupting international supply of medicines.

2.19 In essence, the GBMA Strategic Agreement will strengthen the PBS for patients and ensure improved stability and viability for the medicines industry. It will also ensure pharmacy shelves across Australia are stocked and that some Australians will have early access to new life changing medicines regardless of where they live.

2.20 The generic and biosimilar industry contributes more than two thirds of all medicines dispensed on the PBS each year.

2.21 The GBMA has reconfirmed its commitment to working with Government on the 'Repurposing of Medicines' initiative in order to expand patient access to some medicines.[15]

[15] Generic and Biosimilar Medicines Association, *Generic Medicines Facts*, www.gbma.com.au/generic-facts/, viewed 27 September 2021.

3. Understanding the System

Access to new drugs and medical technologies

Regulation of therapeutic goods

3.1 The Australian Government regulates 'therapeutic goods', which are broadly defined as goods 'for therapeutic use'.[1] This means use in human beings for:

- Preventing, diagnosing, curing or alleviating a disease
- Influencing, inhibiting or modifying a physiological process
- Testing susceptibility to a disease or ailment
- Influencing, controlling or preventing conception
- Testing for pregnancy
- Replacing or modifying parts of the anatomy.[2]

3.2 Therapeutic goods fall into four categories:

- **Medicines**: goods that achieve their intended action by pharmacological, chemical, immunological or metabolic means[3]
- **Biologicals**: goods that contain or are derived from human cells or tissues[4]
- **Medical devices**: devices (including supporting software) used for diagnosis, prevention, monitoring, treatment or alleviation of a disease,

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1 *Therapeutic Goods Act 1989* (Cth) s. 3.

2 Department of Health, Submission 15, p. 4; *Therapeutic Goods Act 1989* (Cth) s. 3.

3 *Therapeutic Goods Act 1989* (Cth) s. 3.

4 *Therapeutic Goods Act 1989* (Cth) s. 32A.

injury or disability; investigation, replacement or modification of the anatomy or a physiological process; or control of conception[5]

- **Other therapeutic goods**.[6]

3.3 Under the *Therapeutic Goods Act 1989* (Cth) the responsibility for regulation of such goods technically rests with the Secretary of the Department of Health (the Department), but in practice this responsibility is delegated to the Therapeutic Goods Administration (TGA), which forms part of the Department.[7] The TGA ensures that therapeutic goods are safe and fit for purpose.[8]

3.4 The TGA is required to recover its costs through fees and charges for all activities that fall within the scope of the *Therapeutic Goods Act 1989* (Cth) including its public health responsibilities.[9] A small amount of appropriation funding is provided for other activities. For example, in the 2019/20 Mid-Year Economic and Financial Outlook statement, the Government provided $33 million over four years (including $6.6 million in 2020/21) for work on improvement of patient safety through regulatory measures for opioids and to partially defray the costs of the TGA Special Access Scheme, Orphan Drugs Program and mandatory reporting of shortages of critical medicines.[10]

3.5 Unless an exception applies, therapeutic goods must be entered on the Australian Register of Therapeutic Goods (ARTG) before they can be imported, exported, supplied or advertised.[11] There are two categories of medicines:

- Higher risk medicines — all prescription medicines, most over-the-counter medicines and some complimentary medicines — are 'registered', which involves them being assessed by the TGA for quality, safety and efficacy

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5 *Therapeutic Goods Act 1989 (*Cth) s. 41BD.

6 *Therapeutic Goods Act 1989* (Cth) s. 3.

7 *Therapeutic Goods Act 1989* (Cth) s. 9A; Department of Health, Submission *15*, p. 4.

8 Department of Health, Submission 15, p. 4.

9 Therapeutic Goods Administration (TGA), *TGA regulatory framework*, Canberra, September 2020, https://www.tga.gov.au/tga-regulatory-framework viewed 23 September 2021.

10 Department of Health, Submission 15.6, p. 1.

11 Department of Health, Submission 15, p. 4.

- Lower risk medicines — medicines containing pre-approved, low risk ingredients for which limited claims of efficacy are made — can simply be listed.[12]

3.6 Biologicals are classified into four classes on the basis of risk to patients. Biologicals in Classes 1 and 4 are listed in Schedule 16 of the *Therapeutic Goods Regulations 1990* (Cth), whereas Classes 2 and 3 are defined by method of preparation and intended use.[13] Class 1 biologicals are lowest risk and only require the sponsor to certify that they meet the necessary requirements, while the remaining classes require the submission of a full dossier of evidence which is evaluated by the TGA, including its Advisory Committee on Biologicals if necessary.[14]

3.7 Medical devices are also classified on the basis of risk to patients, with the classes being Class I, Class IIa, Class IIb, Class III and Class AIMD (Active Implantable Medical Devices) from lowest to highest risk. In vitro diagnostic (IVD) medical devices are classified separately, although likewise on the basis of risk, into classes 1, 2, 3, 4. Devices undergo 'conformity assessment', which means the sponsor must provide evidence that the device conforms to a set of 'Essential Principles'. The level of evidence required depends on the classification of the device.[15]

Therapeutic Goods Administration pathways

3.8 The TGA has a number of options, described as 'pathways', for sponsors which wish to have their therapeutic good included on the ARTG. These include the following pathways that are described below:

- Standard review
- Parallel process

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12 Department of Health, Submission 15, pages 4-5.

13 TGA, *Classification of biologicals*, Canberra, November 2020, www.tga.gov.au/classification-biologicals, viewed 28 July 2021.

14 TGA, *Applying for Inclusion of a Class 1 biological in the ARTG*, Canberra, November 2020, www.tga.gov.au/classification-biologicals, viewed 28 July 2021; TGA, *Applying for inclusion of a Class 2, 3 or 4 biological on the ARTG – a step-by-step guide*, Canberra, November 2020, www.tga.gov.au/applying-inclusion-class-2-3-or-4-biological-artg-step-step-guide, viewed 28 July 2021.

15 TGA, *Overview of medical devices and IVD regulation*, Canberra, October 2020, www.tga.gov.au/sme-assist/medical-devices-regulation-introduction, viewed 31 August 2021.

- Orphan drug[16] fee waiver
- Priority review
- Provisional approval
- Comparable Overseas Regulator
- A
- B
- The Access Consortium
- Project Orbis.

3.9 **Standard review** for prescription medicines is an eight phase process designed to prove the quality, safety and efficacy of the medicine. These phases include submission of a full dossier of evidence by the sponsor, two rounds of assessment by the TGA, a request for information or documents from the TGA to the sponsor, and review by one of the TGA's expert advisory committees. The process is designed to take an average of 330 calendar days in total, or 11 months.[17]

3.10 The **parallel process** is available for medicines and vaccines that meet certain criteria, and means that they are effectively considered by the TGA for regulatory approval and the Pharmaceutical Benefits Advisory Committee (PBAC) for reimbursement at the same time. Nonetheless the PBAC generally requires a positive indication from the TGA before it considers the application at one of its meetings, and the PBAC's final decision must accord with the TGA's.[18]

3.11 An **orphan drug designation** offers waiver of application fees for the designated drug.[19] It is available for prescription medicines (including vaccines and *in vivo* diagnostic agents[20]) that meet the following criteria:

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16 An orphan drug is a pharmaceutical agent developed to treat medical conditions which, because they are so rare, would not be profitable to produce without government assistance.

17 TGA, *Prescription medicines registration process*, Canberra, August 2021, www.tga.gov.au/prescription-medicines-registration-process , viewed 30 August 2021.

18 Department of Health, *TGA and PBAC parallel process and requirements*, Canberra, December 2020, www.pbs.gov.au/info/publication/factsheets/shared/tga-pbac-parallel-process, viewed 31 August 2021.

19 TGA, *Orphan drug designation*, Canberra, August 2018, www.tga.gov.au/publication/orphan-drug-designation, viewed 28 July 2021.

20 In vivo diagnostic testing is a procedure that is performed in the body to identify a disease or medical condition. Introducing the in vivo diagnostic biological into the body will elicit a response which is observed or measured and determines the result of the test.

- The application is for a new orphan indication (specific therapeutic use), if the medicine is already registered, or is for only one indication, if the medicine is unregistered
- The indication is the treatment, prevention or diagnosis of a life-threatening or seriously debilitating condition
- If the medicine is unregistered, it is not medically plausible that it could treat, prevent or diagnose the condition in any class of patients besides the one included in the application
- It is not likely to be financially viable for the sponsor to market the medicine in Australia unless the fees are waived, or, if the medicine is unregistered, the condition affects fewer than five in 10,000 individuals in Australia (for treatment) or is not likely to be supplied to more than five in 10,000 individuals in Australia (for diagnosis or prevention)
- The medicine has not been refused registration in Australia, the United Kingdom (UK), Canada, the United States (US) or Europe for safety reasons
- There are no therapeutic goods for the treatment, prevention or diagnosis of the condition on the ARTG (unless provisionally registered), or there is substantial evidence that the medicine is significantly safer, more efficacious or better for patient care than the goods that are on the ARTG.[21]

3.12 **Priority review** offers a faster assessment of certain medicines. It is available for prescription medicines that meet four criteria:

- The medicine contains an active ingredient that has not previously been included in an ARTG entry, or does not have the same indications as any medicine on the ARTG
- The medicine treats, prevents or diagnoses a life-threatening or seriously debilitating condition
- There are no therapeutic goods for the treatment, prevention or diagnosis of the condition on the ARTG (unless provisionally registered), or there is substantial evidence that the medicine is significantly safer or more efficacious than the goods that are on the ARTG

[21] TGA, *Orphan drug designation eligibility criteria*, Canberra, April 2021, www.tga.gov.au/publication/orphan-drug-designation-eligibility-criteria , viewed 28 July 2021.

- There is substantial evidence that the medicine represents a major therapeutic advance.[22]

3.13 If a priority review designation is granted the TGA aims to complete its assessment within a target timeframe of 150 working days, which is up to three months faster than the standard timeframe. The assessment itself is as thorough as a standard assessment, and the sponsor must provide a full dossier of evidence.[23]

3.14 **Priority review** is also available for medical devices that meet three criteria:

- The device monitors, treats, prevents or diagnoses a life-threatening or seriously debilitating condition
- There is no device for that purpose on the ARTG or there is substantial evidence that it represents a significant improvement in safety or performance over devices already on the ARTG
- The device is a breakthrough technology and there is evidence that it offers a major clinical advantage over existing technology, or there is evidence that it offers a major clinical advantage over alternatives registered on the ARTG, or if the device is an IVD its early availability will result in a major public health benefit.[24]

If a priority applicant determination is made, the device is granted 'front-of-queue' status through TGA processes, meaning it is top priority.[25]

3.15 **Provisional approval** is available for prescription medicine submissions that meet five criteria:

- The submission is for a new medicine or new indication of an already registered medicine
- The medicine treats a serious condition
- The medicine compares favourably to existing therapeutic goods

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22 TGA, *Priority determination eligibility criteria*, Canberra, April 2021, www.tga.gov.au/publication/priority-determination-eligibility-criteria, viewed 27 July 2021.

23 TGA, *Priority review pathway: prescription medicines*, Canberra, August 2018, www.tga.gov.au/publication/priority-determination-eligibility-criteria, viewed 27 July 2021.

24 TGA, *Priority applicant guidelines for medical devices (including IVDs)*, Canberra, viewed 27 July 2021.

25 TGA, *Priority applicant guidelines for medical devices (including IVDs)*, Canberra, December 2020, www.tga.gov.au/priority-applicant-guidelines-medical-devices-including-ivds, viewed 27 July 2021.

- The medicine represents a major therapeutic advance
- The sponsor provides evidence of a plan to submit comprehensive clinical data on the medicine.

The provisional approval initially lasts for two years, with the possibility of two extensions of two years each. It must then transition to full registration to remain on the ARTG.[26]

3.16 The **Comparable Overseas Regulator (COR)** report-based process shortens the registration timeframe for prescription medicines (including biologicals) using work already done by a COR.[27] The TGA publishes a set of criteria it uses to determine which regulators are CORs; [28] as of August 2021 these were the regulators of Canada, Japan, Singapore, Switzerland, the UK, the US and the European Union.[29] Two COR processes are available:

- **COR-A**: for certain medicines approved less than one year ago by the COR, the sponsor need only provide the COR assessment reports, the proposed Australian label, product information and, if required, a risk management plan. The TGA's timeframe is 120 working days
- **COR-B**: for other medicines, including all approved more than one year ago, the sponsor must also provide some additional data. The timeframe is 175 working days.[30]

3.17 Use of **CORs** is standard for medical devices, with more than 90 per cent of devices approved this way (Class 1 devices, which are the most basic,

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26 TGA, *Provisional approval pathway: prescription medicines*, Canberra, March 2018, www.tga.gov.au/provisional-approval-pathway-prescription-medicines, viewed 24 August 2021.

27 TGA, *Comparable Overseas Regulators (CORs): timeframes and milestones*, Canberra, October 2019, www.tga.gov.au/comparable-overseas-regulators-cors-timeframes-and-milestones, viewed 24 August 2021.

28 TGA, *Comparable Overseas Regulators (CORs) for prescription medicines*, Canberra, October 2019, www.tga.gov.au/comprable-overseas-regulators-cors-prescription-medicines, viewed 24 August 2021.

29 Health Canada, Pharmaceuticals and Medicines Devices Agency, Health Science Authority Singapore, SwissMedic, Medicines and Healthcare Products Regulatory Agency, Food and Drug Administration, and European Medicines Agency: TGA, *List of countries and jurisdictions determined to be Comparable Overseas Regulators (CORs)*, Canberra, October 2019, www.tga.gov.au/list-countries-and-jurisdictions-determined-be-comparable-overseas-regulators-cors, viewed 24 August 2021.

30 TGA, *Comparable Overseas Regulators (CORs): timeframes and milestones*, Canberra, October 2019, www.tga.gov.au/comparable-overseas-regulators-cors-timeframes-and-milestones, viewed 24 August 2021.

excepted).[31] Much as in the case of medicines, there are two options: the TGA will either accept the COR's certification as conformity, or will use the COR's assessment in conducting its own abridged conformity assessment. The list of CORs is similar to the list for medicines, although there are some differences.[32]

3.18 The **Access Consortium** is a coalition of international regulators, which the Committee heard was driven by the TGA.[33] Its other members are Canada, Singapore, Switzerland and, since 1 January 2021, the UK.[34] The Consortium has aligned regulatory approaches and technical requirements.[35] New medicines that are submitted to multiple members of the Consortium are evaluated jointly, such as one member evaluating the clinical aspect of the application and another evaluating the manufacturing aspect. This saves time and effort for the regulators, and simplifies applications for sponsor companies.[36]

3.19 **Project Orbis** is a project of the US Food and Drug Administration (FDA) for new, clinically significant oncology medicines. As well as Australia and the US countries involved include Canada, Singapore, Switzerland and Brazil. The Project aims for medicines to be submitted, reviewed and approved at the same time in the participating countries.[37] In the words of Adjunct Professor John Skerritt, Deputy Secretary, Health Products Regulation, Department of Health,:

> …we don't split the work up. We actually independently evaluate it, but, because the US FDA has so many more resources than everyone else, our

31 Department of Health, Submission 15, p. 37.

32 TGA, *Comparable Overseas Regulators for medical device applications*, Canberra, May 2021, www.tga.gov.au/comparable-overseas-regulators-medical-device-applications, viewed 31 August 2021.

33 Adjunct Prof John Skerritt, Deputy Secretary, Health Products Regulation, Department of Health, *Committee Hansard*, Canberra, 3 September 2020, p. 3.

34 TGA, *Australia-Canada-Singapore-Switzerland- United Kingdom (Access) Consortium*, Canberra, June 2021, www.tga.gov.au/australia-canada-singapore-switzerland-united-kingdom-access-consortium, viewed 26 July 2021.

35 Department of Health, Submission 15, p. 30.

36 Adjunct Prof Skerritt, *Committee Hansard*, Canberra, 3 September 2020, p. 3.

37 Department of Health, Submission 15, p. 31.

doctors are able to engage in conversations, say, with the oncologists at the FDA who have been evaluating the drug.[38]

3.20 Nine medicines were approved through the Project between its launch in mid-2019 and September 2020.[39]

Off-label use of therapeutic goods

3.21 When a therapeutic good is entered on the ARTG, one or more indications, meaning specific therapeutic uses, are included in the entry.[40] The good cannot be marketed for any indication that has not been so included. However a prescriber is permitted to issue prescriptions for any indication her or she sees fit, provided he or she has the patient's informed consent to do so. The use of a therapy for an indication that is not included in its ARTG entry is known as 'off-label' use.[41] Such use is particularly common in the treatment of rare and paediatric diseases.[42]

Access to unapproved therapeutic goods

3.22 There are also a number of ways in which patients can access a therapeutic good that is not on the ARTG. These are:

- **Authorised Prescriber Scheme**: this scheme allows authorised medical practitioners to supply unapproved therapeutic goods for a particular medical condition to a particular class of patients[43]
- **Special Access Scheme (SAS)**: this scheme allows registered health practitioners to access unapproved therapeutic goods for a single patient. There are three SAS pathways:

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38 *Committee Hansard*, Canberra, 3 September 2020, p. 4.

39 Department of Health, Submission 15, p. 31.

40 TGA, *Permitted indications for listed medicines guidance*, Canberra, March 2021, www.tga.gov.au/book-page/permitted-indications-listed-medicines, viewed 27 July 2021.

41 TGA, *Special Access Scheme: frequently asked questions*, Canberra, April 2021, www.tga.gov.au/special-access-scheme-frequently-asked-questions, viewed 27 July 2021.

42 Centre for Law and Genetics, University of Tasmania and Sydney Health Law and Sydney Health Ethics, University of Sydney, Submission *179*, p. [11]; Leukaemia Foundation, Submission 103, p. [6]; Luminesce Alliance, Submission *32*, p. 21.

43 TGA, *Authorised Prescribers*, Canberra, 2021, www.tga.gov.au/form/authorised-prescribers, viewed 22 July 2021.

  - Category A: for a seriously ill patient, a prescribing medical practitioner (or a health practitioner on behalf of a prescribing medical practitioner) can supply the good, then notify the TGA
  - Category B: for a patient who does not meet the Category A definition of 'seriously ill', and who requires a good that does not have an 'established history of use' under Category C, a health practitioner can apply to the TGA for permission to supply the good, providing a clinical justification
  - Category C: certain types of health practitioners can supply specified goods that have an established history of use, then notify the TGA[44]
- **Clinical trials**: these are trials to determine the safety and/or efficacy of a therapeutic good[45]
- **Personal Importation Scheme**: subject to certain conditions, an individual may import an unapproved therapeutic good for his or her personal use or that of his or her immediate family, in a quantity not exceeding three months' supply at any one time[46]
- **Medicine shortages**: special arrangements can be put in place if there is a national shortage of a particular medicine, as indicated by the TGA's medicine shortage reports database[47]

Reimbursement

3.23 The Australian Government has a number of reimbursement programs through which it provides Australians access to reimbursed or subsidised therapeutic goods/and or services. These reimbursement programs include:

- For medicines:
  - the Pharmaceutical Benefits Scheme (PBS)
  - Repatriation Pharmaceutical Benefits Scheme (RPBS), and
  - Life Saving Drugs Program (LSDP).
- For vaccines:
  - the National Immunisation Program (NIP)

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44 TGA, *Special Access Scheme*, Canberra, April 2021, www.tga.gov.au/form/special-access-scheme, viewed 29 July 2021.

45 TGA, *Clinical trials*, Canberra, August 2021, www.tga.gov.au/clinical-trials, viewed 12 October 2021.

46 TGA, *Personal importation scheme*, Canberra, March 2015, www.tga.gov.au/personal-importation-scheme, viewed 22 July 2021.

47 TGA, *Accessing medicines during a shortage*, Canberra, May 2020, www.tga.gov.au/accessing-medicines-during-shortage
, viewed 26 July 2021.

- For devices
  - the Medicare Benefits Schedule (MBS)[48]
  - National Diabetes Supply Scheme (NDSS)
- For blood products:
  - the national blood arrangements (in partnership with state and territory governments).[49]
- For prostheses:
  - the Prostheses List (PL), which stipulates the prostheses that private health insurers must completely cover and the amount of the benefit to be paid.[50]

3.24 The Government determines which therapeutic goods to reimburse through a process known as health technology assessment (HTA). One definition of HTA describes it as:

> The systematic evaluation of properties, effects, and/or impacts of health technology. It is a multidisciplinary process to evaluate the social, economic, organisational and ethical issues of a health intervention or health technology. The main purpose of conducting an assessment is to inform policy decision-making.[51]

3.25 Other countries that conduct HTA in some way include England and Wales, Scotland, Canada, Ireland, France, Belgium, the Netherlands, Sweden, Poland, South Korea and the US.[52] The Australian Government has a number of bodies that conduct HTA, which are discussed below.

Pharmaceutical Benefits Advisory Committee

Role and composition

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48 The MBS does not reimburse devices per se, only services, however when a device is required for a particular service its cost is included in the amount reimbursed: Medical Technology Association of Australia (MTAA), Submission 148, pages 42-44.

49 Pharmacy Guild of Australia, Submission 108, p. 2; MTAA, Submission 148, p. 38; CSL Behring, Submission 145, p. 9

50 Department of Health, *Prostheses List*, Department of Health, Canberra, July 2021, https://www.health.gov.au/resources/publications/prostheses-list, viewed 23 July 2021.

51 Centre for Law and Genetics, University of Tasmania, and Sydney Health Law and Sydney Health Ethics, Sydney University, Submission 179, p. [20].

52 Macquarie University Centre for the Health Economy, Submission 62, p. 6.

3.26 The PBAC is established by the *National Health Act 1953* (Cth).[53] It recommends drugs to the Minister for Health (the Minister) for listing on the PBS and vaccines for inclusion in the NIP. The PBS subsidised 208.5 million prescriptions in 2019-20, highlighting the key role it plays in healthcare. In the 2021-22 Federal Budget, $43 billion was budgeted for the PBS over four years.[54]

3.27 Under the Act, the PBAC must to consist of a Chair and between 11 and 20 other members, including at least one representative from each of the following categories:

- Industry
- Consumers
- Health economists
- Practising community pharmacists
- General practitioners
- Clinical pharmacologists
- Specialists[55]

3.28 As of August 2021 the PBAC was at its full complement of 21 members. The Chair, Professor Andrew Wilson (Prof Wilson), is an epidemiologist and the Deputy Chair, Ms Jo Watson, is a consumer advocate. The other members consist of a psychiatrist, an industry nominee, a nephrologist, a geriatrician and clinical pharmacologist, three medical oncologists, an endocrinologist, a rheumatologist, two haematologists, a health economist, clinical epidemiologist and cognitive neurologist, another consumer advocate, two general practitioners, a community pharmacist, a cardiologist and an infectious diseases expert.[56] The Chair and Deputy Chair gave evidence before the Committee for this inquiry.[57]

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53 s. 100A.

54 The Hon Greg Hunt MP, Minister for Health and Aged Care, and Senator the Hon Richard Colbeck, Minister for Senior Australians and Aged Care Services and Minister for Sport, 'Budget 2021–22: Generational change and record investment in the health of Australians', *Media Release*, 11 May 2021.

55 *National Health Act 1953* (Cth) ss. 100A(2)-(3).

56 Department of Health, *Pharmaceutical Benefits Advisory Committee (PBAC) Membership*, Canberra, July 2021, www.pbs.gov.au/info/industry/listing/participants/pbac , viewed 3 August 2021.

57 See *Committee Hansard*, Canberra, 24 June 2021.

3.29 The PBAC also has two subcommittees, the Drug Utilisation Subcommittee and the Economics Subcommittee. Each subcommittee is chaired by a PBAC member and includes the Chair and Deputy Chair of the PBAC, but most of the rest of their members are not members of the full PBAC. The Drug Utilisation Subcommittee assesses projected usage and financial cost for drugs submitted for reimbursement, and collects and analyses data on actual usage of listed drugs, including in comparison to overseas.[58] The Economics Subcommittee assesses clinical and economic evaluations of medicines submitted for reimbursement, and provides technical advice to the PBAC.[59]

3.30 The PBAC has also developed a non-statutory body called the 'Executive', which consists of the Chair, Deputy Chair and the Chairs of the two subcommittees.[60] Prof Wilson described the purpose of this body as to 'to try and take some of the stuff that could be dealt with, that doesn't require detailed discussion, out of the committee meetings to be dealt with in the executive.'[61]

Process

3.31 The *PBAC Guidelines* provide comprehensive guidance to sponsors on how to submit a product for listing on the PBS or inclusion in the NIP. As of September 2021 these had last been updated in September 2016.[62]

3.32 The full PBAC meets three times per year, usually in March, July and October. A calendar for its meetings is published on its website. The process differs for different types of application, but includes opportunities for pre-submission meetings between the sponsor and the PBAC secretariat, publication of the meeting agenda online and opportunity for consumers to comment on that agenda, the subcommittee meetings and opportunities for the sponsor to provide additional information and to comment on the consumer comments and advice of the subcommittees (and the Australian

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58 Department of Health, *Drug Utilisation Sub Committee (DUSC)*, Canberra, July 2021, www.pbs.gov.au/info/industry/listing/participants/drug-utilisation-subcommittee, viewed 3 August 2021.

59 Department of Health, *Economics Sub Committee*, Canberra, May 2021, www.pbs.gov.au/info/industry/listing/participants/economics-subcommittee-esc,, viewed 3 August 2021.

60 Department of Health, Submission 15.4, p. 6.

61 *Committee Hansard*, Canberra, 24 June 2021, p. 5.

62 Department of Health, *Guidelines for preparing submissions to the Pharmaceutical Benefits Advisory Committee (PBAC)*, Canberra, September 2016, pbac.pbs.gov.au, viewed 30 August 2021.

Technical Advisory Group on Vaccines (ATAGI), in the case of vaccine products). Post-meeting, the meeting minutes are provided to the sponsors, there are opportunities for a meeting with the PBAC and Independent Review of PBAC's decision, and draft Public Summary Documents are provided to the sponsors before being eventually published online.[63]

3.33 There are six categories of submissions for listing on the PBS or NIP. The most complex are Category 1, which involve a first in class medicine or vaccine, a medicine or vaccine for a new population, a drug with a co-dependent technology that requires an integrated co-dependent submission to PBAC and the Medical Services Advisory Committee (MSAC), or a drug or vaccine with a TGA provisional determination. These submissions were the primary focus of this Inquiry, but the other categories range in simplicity all the way up to 'Applications for a new brand of an existing pharmaceutical item', which go straight to the Department of Health and have no PBAC involvement.[64]

3.34 Two important submission pathways for the purposes of this inquiry are:

- The parallel process with the TGA
- The integrated co-dependent submission process.

3.35 The **parallel process** involves consideration of a medicine or vaccine by the PBAC at the same time as the TGA. As discussed above, the TGA's decision effectively trumps PBAC's in the sense that the latter depends on and must accord with the former.[65]

3.36 The integrated co-dependent submission process is available for co-dependent technologies, where one technology must be considered by the PBAC and another by the MSAC. A joint evaluation document is prepared and considered at a joint meeting of the PBAC's Economic Subcommittee and the MSAC's Evaluation Subcommittee. The full PBAC meets three weeks before the full MSAC, 'which gives enough time for the PBAC to raise

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63 Department of Health, *PBS calendars*, Canberra, August 2021, www.pbs.gov.au/info/industry/useful-resources/pbs-calendar, viewed 31 August 2021.

64 Department of Health, *4.1 Types of submissions*, Canberra, www.pbs.gov.au/info/industry/listing/procedure-guidance/4-presubmission-requirements/4-1-types-of-submissions, viewed 31 August 2021.

65 Department of Health, *TGA and PBAC parallel process and requirements*, Canberra, December 2020, www.pbs.gov.au/info/publication/factsheets/shared/tga-pbac-parallel-process, viewed 31 August 2021.

any questions if needed for MSAC consideration, for the applicant to comment on the questions and for the MSAC to consider its advice.'[66]

Life Saving Drug Expert Panel

Role and composition

3.37 The Life Saving Drug (LSDP) Expert Panel considers applications for medicines to be listed on the LSDP. It advises the Commonwealth Chief Medical Officer on such applications, who t advises the Minister. The LSDP has been in operation for over 20 years.

3.38 Members are appointed by the Minister. As of August 2021, the LSDP Expert Panel was chaired by Professor Andrew Roberts, a researcher and clinical haematologist, and former member of the PBAC. Its five other members consist of two clinical experts, a nephrologist and paediatrician, one of whom is also a member of the PBAC and the MSAC, a health economist, industry nominee and consumer nominee.[67]

Process

3.39 To be eligible for listing on the LSDP a medicine must met the following criteria:

- It has been approved by the TGA to treat a disease with a prevalence of 1 in 50,000 people or less (about 500 people or less Australia-wide)
- The disease can be identified 'with reasonable diagnostic precision' and has been shown to reduce life expectancy
- Evidence predicts that use of the medicine will extend the patient's life
- The PBAC has accepted the clinical effectiveness of the medicine but rejected listing it on the PBS for cost effectiveness reasons
- There is no other medicine listed on the PBS or available for public hospital inpatients for life-extending treatment of the disease (there can be such a medicine already listed on the LSDP)
- There is no suitable and cost-effective non-medicine treatment for the condition (such as surgery or radiotherapy)
- The cost of the medicine would be an unreasonable financial burden for the patient or his or her guardian.

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66 Department of Health, Submission 15.6, p. [23].

67 Department of Health, *Life Saving Drugs Program Expert Panel*, Canberra, July 2021, www.health.gov.au/committees-and-groups/life-saving-drugs-program-expert-panel, viewed 24 August 2021.

3.40 The starting point for a LSDP application is the release of the PBAC minutes, advising that the PBAC accepts the clinical effectiveness of the medicine but has rejected it for cost effectiveness reasons. The sponsor must make the LSDP application within four weeks of the publication of those minutes. The LSDP Expert Panel secretariat then takes two weeks to prepare an overview, and publishes an agenda for the Expert Panel meeting four weeks before that meeting. Interested parties such as patients, families and clinicians can then provide their comments on the agenda prior to the hearing. The Expert Panel meet to consider the medicine and hold a stakeholder forum. Two weeks later the Panel sends its advice and a 'consumer summary' to the sponsor. The sponsor has a week to respond.[68]

3.41 Finally, the Chief Medical Officer provides a recommendation to the Minister two to six weeks after the sponsor response, at which point a notification is published online that the recommendation is with the Minister. From the publication of the PBAC minutes to the Minister receiving the recommendation is therefore a total time of 15-19 weeks.[69]

3.42 The Department of Health provided the Committee with a flowchart summarising the LSDP application process.[70]

Jurisdictional Blood Committee

Role and composition

3.43 The Jurisdictional Blood Committee (JBC) 'is responsible for all jurisdictional issues relating to the national blood supply'.[71] It is chaired by a Deputy Secretary of the Commonwealth Department of Health and has nine other members — one other official from that Department and a representative

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68 Department of Health, *Life Saving Drugs Program for medicine sponsors*, Canberra, February 2021, www.health.gov.au/initiatives-and-programs/life-saving-drugs-program/for-medicine-sponsors, viewed 24 August 2021.

69 Department of Health, *Life Saving Drugs Program for medicine sponsors*, Canberra, February 2021, www.health.gov.au/initiatives-and-programs/life-saving-drugs-program/for-medicine-sponsors, viewed 24 August 2021.

70 Department of Health, *Submission 15.6*, p. [16].

71 Department of Health, *Life Saving Drugs Program for medicine sponsors*, Canberra, February 2021, www.health.gov.au/initiatives-and-programs/life-saving-drugs-program/for-medicine-sponsors, viewed 24 August 2021.

from each state and territory.[72] The national blood arrangements supply '…fresh blood components, plasma-derived and recombinant products and diagnostic reagents (blood-related)', administered by the National Blood Authority (NBA), a statutory Commonwealth agency.[73] The products funded are those listed on the National Product Price List, which are two thirds funded by the Commonwealth and one third by the states and territories.[74]

Process

3.44 The sponsor can submit a 'National Blood Supply Change Proposal' to the NBA at any time for a Cycle 1 evaluation, which considers the submission at a high level to determine whether it should be referred to the JBC. There is no timeframe within which it must be evaluated. If more evidence or analysis is required, the product undergoes a Cycle 2 evaluation, which can consider the product's safety, efficacy or cost effectiveness, according to terms of reference developed by the JBC. If still further analysis is required, the product may then be referred to the MSAC - discussed below - for a full evaluation. The MSAC's advice is then considered by the JBC. If the JBC agrees to fund the product, the NBA may then run a competitive tender process for its supply.[75]

Medical Services Advisory Committee

Role and composition

3.45 The MSAC is a non-statutory committee appointed by the Minister that was formed in 1998. It recommends medical services to the Minister for public reimbursement, principally through listing on the Medicare Benefits Schedule (MBS).[76]

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72 National Blood Authority, *Jurisdictional Blood Committee (JBC)*, National Blood Authority, Canberra, undated, www.blood.gov.au/jbc, viewed 9 August 2021.

73 CSL Behring, Submission 145, p. 9.

74 National Blood Authority, *What blood products are supplied—National Product Price List*, Canberra, July 2021, www.blood.gov.au/national-product-price-list, viewed 30 August 2021.

75 CSL Behring, Submission 145, p. 12. CSL Behring refers to a JBC guidelines document for applications, but this does not appear to be available publically.

76 Department of Health, *What is MSAC?*, Canberra, July 2016, http://www.msac.gov.au/internet/msac/publishing.nsf/Content/factsheet-04 , viewed 3 August 2021.

3.46 The MSAC uses a 24 week process and meets three times a year.[77] It also has two subcommittees:

- The ESC (Evaluation Subcommittee) considers the clinical evidence and economic assessment presented in an assessment report in detail, provide advice on the quality, validity and relevance of the assessment, and identify any issues that MSAC will consider, for example, where evidence may be weak
- The PICO (Population, Intervention, Comparator and Outcome) Advisory Subcommittee (PASC) is a 22 week pre-assessment process that is non-compulsory and occurs before a submission is put to the MSAC. It captures any current clinical practice and identifies any impacted healthcare resources.[78]

3.47 As of August 2021, the MSAC consisted of 23 members. It is chaired by Professor Robyn Ward, a medical oncologist, and has two Co-Deputy Chairs, Professor Kwun Fung, a thoracic and sleep physician, and Professor Tim Davis, an endocrinologist. Its remaining members consist of two cardiologists, an academic pharmacist, a rheumatologist, two general practitioners, a nephrologist, a general surgeon, a geneticist and genetic pathologist, two consumer representatives, a pathologist, two health economists, a diagnostic radiographer and nuclear medicine technologist turned health economist, a cardiac anaesthetist, a nuclear medicine specialist and a cardiothoracic surgeon.[79]

3.48 It is through MBS services that many medical devices are reimbursed — that is, the cost of a device is included in the cost of a service — but the Medical Technology Association of Australia (MTAA) noted that 'MBS items frequently incorporate the cost of diagnostic devices but not therapeutic devices' and that there are a variety of other mechanisms through which

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77 Department of Health, *PASC, ESC, MSAC key dates*, Canberra, July 2021, www.msac.gov.au/internet/msac/publishing.nsf/Content/pasc-calendar-key-dates , viewed 31 August 2021.

78 Department of Health, *MSAC and its sub-committees*, Department of Health, Canberra, July 2017, http://www.msac.gov.au/internet/msac/publishing.nsf/Content/Factsheet-05, viewed 20 September 2021.

79 Department of Health, *MSAC membership*, May 2021, Canberra, http://www.msac.gov.au/internet/msac/publishing.nsf/Content/msac-membership , viewed 3 August 2021.

devices are funded.[80] One of these mechanisms, the Prostheses List, is discussed further below.

3.49 In addition to performing HTA for the MBS, the MSAC also provides advice in relation to other forms of funding.[81] Instances of this include assessment of blood products for the national blood arrangements, described above, and the assessment of Highly Specialised Therapeutics jointly funded by the Commonwealth, state and territory governments and delivered in public hospitals.[82] The most discussed example of the latter in this inquiry was CAR-T cell therapy.[83]

Process

3.50 The MSAC encourages engagement between the sponsor and its secretariat prior to the making of an application, which can include a meeting. Once an application is received and accepted as suitable to proceed, the MSAC begins targeted and public consultation. If the application is new it will then proceed to the PASC, which involves the formulation with input from the sponsor of a draft PICO Confirmation, typically by a 'HTA Group' contracted by the Department. Once ratified by the PASC the PICO Confirmation is published online for further public consultation.

3.51 The sponsor can then develop its own assessment report, or the Department can contract an 'HTA Group' to prepare one. If the former option is chosen the Department then contracts an HTA Group to critique the assessment report, with the sponsor being able to see and comment on the critique prior to consideration of the application by the ESC. If the latter option is chosen the sponsor has input into the development of the report, and then can comment on the report prior to consideration of the application by the ESC. The ESC considers the assessment report and prepares the 'ESC report', a copy of which is provided to the sponsor. Some resubmitted applications can skip the PASC and ESC stages.

3.52 The full MSAC considers the ESC report, the sponsor's comments on it, feedback received by MSAC's consultations and other documents. In certain

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80 MTAA, Submission 148, pages 7, 38.

81 Department of Health, *What is MSAC?*, Canberra, July 2016, http://www.msac.gov.au/internet/msac/publishing.nsf/Content/factsheet-04 , viewed 4 August 2021.

82 Department of Health, Submission 15.6, p. [12].

83 Department of Health, Submission 15, p. 11.

circumstances the sponsor may request or be requested to present orally at the MSAC meeting. The MSAC itself does not make a final decision on the application, but rather provides advice to the Minister. A Public Summary Document explaining the rationale for its advice is published on its website sometime after the meeting.[84]

3.53 Like the PBAC, the MSAC has detailed guidelines for applicants, which were updated in May 2021.[85]

Prostheses List Advisory Committee

Role and composition

3.54 The Prostheses List Advisory Committee (PLAC) makes recommendations to the Minister on the listing of devices on the PL and related matters.[86] The PL specifies devices private health insurers must cover (given the fulfilment of certain conditions) and the minimum benefit that must be paid. The regulations specify that the device must be surgically implanted. Therefore, external prostheses such as prosthetic limbs are ineligible for listing, as are certain surgically implanted devices such as diagnostic devices and some cosmetic implants.

3.55 The PL is updated at least three times a year. [87] As of August 2021 the current List was contained in Schedule 1 of the *Private Health Insurance (Prostheses) Rules (No. 2) 2021* (Cth). Rule 12 of those *Rules* makes clear that the Minister can take advice from the PLAC, but is not bound to follow it.

3.56 PLAC members are appointed by the Minister. As of August 2021 the PLAC consisted of its Chair, Professor Terry Campbell AM, a cardiologist, a consumer representative, nine expert members being experts in orthopaedic

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84 Department of Health, *Engaging with MSAC: information for applicants*, Canberra, May 2021, www.msac.gov.au/internet/msac/publishing.nsf/Content/Information-for-Applicants, viewed 31 August 2021.

85 Department of Health, *Guidelines for preparing assessments for the MSAC*, Canberra, May 2021, www.msac.gov.au/internet/msac/publishing.nsf/Content/MSAC-Guidelines, viewed 1 September 2021.

86 Department of Health, *Prostheses List Advisory Committee (PLAC)*, Canberra, December 2020, www.health.gov.au/committees-and-groups/prostheses-list-advisory-committee-plac, viewed 24 August 2021.

87 Department of Health, *Prostheses cover under private health insurance*, Canberra, October 2020, www.health.gov.au/health-topics/private-health-insurance/what-private-health-insurance-covers/prostheses-cover-under-private-health-insurance, viewed 24 August 2021.

surgery, spinal surgery, epidemiology, cardiology, thoracic medicine, bioengineering, vascular medicine, health economics and a representative of the MSAC, five advisory members being representatives of private hospitals, not-for-profit insurers and the device suppliers, and two invited attendees representing device suppliers and private insurers. Its meetings are also attended by representatives of the Department of Health (including the TGA) and Department of Veterans' Affairs.[88]

Process

3.57 The PLAC meets at least three times a year.[89] It has Clinician Advisory Groups (CAGs) for cardiac, cardiothoracic , knee, hip, ophthalmic, spinal, specialist orthopaedic and vascular products, each of which includes a patient representative in addition to expert clinicians, which advise it on the clinical effectiveness of the products it considers. It also has a Panel of Clinical Experts, which assesses products outside the categories for which CAGs have been established. Sponsors are able to comment of the assessment by the CAG or Panel, which is then provided to the PLAC for its final decision.[90] Certain complex applications, such as for devices used in services that are not listed on the MBS, are referred to the MSAC.[91]

Ad hoc

3.58 The Committee also heard that occasionally the Government conducts 'ad hoc' HTA outside the structures described above, for example for a glucose

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88 Department of Health, *Prostheses List Advisory Committee (PLAC)*, December 2020, www.health.gov.au/committees-and-groups/prostheses-list-advisory-committee-plac, viewed 24 August 2021.

89 Department of Health, *Prostheses List Advisory Committee (PLAC)*, Canberra, December 2020, www.health.gov.au/committees-and-groups/prostheses-list-advisory-committee-plac, viewed 31 August 2021.

90 Department of Health, 'Prostheses List – guide to listing and benefits for prostheses', Canberra, February 2017, pages 19-20, 22, www.health.gov.au/sites/default/files/documents/2020/06/prostheses-list-guide.pdf , viewed 12 October 2021.

91 Department of Health, 'Prostheses List Advisory Committee (PLAC) Terms of Reference', Canberra, undated, pages 1-2, www1.health.gov.au/internet/main/publishing.nsf/Content/12D65D189A8D6991CA25816400224C9A/$File/PLAC_Terms-of-Reference.pdf, viewed 12 October 2021.

monitoring system for people with type 1 diabetes in partnership with the supplier.[92]

Participants' understanding of the current system

3.59 One of the major themes to emerge from the evidence received by the Committee was that many of those who rely upon or interact with Australia's current regulatory and reimbursement system struggle to understand it.

3.60 Cystic Fibrosis Australia, the Australian Patient Advocacy Alliance and Lymphoma Australia all submitted that clinicians lack knowledge of the TGA and access options for treatments, and that they should receive education on these issues and the broader HTA process.[93] The two former organisations also wanted to see more 'support, education and updates' for patients with an interest in a product undergoing HTA.[94]

3.61 The grandfather of a girl with cystic fibrosis stated that 'very specific information on the development and assessment of new drugs' is available in the US, but not in Australia, and that patients and carers should be supported and educated through the HTA process.[95] The Patient Voice Initiative likewise suggested that it is difficult for patients to find about what treatments are available and how the system for providing access to new treatments works.[96]

3.62 MS Australia described one of the relevant government websites as 'impenetrable' and recommended that the Government:

> …provide those directly affected – patients and clinicians – with appropriate, clear, accessible publically available information on HTA processes plus updates and feedback throughout the process.[97]

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92 Abbott Diabetes Care, Submission 191, p. 1.

93 Cystic Fibrosis Australia (CFA), Submission 8, p. [2]; Australian Patient Advocacy Alliance (APAA), Submission 67, p. [4]; Lymphoma Australia, Submission 143, pages [2]-[3].

94 CFA, Submission 8, p. [4]; APAA, Submission 67, p. [4].

95 Name withheld, Submission 22, pages [1]-[2].

96 Patient Voice Initiative, Submission 71, p. 1.

97 MS Australia, Submission 85, p. 10.

3.63 On a broader level, XLH Australia suggested that 'additional support and education for advocacy groups would be beneficial to ensure meaningful consultation and collaboration with policymakers.'[98]

3.64 The Metabolic Dietary Disorders Association (MDDA) drew the Committee's attention to action 2.4.3.1 of the National Strategic Action Plan for Rare Diseases:

> Ensure the HTA Consumer Evidence and Engagement Unit provides education and support to people living with a rare disease and their families and carers, and/or rare disease organisations to support them to take a more active role in HTA processes[99]

3.65 Rare Voices Australia (RVA) commended the new Consumer Evidence and Engagement Unit, which sits within the Department, as 'a great initiative', but added that 'more clarity around [HTA] decision-making is vital' and that there is still a major problem with lack of transparency in that regard.[100] Concerns about transparency were also raised by a number of submitters from industry, including Specialised Therapeutics Australia for the PBAC and MSAC and Edwards Lifesciences for the MSAC and PLAC.[101]

3.66 A number of patient organisations went so far as to call for direct financial support from the Government for their work in assisting patients to navigate and participate in the system.

3.67 SCN2A Australia stated that 'funding of rare organisations to offer peer support and education is required so each [organisation] is not reinventing the wheel;'[102] ausEE Inc recommended that the Government recognise and strengthen the role played by rare disease patient organisations by 'providing resources and funding opportunities;'[103] and the CF Pipeline Patient Interest Group proposed 'investment in capacity building for patient groups' to enable them to contribute better to HTA processes.[104]

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98 XLH Australia, Submission 81, p. [1].

99 Metabolic Dietary Disorders Association (MDDA), Submission 109, p. [8].

100 Rare Voices Australia (RVA), Submission 86, p. 10.

101 Specialised Therapeutics Australia, Submission 7, pages 14-15, 17-18; Edwards Lifesciences, Submission 83, p. 35.

102 SCN2A Australia, Submission 127, p. [3].

103 ausEE Inc, Submission 73, p. 5.

104 CF Pipeline Patient Interest Group, Submission 169, p. 3.

3.68 The Committee heard calls for more education for industry, typically focusing on specific features of the system. The PFIC Network, for example, asked the Government to 'raise awareness among industry and rare disease organisations as to the availability of the HTA Access Point'.[105] The Centre for Law and Genetics, University of Tasmania and Sydney Health Law and Sydney Health Ethics, University of Sydney called for 'education of those involved in health technology innovation' in relation to combination products, which are discussed below.[106] Finally, the MTAA recommended education and training for Australian medical technology companies on the TGA's recently introduced priority review option for medical devices.[107]

3.69 The clearest evidence that many participants struggle to understand Australia's regulatory and reimbursement system were the number of submissions and statements in public hearings that proposed changes that the Department has already made. This was highlighted by Adjunct Prof Skerritt of the TGA, who told the Committee in his second appearance:

> Actually, if I could be self-critical, it means that we hadn't reached out enough. We have actually written to all those people, not saying 'Hey, you're wrong,' but saying clearly: 'We haven't communicated enough. Here's some information, and we're happy to meet. Indeed, some of them have already put appointments in the diary to meet in the coming weeks, which is really good.[108]

Gaps in the current system

Combination products

3.70 A combination product is a product 'composed of any combination of a device, medicine and biologic.'[109] A number of terms were used in the evidence to refer to a similar concept, including co-dependent technology, which was described as 'a medical technology or service that relies on another technology to achieve its intended purpose or enhance its effect. '[110]

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105 PFIC Network, Submission 19, p. [2].

106 Centre for Law and Genetics, University of Tasmania and Sydney Health Law and Sydney Health Ethics, University of Sydney, Submission 179, p. [21].

107 MTAA, Submission 148, pages 35, 58.

108 *Committee Hansard*, Canberra, 18 June 2021, p. 15.

109 Roche Australia (Roche), Submission 92, p. 28.

110 Roche, Submission 92, p. 28.

3.71 One submitter commented that under Australia's current regulatory scheme 'combination products are not clearly defined…increased clarity around terminology and regulatory pathways…would be immensely beneficial.'[111]

3.72 Several submitters raised the regulation and reimbursement of combination products as a particular problem for Australia's current system.[112] The Myeloma and Related Diseases Registry noted that the current 'drug reimbursement model was adopted prior to the advent of…the concept of multi-agent or combinatorial treatments.'[113] Medicines Australia explained that use of such combination treatments is increasing and that they are not adequately valued by current HTA processes, but 'recent attempts to examine and resolve this ongoing concern have made little progress.'[114]

3.73 Johnson & Johnson echoed this view, noting that while the PBS currently includes some combination therapies, which were recommended by the PBAC, there are difficulties in listing many others, including its unsuccessful attempt to list daratumumab as a treatment for multiple myeloma in combination with another medicine.[115] UCB Australia gave the example of a combination therapy it has developed for epilepsy, which combines the off-patent drug alprazolam with 'an innovative delivery system', explaining it is concerned that the PBAC will not 'adequately take into account the cost of the ancillary equipment used to deliver the medication. '[116] It urged that 'the value of the device' in a combination therapy should be seen as 'a critical part of the overall effectiveness of the therapy'.[117]

3.74 Amgen Australia submitted that combination therapies pose two major problems, which it described as:

- Value attribution problem: the problem of appropriately attributing value between the multiple sponsors of the components of the combination

111 Centre for Law and Genetics, University of Tasmania, and Sydney Health Law and Sydney Health Ethics, University of Sydney, Submission 179, p. [21].

112 ARCS Australia, Submission 41, p. 5; Sanofi, Submission 99, p. 5.

113 Myeloma and Related Diseases Registry, Submission 12, p. [2].

114 Medicines Australia, Submission 141, p. 12.

115 Johnson & Johnson, Submission 134, pages 8-9.

116 UCB Australia, Submission 74, p. 2.

117 UCB Australia, Submission 74, p. 3.

- Incentive problem: the problem of the listing of a medicine in combination indication lowering the price of existing indications of that medicine, disincentivising combination listings.[118]

3.75 It recommended that the Government 'develop and implement a transparent framework and guidance on the assessment of high cost combination regimens that will solve the key problems limiting patient access.'[119]

3.76 Neuroendocrine Cancer Australia likewise encouraged the development of a 'combined governance framework' for the approval and funding of 'holistic treatments'.[120] It focused particularly on theranostics, a specific category of combination product which consist of two radioactive substances, one diagnostic and one therapeutic, suggesting that the TGA, PBAC and MSAC must 'work together' on the approval of such products.[121]

3.77 Like Amgen Australia, Novartis Australia and New Zealand (Novartis) identified the uncertainty of value determination as a major challenge for reimbursement of combination products, as it deters the sponsor of a therapy that is already listed from cooperating in the combination listing. It proposed three solutions:

- 'A framework for attributing value within the combination'
- 'A means of facilitating…intercompany agreement' without breaching the *Competition and Consumer Act 2010* (Cth) (that is, anti-cartel law)
- 'The ability of companies to have different prices for a therapy within the same indication'.[122]

3.78 Roche Australia stated that 'there are some methodological issues associated with the HTA for co-dependent technologies that make the process unworkable', and suggested that this is a particular problem for genomic panel tests, which test for many genetic mutations simultaneously.[123] This is because of the difficulty of assessing the cost effectiveness of such tests, amongst other challenges.[124] It recommended that the Government 'review how economic evaluations for co-dependent technologies are conducted to

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118 Amgen Australia (Amgen), Submission 82, p. 7.

119 Amgen, Submission 82, p. 7.

120 Neuroendocrine Cancer Australia, Submission 155, p. 13.

121 Neuroendocrine Cancer Australia, Submission 155, pages 13-14.

122 Novartis Australia and New Zealand (Novartis), Submission 138, p. [3].

123 Roche, Submission 92, p. 20.

124 Roche, Submission 92, p. 21.

ensure they are feasible and identify a pragmatic solution to valuing test costs for rare genetic mutation.'[125]

3.79 The Western Australian Department of Health submitted that 'for areas of innovation where there is an interface between drugs and novel therapies such as CAR-T therapy, current assessment pathways…may need to be clarified.' It proposed that this issue be referred to the interjurisdictional working group on HTA elements of the *National Health Reform Agreement*, or alternatively that there be created 'an adjunct, or expert advisory committee…to advise on these kinds of therapies into the future.'[126]

3.80 Bayer Australia and New Zealand identified a more concrete challenge for the assessment of many combination products, namely that the MSAC outcome of a submission of a diagnostic combination component is not available in time for the PBAC's consideration of the therapeutic component, requiring an 'automatic' resubmission of the latter.[127] It recommended 'a revised schedule for co-dependent submissions in which the MSAC advice on the test is finalised before the PBAC meeting.'[128]

3.81 Pathology Technology Australia (PTA) stated that the MSAC is encountering more difficulties than PBAC in assessing companion products, commenting that 'so much so we now see at least two cases where a companion diagnostics product is up before PBAC for a reimbursement rather than MSAC.'[129]

3.82 In contrast to the submissions just discussed, Omico: the Australian Genomic Cancer Medicine Centre, suggested a very different approach to solving the combination product challenge. It submitted that:

> Provision of comprehensive genomic profiling for all Australians with advanced cancers essentially nullifies the majority of co-dependent screening test evaluation, since the population will automatically have access to a test which will identify the subpopulation who will benefit.[130]

Cell and gene therapies

125 Roche, Submission 92, p. 22.

126 Western Australian Department of Health, Submission 129, p. [2].

127 Bayer Australia and New Zealand (Bayer), Submission 175, p. 6.

128 Bayer, Submission 175, p. 7.

129 Pathology Technology Australia (PTA), Submission 178, p. [4].

130 Omico: the Australian Genomic Cancer Medicine Centre (Omico), Submission 184, p. [1].

Funding and pathways

3.83 Many submitters were of the view that current funding and approval pathways for cell and gene therapies are inadequate. AusBiotech focused its concerns on how the TGA approaches such therapies, noting that:

> The current TGA expedited pathways to registration…are available for prescription medicines (which include gene therapies) but not for biologicals (cell and gene-modified cell therapies).
>
> The classification of biologicals, and drug substance versus drug product when it comes to cell and gene therapies, is not clear across international jurisdictions. The definitions affect the compilation of the Common Technical Document (CTD) for registration of a cell-based therapy.[131]

3.84 AusBiotech recommended the creation of 'a dedicated pathway for cell and gene therapies'.[132]

3.85 Medicines Australia noted that biologicals are ineligible for the TGA's priority review and provisional registration, and suggested that this should be changed.[133]

3.86 Better Access Australia noted that 'different evaluation processes and approaches to decision-making are determined by their funding mechanism and treatment setting.' It commented that at the time of making its submission Novartis had two different gene therapies navigating the HTA system, one through the PBAC and one through the MSAC.[134] In its submission Novartis stated that it had 'experienced significant confusion in advice from the Department over the choice of evaluation committee' for one of the therapies.[135]

3.87 RVA highlighted the 'lack of clarity and transparency around approval pathways for gene therapy', citing the consideration of a therapy (apparently the one sponsored by Novartis) by the PBAC, despite it being under the impression from the Department that all such therapies would be evaluated by the MSAC. It explained that it was concerned that the MSAC is

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131 AusBiotech, Submission 114, p. 12.

132 AusBiotech, Submission 114, p. 13.

133 Medicines Australia, Submission 141, p. 18.

134 Better Access Australia, Submission 160, p. 20.

135 Novartis, Submission 138, p. [10].

'likely to have no experience with assessing comparative current therapies, or knowledge of the particular patient cohort.'[136]

3.88 As discussed above PTA also raised concerns about the MSAC's capacity in assessing 'personalised medicine and companion diagnostics'.[137]

3.89 Ms Julia Burlison and the Save Our Sons Duchenne Foundation both endorsed a recommendation from a recent report on Duchenne Muscular Dystrophy and Becker Muscular Dystrophy calling for 'clear funding mechanisms for gene therapies'.[138] PTA argued that funding for genomic testing in particular is 'inadequate and inconsistent';[139] it added that there is no clear pathway for *in vitro* diagnostic devices (a category that includes genomic tests).[140]

3.90 The New South Wales Government stated that 'the diversity of advances in diagnostics, gene and cell therapies and gene editing to date require a simplified and clearly defined approval process' and 'the current regulatory pathways… are not sufficiently flexible to address the range of novel agents and methods of manufacture and delivery that may be involved in novel and personalised therapies.' It recommended 'implementation of alternative regulatory pathways better suited to the bespoke nature of personalised medicine.'[141]

3.91 Pfizer Australia commented that 'the breadth and complexity of [gene therapies] will bring challenges to regulatory and reimbursement processes' and 'the issue remains that there is currently no defined HTA pathway and no defined reimbursement or funding mechanism for some of these innovative treatments and technologies.' It recommended that 'fit-for-purpose…pathways and processes' be established, 'including novel funding sources and payment mechanisms where appropriate.' It also drew attention

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136 RVA, Submission 86, p. 11.

137 PTA, Submission 178, p. 4.

138 Ms Julia Burlison, Submission 5, p. 2; S Save Our Sons Duchenne Foundation, Submission 33, pages 14-32, citing A Jackson and Equity Economics, 'Living with Duchenne & Becker in Australia: supporting families waiting for a cure', McKell Foundation, Sydney, April 2020, cdn.shopify.com/s/files/1/0506/8367/4813/files/McKell_Institute_-_Equity_Economics_-_Report_into_Duchenne_and_Becker_-_SOSDF_-_Final_Version_PDF.pdf?v=1614568181, viewed 12 October 2021.

139 PTA, Submission 178, p. [4].

140 PTA, Submission 178, p. [2].

141 New South Wales Government, Submission 93, pages 6, 19.

to the problem that gene therapies often have long term benefits but there may be limited long term data available at the time of assessment, which it recommended solving by allowing patients access to treatment while simultaneously collected longer term real world evidence.[142]

3.92 The mother of a young man with Duchenne Muscular Dystrophy submitted that 'consideration must be given to the individuality of genetic therapy, that this technology be assessed differently to drug therapy, making the overall journey cheaper'.[143] The Queensland Genomics Community Advisory Group and Duchenne Australia both emphasised the need for Australians to have faster access to gene therapies that become available overseas.[144]

Genomic testing

3.93 Another argument made by many submitters was that there needs to be greater government-funded provision of genomic testing.[145] Many suggested that this should be provided at a national level, which would mean the same tests in all states and territories.[146] Support was particularly strong from patient groups such as the Australian Pompe Association, which submitted that 'without neonatal testing in Victoria alone, three babies have been lost in the last 14 months to Pompe because the disease was not diagnosed fast enough for treatment to be initiated or was started far too late.'[147] Other patient groups that advocated for increased testing for their respective conditions included Myeloproliferative Neoplasms Alliance Australia, Spinal Muscular Atrophy Australia (for newborns), MND Australia, the Leukaemia Foundation, Rare Cancers Australia, Rare Ovarian Cancer and the FSHD Global Research Foundation (including prenatal testing).[148]

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142 Pfizer Australia, Submission 137, pages [4]-[5].

143 Name withheld, Submission 131, p. [1].

144 Queensland Genomics Community Advisory Group, Submission 44, p. 1; Duchenne Australia, Submission 77, p. 2.

145 Myeloma and Related Diseases Registry, Submission 12, pages [2]-[3]; Name withheld, Submission 48, p. [1]; Better Access Australia, Submission 160, p. 5.

146 Victorian Comprehensive Cancer Centre, Submission 61, p. 2; Amgen, Submission 82, p. 6; Australasian Society of Clinical Immunology and Allergy (ASCIA), Submission 147, p. 5; Omico, Submission 184, p. [1].

147 Australian Pompe Association, Submission 26, pages 2-3.

148 Myeloproliferative Neoplasms Alliance Australia, Submission 11, pages 5-6; Spinal Muscular Atrophy Australia, Submission 37, p. [1]; MND Australia, Submission 64, p. 4; Leukaemia

3.94 MND Australia, Research Australia, the Australasian Society of Clinical Immunology and Allergy (ASCIA) and the FSHD Global Research Foundation all argued that expanded testing itself is insufficient, but must also be accompanied by adequate 'genetic counselling'.[149]

3.95 Research Australia, MDAA, the Prader-Willi Research Foundation Australia and the Foundation for Angelman Syndrome Therapeutics Australia all endorsed Action 2.4.1.2 of the *National Strategic Action Plan for Rare Diseases*:

> Align with and build on the existing *National Health Genomics Policy Framework* for the systematic, equitable and timely delivery of genomic services such as genetic testing (diagnostics) and gene therapies (treatments) and genomic counselling to Australians with, suspected of having, or with an increased chance of a rare disease.[150]

3.96 MND Australia and the ASCIA also supported increased provision of genomic counselling in more general terms.[151]

Other issues

3.97 PTA did not mention the *National Health Genomics Policy Framework*, but submitted that Australia currently has 'no comprehensive genomics policy' and needs such a policy to guide 'the end to end applications of genomics in healthcare, from screening to diagnostics, to therapeutics and monitoring.'[152] It also commented that Australia's 'framework for capture, storage and use of digital genomic data is fragmented across state-based and commercial databases' and that consideration needs to be given to 'establishing a secure service for storing and sharing genomic data' and a 'clear protocol for data interchange.'[153]

3.98 Roche Australia put forward a proposal for a 'national genomic service to bring research and clinical practice together within a quality framework and

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Foundation, Submission 103, pages [5], [8]; Rare Cancers Australia, Submission 166, p. [2]; Rare Ovarian Cancer, Submission 167, p. [2]; FSHD Global Research Foundation, Submission 200, pages 5, 7.

149 MND Australia, Submission 64, p. 4; Research Australia, Submission 78, p. 6; ASCIA, Submission 147, p. 5; FSHD Global Research Foundation, Submission 200, p. [2].

150 Research Australia, Submission 78, p. 6; MDDA, Submission 109, p. [2]; Submission 110, p. 5; Submission 153, p. [3].

151 MND Australia, Submission 64, p. 3; ASCIA, Submission 147, p. 5.

152 PTA, Submission 178, pages [4], [7].

153 PTA, Submission 178, pages [4], [5].

generate the evidence to support applications for repurposing medicines in rare diseases and cancers.' It proposed that the service would provide testing and treatment (when possible) to patients, collecting 'structured data' for research purposes and to support regulatory and reimbursement applications for repurposed treatments. It argued that the service would also educate patients and the health workforce, and suggested it should initially focus on rare diseases and cancer. It noted that the National Health Service (NHS) England established such a service in 2018.[154]

3.99 The Gene Therapy Advisory Steering Group, Sydney Children's Hospital Network described the 'Gene Therapy Assessment Tool' it has developed to 'provide a framework with which to assess the merits of a gene therapy for clinical testing.' It urged that an 'evidence-based and clearly defined set of criteria' such as its Tool be adopted for this purpose. It also recommended that more use be made of 'state-based panels of experts' such as the Steering Group in the approval processes for gene therapies, and that the Government 'fund two or three state-based gene therapy trials with adjunct infrastructure to demonstrate a proof of principle approach to approve gene therapy.'[155]

3.100 Medicines Australia submitted that:

> …new types of medicines require specific expertise, infrastructure or aligned processes to achieve access. Examples include those in the cell and gene therapy space, where large overseas biotechnology companies without a presence in Australia experience barriers to entering this market, or delay filing registration due to uncertainty or factors related to the small size of the Australian market.[156]

Blood products

3.101 A small number of submitters discussed the position of the national blood arrangements in the current system. Their views summed up by CSL Behring's statement that 'the current funding appraisal process for new blood and blood-related products can be characterised as complex, uncertain, and at times repetitive.'[157]

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154 Roche, Submission 92, pages 25-27.

155 Gene Therapy Advisory Steering Group, Sydney Children's Hospital Network, Submission 102, pages [1]-[2].

156 Medicines Australia, Submission 141, p. 12.

157 CSL Behring, Submission 145, p. 1.

3.102 Sanofi made two recommendations in this regard: introduce approval timelines and increase transparency; and review the current process.[158]

3.103 AusBiotech submitted that access to new blood products is inferior to access to new medicines and medical technologies. It based that claim on the fact that approval of new blood products for reimbursement takes 'significantly longer' than for medicines, and the fact that there is no Government commitment to funding new blood products, with funding instead being reliant on there being capacity within the National Blood Agreement budget.

3.104 It made two broad recommendations, which largely aligned with Sanofi's: introduce statutory timelines, an appraisal cycle, assessment performance measures and parallel registration and reimbursement; and reform the blood products process in keeping with reform in approvals for other therapeutic products.[159]

3.105 The Haemophilia Foundation Australia (HFA) made a comprehensive submission on this topic, supporting retention of the current system (with significant reforms) and discouraging any move to incorporate blood products into the PBS.[160] Many of the issues it touched on such as patient involvement and assessment of cost effectiveness were equally applicable to other categories of therapeutic products, and accordingly are considered in later chapters of this report. Its recommendations that were uniquely relevant to blood products included expanding the objectives of the National Blood Agreement to recognise the importance of innovation, a review of the reimbursement process for new bleeding disorder therapies, inclusion of a haematologist on the MSAC's PICO Subcommittee, setting timelines for assessment of blood products, and introduction of parallel TGA and MSAC processing of blood products.[161]

3.106 CSL Behring's submission focused on blood products. Like the HFA, it emphasised that 'plasma-derived products are a unique category of specialised therapies that require a bespoke HTA approach', and that they are mostly used in treating rare diseases which brings further challenges as discussed throughout this report.[162]

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158 Sanofi, Submission 99, p. 5.

159 AusBiotech, Submission 114, pages 15-16.

160 Haemophilia Foundation Australia (HFA), Submission 119, p. 5.

161 HFA, Submission 119, pages 1-2.

162 CSL Behring, Submission 145, p. 6.

3.107 CSL Behring made a number of recommendations for improvements to the system, including: governments committing to fund access to new blood products within six months of a sponsor accepting a positive recommendation; devolution of the JBC's HTA role to an independent expert committee; provision of a 'clearly documented process', including publication of guidance documents and an 'appraisal cycle calendar'; development and implementation of policies for rare disease treatments; allowing parallel registration and reimbursement processing; creation of 'a web portal for consumer comments'; and development and application of Key Performance Indicators for the blood product HTA process.[163]

Committee Comment

3.108 Over the course of the inquiry it became apparent to the Committee just how complex Australia's regulatory and reimbursement system is. The Committee appreciates that a high level of complexity is necessary given the broad range of medicines and technologies the system must cover and the difficult and complex nature of the many of the decisions it must make.

3.109 If the Committee recommended every change suggested over the course of the inquiry and those recommendations were adopted the system would become considerably more complex, and potentially unworkable. Therefore the Committee has endeavoured to keep simplicity of the system front of mind in all its recommendations in this report. The Committee is supportive of the key measure in the *Strategic Agreement 2022-2027* between Medicines Australia and the Australian Government that proposes a full independent review of the HTA process starting in July 2022.

3.110 The Committee acknowledges the hard work of the Department of Health and its staff in making the system more comprehensible to patients and the general public, particularly in the case of the TGA in the face of the unprecedented pressure of the COVID-19 pandemic. Nonetheless, the Committee believes that the publically available information about the regulatory and reimbursement system, on the Department of Health's website, is still largely targeted at experienced industry members and their consultants. The Committee believes improvements should be made to the Department of Health's websites to explain the regulatory and reimbursement system.

163 CSL Behring, Submission 145, pages 1-2.

3.111 The Committee sympathises with MS Australia when it describes the Department of Health's website as 'impenetrable.'[164] While it is necessary for the TGA and the Health Technology Assessment (HTA) websites to include detailed technical information for applicants, the Committee believes that the Department should also include plain English explanations of the TGA and HTA processes on their websites for the benefit of the patients and families, who depend on the medicines and medical devices.

3.112 The Committee believes that the creation of the Department's HTA Consumer Evidence and Engagement Unit was a significant step in the right direction in terms of engaging with patients, and was impressed by the TGA's efforts to reach out to submitters to this inquiry to educate them about its work. It is the Committee's view that education and engagement is an area that needs continual enhancement from the Department of Health. The Committee emphasises that while the Department of Health should do all that it can to better educate and engage with industry and clinicians, these groups need to continue to keep informed of how the system works. The Committee believes more resourcing from the Australian Government either directly to patient groups or through education programs is required.

3.113 For combination products, the Committee believes that the current system is well adapted to assessing some products, particularly where both products have the same sponsor and are submitted at the same time. The system struggles with products from different sponsors submitted at different times. The Committee recognises that medical innovations in health care are progressing rapidly and Australia's HTA systems must adapt quickly to provide an agile assessment system. Therefore the Committee recommends a review of the HTA system to streamline the assessment of combination products, particularly combination products with different sponsors.

3.114 The national blood arrangements appear to be something of an anomaly within the current system. The Committee believes that this added complexity of the reimbursement and HTA system should be reviewed as part of the independent review in July 2022, as proposed in the *Strategic Agreement 2022-27*. The Committee believes that all reforms made to the broader HTA system should be applied to the national blood arrangements, so that the patients who depend upon them are not disadvantaged compared to patients of other diseases.

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164 MS Australia, Submission 85, p. 10.

4. The Patient Voice

Overview

The concept of 'the patient voice'

4.1 The Committee's inquiry attracted strong interest from patients, their families and advocacy organisations.[1] They offered many suggestions for improving Australia's current regulatory and reimbursement system, covering a wide range of issues, but the most dominant theme to emerge from their evidence was the importance of 'the patient voice'. No exact definition of this concept was offered to the Committee, but when asked whether the current system 'recruits it', Ms Deidre Mackechnie, Executive Officer, Australian Patient Advocacy Alliance (APAA), replied:

> I think it recruits a patient voice; I don't think it recruits the patient voice. There is certainly an attempt—and that sounds a weaker word than it probably should—by the department to actually consider the perspective of people who are affected by the healthcare system. But often they are—again, for want of a better term—vanilla patients. They often don't include early on in the process, in the design of what they're actually looking at, patients who are specifically affected by that condition. I think that's a real opportunity to actually improve the system, whereby we can include someone who is directly

[1] The terms 'patient' and 'consumer' were both used throughout the inquiry, apparently with the same meaning, and indeed some submitters used both interchangeably: Lymphoma Australia, Submission 143, p. [4]; Consumers Health Forum of Australia (CHF), Submission 205, p. 9. The term 'patient' is preferred in this report, but references to 'consumers' should be read as having the same meaning.

> affected, either as a patient or as a carer or parent, so that they are able to more meaningfully contribute to the process.[2]

4.2 The importance of family and carers noted by Ms Mackechnie was emphasised throughout the inquiry. Many submitters were patients themselves, such as Ms Fiona Mobbs and Ms Patricia Pontynen, who wrote to the Committee as sufferers of Type 1 Narcolepsy and Non Small-Cell Lung Carcinoma, a form of lung cancer, respectively.[3] However the Committee heard from many parents and carers of patients who are unable to speak for themselves, typically because they are too young or too affected by their illness. These advocates included Dr Elizabeth Patterson, who appeared before the Committee as the mother of an adult son with Prader-Willi Syndrome, and Ms Michelle and Mr Eliot Jones, who wrote on behalf of their eight year old son Joshua, one of the many boys with Duchenne Muscular Dystrophy whose parents submitted to the inquiry.[4]

4.3 Patients were keen to emphasise how different their voice is from that of other key parties to the regulatory and reimbursement system such as government, sponsor companies and clinicians, and how important that difference makes it for their voice to be included properly in the system. Mr Mike Wilson, the Chief Executive Officer of JDRF Australia, a Type 1 Diabetes group, told the Committee:

> The patient voice is of course one that is important, but it is also under recognised in most of our systems and structures in Australia today. It is not the same as a professional voice or a manufacturer voice, but that is its benefit. … A patient's assessment of risk is not the same as that of a regulator. It should be informed by a doctor, but it is also informed by the ultimate need of the individual. I can assure you a patient's assessment of urgency is very different to that of bodies assessing a line-up of drugs and devices awaiting their attention.[5]

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2 *Committee Hansard*, Melbourne, 22 April 2021, pages 3-4.

3 Ms Fiona Mobbs, Submission 38, p. [2]; Ms Patricia Pontynen, Submission 60, p. 3.

4 Prader-Willi Research Foundation Australia (PWRFA), *Committee Hansard*, Melbourne, 22 April 2021, pages 10-11; Ms Michelle and Mr Eliot Jones, Submission 132.

5 *Committee Hansard*, Sydney, 11 March 2021, p. 23.

4.4 The Committee heard from the Patient Voice Initiative, which describes itself as 'a multidisciplinary collaboration which advocates for a greater patient voice in health policy.'[6] It submitted that:

> …researchers and policy-makers overlook critical issues when striving to improve health outcomes because they lack essential contextual knowledge which patients gain from living with a condition or using a treatment. This includes:
>
> - Outcomes that are important to patients
> - Benefits not documented in traditional evidence, including non-health benefits
> - Risks and adverse events not documented in traditional evidence, including non-health risks
> - Knowledge of service variation (especially what really happens as opposed to what is meant to happen), often crucially important for people outside of our capital cities
> - Knowledge of why some patients cannot access existing drugs and services
> - Knowledge of unmet needs
> - Knowledge of wider societal consequences.[7]

4.5 Patients insisted that, far from being confined to any one particular stage of the regulatory and reimbursement process, the patient voice must be included throughout the entire system.[8]

The patient voice and the Therapeutic Goods Administration

Current patient input into Therapeutic Goods Administration decision-making

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6 Patient Voice Initiative, Submission 71, p. 1.

7 Patient Voice Initiative, Submission 71, p. 2.

8 Name withheld, Submission 22, p. [2]. Queensland Genomics Community Advisory Group, Submission 44, p.2; GUARD Collaborative (GUARD), Submission 46, p. 2; MND Australia, Submission 64, pages 7-8; XLH Australia, Submission 81,p. [1]; Rare Voices Australia (RVA), Submission 86, p. 4; Metabolic Dietary Disorders Association (MDDA), Submission 109, p. [7]; PWRFA, Submission 110, p. [4]; Lymphoma Australia, Submission 143, p. [1]; Juvenile Arthritis Foundation Australia (JAFA), Submission 154, p. [3]; FSHD Global Research Foundation, Submission 200, p. 5.

4.6 Adjunct Professor John Skerritt, Deputy Secretary, Health Products Regulation, Department of Health (Adjunct Prof Skerritt), who leads the Therapeutic Goods Administration (TGA), said of the role of the patient voice in TGA decision-making that 'I think that is an area we need to do more in.'[9] He stated that the most important role patient input can play in the TGA's decisions is through the inclusion of patient-reported outcomes. These enable the TGA to assess the impact a medicine or device has on patients' quality of life. According to Adjunct Prof Skerritt such outcomes are often more difficult to measure than more traditional clinical trial outcomes, but this difficulty will be minimised in the future as there is a 'global trend' towards including such outcomes.[10]

4.7 Adjunct Prof Skerritt explained that patients have a more direct voice in the TGA's activities through its advisory committees. These committees consider most new drugs and many new devices as part of their registration processes, and include consumer representatives.[11] Since a 2017 reorganisation there are seven such committees, one each for biologicals, chemical scheduling, complementary medicines, medical devices, medicines, medicines scheduling and vaccines.[12] Since March 2019, the consumer representatives from the Advisory Committees on Medicines and Medical Devices have served as members of the Department of Health's Health Technology Assessment (HTA) Consumer Consultative Committee alongside the consumer representatives from the Pharmaceutical Benefits Advisory Committee (PBAC), Medical Services Advisory Committee (MSAC) and Prostheses List Advisory Committee (PLAC).[13] Adjunct Prof Skerritt noted that the membership of the TGA's advisory committees is term-limited, and the TGA conducts call-outs for new members, including engagement with consumer groups.[14]

4.8 A final important role that patients already play in the regulation of therapeutic goods is through adverse event reporting, meaning reporting

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9 *Committee Hansard*, Canberra, 18 June 2021, p. 28.

10 *Committee Hansard*, Canberra, 18 June 2021, p. 29.

11 *Committee Hansard*, Canberra, 18 June 2021, p. 28.

12 Therapeutic Goods Administration (TGA), *TGA Statutory Advisory Committees*, Canberra, July 2021, www.tga.gov.au/tga-statutory-advisory-committees, viewed 12 October 2021.

13 Department of Health, Submission 15, p. 28.

14 *Committee Hansard*, Canberra, 18 June 2021, p. 28.

problems with already approved medicines and devices to the TGA.[15] The Department of Health (the Department) explained that in January 2018 the TGA introduced the Black Triangle scheme, which provides information about how patients can report adverse events on product labels, for medicines for which 'use in the general population is yet to be fully characterised.'[16]

4.9 Adjunct Prof Skerritt told the Committee how he and other TGA officials had met with a group of women who were suffering from a rare cancer linked to TGA-approved breast implants. He explained that:

> We were working with them on how we could incorporate their voice, and we have a whole program, known as the medical devices action plan, that gives the patient voice a much a much greater input….What we wanted and what we are achieving…was to make sure of our communications for patients around what to do….How do we shape our communications? You don't want to write in regulator-speak or bureaucrat-speak. Increasingly, we're sitting across the table and they're actually shaping the communications.[17]

Patient views on the Therapeutic Goods Administration

The Therapeutic Good Administration's engagement with patients and patient evidence

4.10 Some patient groups were complimentary about the TGA. Migraine Australia commented that the 'the TGA process appears to work well and have a high level of transparency and trust';[18] APAA wrote that 'our regulatory process (TGA and PBAC) is robust and trustworthy,' a sentiment echoed by Cystic Fibrosis Australia (CFA);[19] and Rare Voices Australia (RVA) said that it 'would like to acknowledge the strengths of the Therapeutic Goods Administration and Australia's [HTA] approval processes.'[20] Nonetheless all three of these groups, along with many other

15 Department of Health, Submission 15, pages 36-37.

16 Department of Health, Submission 15, p. 34.

17 Adjunct Prof Skerritt, *Committee Hansard*, Canberra, 18 June 2021, p. 29.

18 Migraine Australia, Submission 24, p. 16.

19 Australian Patient Advocacy Alliance (APAA), Submission 67, p. [1]; Cystic Fibrosis Australia (CFA), Submission 8, p. [5].

20 RVA, Submission 86, p. 1.

groups and individual patients, had suggested improvements to how the TGA functions.

4.11 Patients were clearly of the view that they need more of a voice in the TGA's activities. CFA stated that there is 'no consumer consultation at the TGA stage or prior', stating that 'consumers must be part of the registration process.'[21] The GUARD Collaborative (GUARD), a coalition of genetic, undiagnosed and rare disease organisations, called for 'dialogue at a very early stage, on a specific disease, in a multi-stakeholder format including patient representatives, rare disease clinicians, regulators, HTA experts and industry…' to consider a wide variety of issues, and for 'patient organisations [to] be supported to create Community Advisory Boards composed of trained patient advocates, per disease or group of diseases, in order to enable a structured, high quality, and transparent dialogue with all stakeholders.'[22]

4.12 APAA commented that 'there is a lack of inclusion of consumers and consumer organisations at all steps in the HTA process,' and that 'there are few patient-specific measures included in evaluation.' It proposed creating 'a consultative mechanism to co-design process improvements' to increase engagement, and 'inclusion of patient measures…in the process'.[23] The Consumers Health Forum of Australia (CHF) stated that 'working collaboratively with consumers and consumer organisations to access and understand real world data around co-design, disease-specific, patient relevant/patient-reported health outcomes (PROMs) and patient-reported experience measures (PREMs), quality of life and patient preference data, must be included as part of the…regulatory…clinical assessments.'[24] The CF Patient Pipeline Interest Group asked that the Pharmaceutical Benefits Scheme (PBS) Medicines Status website, discussed below, be expanded to include TGA information.[25]

Patient comments on other Therapeutic Goods Administration issues

Use of Overseas Regulators

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21 CFA, Submission 8, p. [2].

22 GUARD, Submission 46, pages 11-12.

23 APAA, Submission 67, p. [1].

24 CHF, Submission 205, p. 7.

25 CF Pipeline Patient Interest Group, Submission 169, p. 3.

4.13 Beyond the issues of engagement with patients and patient evidence, a number of broad themes emerged from patients' commentary on the TGA. The most popular of these was the need for the TGA to rely more on the work of overseas regulators, or engage in more collaboration and harmonisation with them. Many submitters kept their comments on this issue to the general proposition that this would increase the speed of Australia's regulatory process and/or result in more products being registered.[26]

4.14 Allergy and Anaphylaxis Australia (A&AA) submitted that the regulators in question should only be those of 'countries Australia has trusted relationships with',[27] while other submitters proposed: those regulators already designated Comparable Overseas Regulators (CORs) by the TGA;[28] the European Union's European Medicines Agency (EMA) and the United States (US) Food and Drugs Administration (FDA);[29] or just the FDA.[30] The Australian Federation of AIDS Organisations (AFAO) proposed that the TGA should make use of assessment reports from CORs for diseases 'with low prevalence among the general population'.[31] MND Australia suggested that programs similar to Project Orbis be developed for rare diseases, while Ms Pontynen supported referring to overseas regulators when adjusting a product's indication after it has been registered.[32]

Length of review

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26 Alpha-1 Organisation Australia (A1OA), Submission 29, p. 5; Sanfilippo Children's Foundation, Submission 36, p. [2]; JDRF Australia, Submission 52, p.[3]; Mrs Melissa Jose, Submission 54, p. [1]; APAA, Submission 67, pages [3]-[4]; SCN2A Australia, Submission 127, p. [2]; Mr and Ms Jones, Submission 132, p. [5]; Ovarian Cancer Australia (OCA), Submission 135, p. [4]; JAFA, Submission154, p. 4; FSHD Global Research Foundation, Submission 200, p. 5; CHF, Submission 205, pages 10-11.

27 Allergy and Anaphylaxis Australia (A&AA), Submission 128, p. 6.

28 Eczema Support Australia, Submission 39, p. 2; National Allergy Strategy, Submission 156, p. [4]. For more on the CORs see above Chapter 3.

29 Migraine Australia, Submission 24, p. 16; A1OA, Submission 29, p. 5; Spinal Muscular Atrophy Association of Australia (SMA Australia), Submission 37, p. [2]; Narcolepsy Australia, Submission 55, p. 4; Duchenne Australia, Submission 77, p. 3; CF Pipeline Patient Interest Group, Submission 169, p. 3.

30 Save Our Sons Duchenne Foundation (SOSDF), Submission 33, p. 17.

31 Australian Federation of AIDS Organisations (AFAO), Submission 196, p. 4.

32 MND Australia, Submission 64, p. 8; Ms Pontynen, Submission 60, p. 2.

4.15 Another common refrain – reflected in many of the calls for more use of international regulatory work - was the need for TGA process to occur faster. Fabry Australia highlighted the importance of fast registration for patients with chronic progressive conditions, and suggested that current timeframes are not fast enough.[33] Migraine Australia submitted that registration 'could be faster', and the CF Pipeline Patient Interest Group made a similar point, noting that TGA registration times are slower than the FDA's and EMA's.[34]

Off-Label use

4.16 Several patient groups also raised concerns about the reliance currently placed on off-label use of medicines in treatment of certain conditions. The Leukaemia Foundation noted that there are no definitive statistics on off-label usage – which is one of the problems with such usage from a system-wide perspective - but that it appears to be common in treatment of cancers, especially blood cancers.[35] The Foundation proposed a 'Right to Trial' program to provide 'a mechanism for the more regular and systematic use and evaluation of off-label medicines'.[36] In a similar vein, CHF suggested consideration of 'right to trial', a US concept whereby terminally ill patients are allowed access to therapeutic goods that have completed Phase 1 trials but not yet received regulatory approval.[37]

4.17 Rare Ovarian Cancer expressed concern about how common off-label use is in the treatment of rare cancers, since it means these medicines are not being funded by the Government and so are 'inaccessible to most patients'.[38] RVA argued that there are two other problems with ongoing off-label usage: it relies on a prescriber 'who has an understanding of the rare condition and the benefits of off-label use' and it often relies on hospital funding which is by individual application, so there is no ongoing certainty for the patient.[39]

Post-market surveillance

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33 Fabry Australia, Submission 4, p. [2].

34 Migraine Australia, Submission 24, p. 16.

35 Leukaemia Foundation, Submission 103, p. [6].

36 Leukaemia Foundation, Submission 103, p. [9].

37 CHF, Submission 205, p. 11.

38 Rare Ovarian Cancer, Submission 167, p. [2].

39 RVA, Submission 86, p. 12.

4.18 Varying views were expressed concerning the TGA's post-market surveillance. Migraine Australia commented that 'there seems to be a very low rate of reporting of side effects and adverse events to the TGA, and perhaps that reporting process could be made simpler and more consumer friendly.'[40]

4.19 AFAO expressed concern that 'overregulation and the costs incurred with random and unexpected monitoring can act as a disincentive for manufacturers to enter the Australian market.'[41] It urged the TGA to 'strike a balance between conducting essential post market monitoring and assessments of approved devices and creating an environment that encourages innovation'.[42]

Miscellaneous patient comments on the Therapeutic Goods Administration

4.20 In addition to the general concerns with the TGA just discussed, some patients and patient groups had more varied comments. The CF Pipeline Patient Interest Group recommended that the process be changed to allow 'data to be added during the TGA process,' thereby potentially allowing for indications to be expanded without requiring further applications to the TGA.[43] RVA suggested that 'all rare disease applications should be routinely flagged as complex and may require additional scoping and stakeholder engagement to address potential challenges and uncertainties,' a comment that ties into the discussion of patient engagement above.[44]

4.21 There were a number of suggestions that the TGA should copy initiatives of the US FDA, including establishing a Priority Review Voucher system, and producing guidance for industry for developing drugs for the rare disease Eosinophilic Esophagitis (EoE).[45] The Priority Review Voucher system involves the FDA rewarding a company that has secured approval of a treatment for certain rare diseases with a priority review voucher that can be

40 Migraine Australia, Submission 24, p.16.

41 AFAO, Submission 196, p. 5.

42 AFAO, Submission 196, p. 6.

43 CF Pipeline Patient Interest Group, Submission 169, p. 1.

44 RVA, Submission 86, p. 9.

45 Fragile X Association of Australia (FXAA) Submission 159, p. 2; ausEE Inc., Submission 73, p. 4.

used to access priority review for a drug that would not normally be eligible for it.[46]

4.22 Ovarian Cancer Australia (OCA) called for the introduction of a 'fast track short-term approval, with subsequent full review' process, while the AFAO proposed 'a priority track for the registration of medicines'.[47] AFAO recommended changing the TGA's regulation of advertising 'to accommodate health promotion campaigns by non-government organisations', specifically to enable it to promote HIV testing.[48]

4.23 Lymphoma Australia raised the problem of pharmaceutical companies being unwilling to submit a medicine to the TGA for registration if it will not be reimbursed, even though it may be registered in other countries.[49] It recommended that this be dealt with through the creation of 'a pathway for registration…that can also be clinician/research [sic] or patient-initiated'.[50] This issue forms part of the broader question of the regulatory and reimbursement system's reliance on sponsor companies. Lymphoma Australia commented that many clinicians are unaware of the Special Access Scheme - which allows access to unregistered medicines – and recommended that they be educated about such matters.[51]

The patient voice and the Pharmaceutical Benefits Advisory Committee

Current patient input into Pharmaceutical Benefits Advisory Committee decision-making

4.24 In its submissions to the inquiry, the Department stated that the mechanisms through which it engages with patients and stakeholders include:

- Stakeholder consultation to facilitate access and engagement of specialist clinicians, patient networks, research bodies, registries and international contacts to enable contribution of rare disease expertise

46 FXAA. Submission 159, p. 2.

47 OCA, Submission 135, p. 4; AFAO, Submission 196, p. 3.

48 AFAO, Submission 196, pages 3-4.

49 Lymphoma Australia, Submission 143, p. [2].

50 Lymphoma Australia, Submission 143, p. [3].

51 Lymphoma Australia, Submission 143, p. [3].

- Inputs to submissions through written submissions, consumer hearings, stakeholder meetings, patient/family interview and organisational surveys, for consideration by the committee.[52]

4.25 The Department outlined the work being done by the recently established HTA Consumer Consultative Committee, made up of the consumer representatives from across the regulatory and reimbursement system, including holding workshops and fora for patient organisations, assisting in the development of the Medicines Status website to allow the public to track medicines as they progress through the listing process, and 'developing a mentoring pilot program for HTA consumer committee representatives.'[53]

4.26 The Department described the work of its HTA Consumer Evidence and Engagement Unit, established in 2019, 'to support broader consumer participation strategies.' This Unit has been involved in the mentoring of the consumer committee representatives, and is 'exploring ways to enhance the transparency of HTA processes further.' This includes by considering the methods used by the United Kingdom (UK) National Institute of Health and Care Excellence (NICE), and working on a pilot project for sponsors of PBAC submissions to provide patients with a simple summary of their submission.[54] Ms Jo Watson, Deputy Chair, PBAC, summarised the Unit's work as follows:

> That Unit has been able to inform the work of the consumer representatives working within committee processes, as well as to start formally developing better ways that we can structure liaison and opportunities for participation with our external patient representatives, their networks and organisations.[55]

4.27 There are currently two patient representatives on the PBAC, including Ms Watson, and other patients have the opportunity to provide input into the assessment of individual submissions. The Department identified four principal processes through which that input is contributed:

- Direct input through consumer comments made to the committees
- Invitations to present in person at specific hearings
- Representation in expert clinical consultations about specific submission items

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52 Department of Health, Submission 15, p. 38.

53 Department of Health, Submission 15, pages 38-39.

54 Department of Health, Submission 15.6, p. [25].

55 *Committee Hansard*, Canberra, 24 June 2021, p. 4.

- Representation and input to formal stakeholder meetings and public consultations.[56]

4.28 In its own submission to the inquiry, the PBAC noted the recent changes that have been made to the system, submitting that:

> PBAC initiated changes include measures to increase patient engagement, patient hearings, increase transparency of information that informs PBAC decisions and implementing a process for review of PBAC recommendations which have not resulted in a PBS listing of a medicine.[57]

Patient views on the Pharmaceutical Benefits Advisory Committee

The Pharmaceutical Benefits Advisory Committee's engagement with patients and patient evidence

4.29 Patients were overwhelmingly of the view that the PBAC needs to be more engaged with them and pay more attention to their views. Painaustralia reflected the views of many patients when it submitted that 'existing mechanisms for consumer input into PBAC processes [are] limited, and inaccessible to grassroots consumers.' It gave a recent example when it was consulted by the PBAC through its Deputy Chair on belimumab, a treatment for lupus, on a 'limited timeframe and quick turnaround', which it said showed 'the inadequacy of PBAC's current mechanisms to seek consumer input.' It recommended the values developed by Health Technology Assessment International's Interest Group for Patient and Citizen Involvement in HTA as a 'useful starting point' for improvement.[58]

4.30 Similar concerns were shared by Migraine Australia, which stated that 'it is difficult to engage with a PBAC process when there is insufficient information provided from PBAC' and 'bringing doctor and patient bodies in for consultations before a submission is made to PBAC, or very early in the PBAC process, should be required.' It advocated for such bodies to be enabled to 'initiate stakeholder meetings and appeal decisions of PBAC'.[59]

4.31 The Save Our Sons Duchenne Foundation argued that their 'community' needs to be included in HTA and other processes because the disease is so

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56 Department of Health, Submission 15.6, pages 25-26.

57 Department of Health, Submission 15.3, p. 1.

58 Painaustralia, Submission 56, p. 8. See Health Technology Assessment International, *Patient and citizen involvement*, Edmonton, undated, htai.org/interest-groups/pcig/, viewed 13 October 2021.

59 Migraine Australia, Submission 24, pages 17-18.

'poorly understood' and lived experience of it is so valuable. It argued that involvement in HTA would help educate the disease community about how the process works and dispel any 'myths' about it.[60] Duchenne Australia submitted that 'there needs to be a clear and transparent pathway to provide patient experience data through the HTA process,' on matters such as 'lived experience,' 'impact on quality and length of life' and effect on 'social and civic participation.' It said that it was 'essential to embed consumer participation in the HTA processes to flag potential issues early on.'[61]

4.32 MS Australia suggested that the HTA 'process remains mysterious to most consumers and, if they were to consider making a submission, [would] have to imagine the impact a new drug might have on their life.' It advocated for patients and clinicians to be provided with 'appropriate, clear, accessible publicly available information on HTA processes.'[62] The Melanoma and Skin Cancer Advocacy Network (MSCAN) stated that 'consumers bring a crucial lived experience' and 'processes for engagement need to be both meaningful, transparent and have a genuine impact/weighting in the decisions.' It emphasised the need for feedback to be provided to patients 'to facilitate continuous improvement in the contributions made.'[63]

4.33 Speaking specifically of rare diseases, RVA argued that 'it is critical that HTA processes formally embed, capture and promote the voice of people living with rare disease and their families and carers' to 'provide much needed narrative and context to the data presented.' It did note that in its view, within Australia's system, 'PBAC is the gold standard in terms of…consumer engagement.'[64]

4.34 CHF submitted that patient involvement improves the 'legitimacy' of decision-making. It argued that 'methods are needed to incorporate data and evidence provided by patients' into HTA' and 'HTA systems need mechanisms to incorporate data and evidence provided by patients.'[65] The Haemophilia Foundation Australia (HFA) said that HTA should involve 'evidence that analyses patients experiences and [uses] patients' words to

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60 SOSDF, Submission 33, pages 27-28.

61 Duchenne Australia, Submission 77, pages 2, 6.

62 MS Australia, Submission 85, p. 10.

63 Melanoma and Skin Cancer Advocacy Network (MSCAN), Submission 116, p. 4.

64 RVA, Submission 86, p. 10.

65 CHF, Submission 205, p. 9.

explain what an outcome means to them to gain a comprehensive understanding of the actual outcome.'[66]

4.35 Another submitter urged that 'PBAC submissions should include consumer comments.'[67] This view was shared by CFA, which submitted that the Government should 'insist on consumer consultation and sharing of real-life evidence up front in the process,' and 'encourage and incentivise patient organisations to be involved in the process.'[68] ITP Australia submitted that hearing and considering the patient voice 'includes, but is not limited to, working with rare disease organisations and consulting effectively on patient criteria.[69]

Patient reported outcome measures and patient reported experience measures

4.36 Many patient organisations called for the inclusion of Patient Reported Outcome Measures (PROMs) and Patient Reported Experience Measures (PREMs) in the HTA process.[70] The absence of such measures was noted by CFA and MS Australia, the latter of which emphasised the need for them to be included in clinical trials.[71] GUARD emphasised the importance of PROMs, describing them as 'essential measurements in rare disease development', and the need for them to be developed 'at an early stage of product development.'[72] The Spinal Muscular Atrophy Association of Australia (SMA Australia) likewise stated that 'the consumer is not part of the approval process from the beginning with PROMs or PREMs not part of the HTA submission.'[73] RVA submitted that companies should be 'encouraged' to include such measures and to show that patients were

66 Haemophilia Foundation Australia (HFA), Submission 119, p. 1.

67 Name withheld, Submission 22, p. [2].

68 CFA, Submission 8, p. [4].

69 ITP Australia, Submission 139, p. 5.

70 APAA, Submission 67, p. [2]; MSCAN, Submission 116, p. 4; HFA, Submission 119, p. 1; Lymphoma Australia, Submission 143, p. [5].

71 CFA, Submission 8, p. [2]; MS Australia, Submission 85, p. 11.

72 GUARD, Submission 46, p. 9.

73 SMA Australia, Submission 37, p. [2].

involved in research design.[74] Lymphoma Australia recommended that they be included as part of a 'post market assessment process.'[75]

Patients calling for more information – submission summaries

4.37 CFA apparently spoke for many patients when it stated of 'lack of transparency with sponsor submissions. Not enough information is available in the public domain.' It recommended 'provid[ing] consumers with more information about the submission.'[76] The Juvenile Arthritis Foundation Australia (JAFA) similarly submitted that 'transparency is essential and could be achieved without compromising commercially sensitive information.'[77]

4.38 One particular idea that sparked patient interest was the possibility of sponsors providing simplified summaries of their submissions to patients to enable them to provide better informed input into the assessment process.[78] As mentioned above the Department's HTA Consumer Evidence and Engagement Unit is already testing a pilot of such scheme. Most of the discussions of this idea before the Committee related it to a similar system already in place in Scotland, and is discussed further in the 'Overseas Models' section below.

Patient comments on other Pharmaceutical Benefits Advisory Committee issues

Membership and access to expertise

4.39 Many patient groups believe that the PBAC needs to engage more closely with clinical experts in the diseases for which it is evaluating treatments, particularly for rare diseases. Mrs Nicole Millis, Chief Executive Officer, RVA, told the Committee:

> Rare disease expertise should be sought and accessible on every approval process. All of our approval processes deal with rare disease HTA. We need

74 RVA, Submission 86, p. 4.

75 Lymphoma Australia, Submission 143, p. [5].

76 CFA, Submission 8, p. [4].

77 JAFA, Submission 154, p. [3].

78 Lymphoma Australia, Submission 143, p. [5]; Ms Jane Hill, Chief Executive, OCA, *Committee Hansard*, Melbourne, 23 April 2021, p. 45.

> earlier and ongoing consumer input into HTA—and when I say 'consumer' I mean patient and clinician.[79]

4.40 Similarly Mrs Annette Burke, Chief Executive Officer, CFA, stated that:

> So we need the expertise and we need it around precision medicine. There are incredible things that doctors and scientists are doing around organoids, ohmics [sic] and all of those really technical ways of evaluating drugs for the individual, not these big, mass double-blind placebo trials.[80]

4.41 Ms Sharon Caris, Executive Director, HFA, explained that:

> …we strongly advocate for the HTA committees to include specialist clinicians and patients at every step to deliver specialised expertise to underpin decision-making; for example, with rare diseases like haemophilia, we could bring together affected patients, treating clinicians, MSAC [the Medical Services Advisory Committee], the NBA [the National Blood Authority] and the sponsor at the beginning of the process to discuss the submission, share expertise and data, and discuss solutions around access before the process begins.[81]

4.42 The National Aboriginal Community-Controlled Health Organisation (NACCHO) noted that it could 'not identify any member of the HTA consumer committee or PBAC with a primary expertise in Aboriginal and Torres Strait Islander health.'[82] It recommended that the Department 'enhance Aboriginal and Torres Strait Islander people's representation across Commonwealth HTA committees and agencies.'[83] It recommended the establishment of a separate 'Aboriginal and Torres Strait Islander medicines advisory committee,' jointly chaired by NACCHO and the Department, to fulfil roles including reviewing current PBS listings for Aboriginal and Torres Strait Islander people, scoping potential new listings, and advising the Department and HTA committees.[84]

Length of review and resubmissions

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79 *Committee Hansard*, Sydney, 11 March 2021, p. 2.

80 *Committee Hansard*, Sydney, 11 March 2021, p. 2.

81 *Committee Hansard*, Melbourne, 23 April 2021, p. 49.

82 National Aboriginal Community-Controlled Health Organisation (NACCHO), Submission 190, p. 4.

83 NACCHO, Submission 190, p. 5.

84 NACHHO, Submission 190, p. 6.

4.43 Many patient groups expressed concern with how long Australia's HTA processes currently take.[85] MSACN, for example, stated that 'access to new medicines and treatments is too slow, and lags in reimbursement are directly impacting on too many Australians' while Duchenne Australia commented that '…the approval pathway is lengthy and remains uncertain as to whether it will be successful.[86]

International cooperation

4.44 As in the case of the TGA, patient groups were enthusiastic proponents of increased collaboration with international HTA bodies and harmonisation of HTA processes, with several making general recommendations along those lines.[87] CHF focused on what Australia can learn about HTA methods from overseas rather than direct collaboration, and the Alpha-1 Organisation Australia (A1OA) likewise suggested the Government should review international pricing strategies for low volume drugs, such as New Zealand's bundling approach.[88]

4.45 A&AA called for 'improved utilisation' of the COR pathway and ACCESS Consortium, seemingly for HTA purposes, while Lymphoma Australia asked that there be in similar progress in this area for HTA as there has recently been by the TGA with initiatives such as Project Orbis.[89] CFA and SMA Australia made arguably the most radical proposal, both suggesting that Australia should jointly negotiate medicine reimbursement with other similar countries such as the UK, Canada and New Zealand.[90]

Interim access

4.46 Many patient groups were strong supporters of some form of 'interim access' model, meaning patients would get access to medicines before the final negotiation between sponsor and Government is complete. The CF

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85 For example: Name withheld, Submission 91, p. [1]; The Mito Foundation, Submission 125, p. [2]; CHF, Submission 205, p. 8.

86 MSCAN, Submission 116, p. 2; Duchenne Australia, Submission 77, p. 6.

87 Sanfilippo Children's Foundation, Submission 36, p. 1; GUARD, Submission 46, pages 11-12; JDRF Australia, Submission 52, p. 5; OCA, Submission 135, p. 4; Carers and Patients of Braf V600E Colorectal Cancer, Submission 144, pages [3]–[4]; JAFA, Submission 154, p. [4]; FSHD Global Research Foundation, Submission 200, p. 5.

88 CHF, Submission 205, p. 8; A1OA, Submission 29, p. 5.

89 A&AA, Submission 128, p. [5]; Lymphoma Australia, Submission 143, p. 5.

90 CFA, Submission 8, p. [4]; SMA Australia, Submission 37, p. [2].

Pipeline Patient Interest Group encouraged the Government to 'consider the German model…when long negotiations are likely.'[91] The 'German model' is discussed in Chapter 6. This was likewise supported by OCA and the APAA, particularly for 'life-saving drugs,' and by CFA.[92] SMA Australia advocated a similar course, namely 'immediate access to life-saving drugs following TGA approval.'[93] Rare Cancers Australia proposed 'granting access to treatments once they are assessed as effective and then using real world patient experience to assess pricing after the fact.'[94]

Real world evidence

4.47 Closely linked to the ideas of interim access and patient evidence such as PROMs and PREMs , many patient groups agreed that there needs to be more use made of so-called 'real world evidence' (RWE) in HTA.[95] CHF supported this proposition and stated that RWE:

> …includes electronic medical/health records, registries, patient reported data inclusive of quality-of-life data, qualitative research, use of surrogate outcomes, deciding which outcomes are to be included in an assessment which needs patient and clinician input, costing, monitoring over time, and analysis of uncertainties.[96]

4.48 SMA Australia pointed out that RWE has the advantage over clinical trials that it draws from a broad population, not a narrow one, and does not 'result in disparities in access for those not enrolled.'[97]

4.49 RVA commented that 'currently, there is no process in Australia for translating and utilising valuable real world data as it emerges, yet this remains a potentially invaluable strategy to facilitate timely regulatory approval and to enable equitable therapeutic access.'[98]The CF Pipeline Patient Interest Group recommended a 'broadening of the range of accepted

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91 CF Pipeline Patient Interest Group, Submission 169, p. 2.

92 OCA, Submission 135, p. 4; APAA, Submission 67, p. [4]; CFA, Submission 8, p. [4].

93 SMA Australia, Submission 37, p. [2].

94 Rare Cancers Australia, Submission 166, p. [4].

95 CFA, Submission 8, pages [2], [4]; Name withheld, Submission 22, p. [2]; Migraine Australia, Submission 24, p. 21; APAA, Submission 67, p. [2]; MS Australia, Submission 85, p. 11; Lymphoma Australia, Submission 143, p. [5].

96 CHF, Submission 205, p. 8.

97 SMA Australia, Submission 37, p. [1].

98 RVA, Submission 86, p. 9

evidence to include more universal and appropriate use of [RWE]' and the creation of guidelines to recognise its value;[99] it proposed gathering such evidence through data registries, and the 'German model', discussed above.[100] GUARD noted the importance of 'continuous generation of RWE post approval to reduce uncertainties.'[101]

The comparator requirement

4.50 Some patient groups called for reform to the PBAC's comparator requirements. Migraine Australia submitted that a no comparator should be used for new medicines 'where there is no real comparator drug' instead of the current procedure of using the nearest alternative, pointing to what it regards as the inappropriate use of onabotulinum toxin A (Botox) as the comparator for a new class of migraine treatments known as Calcitonin Gene Related Peptides (CGRPs).[102] The CF Patient Pipeline Interest Group likewise noted that many of the cystic fibrosis treatments in development are 'highly innovative genetic therapies', and submitted that consequently 'the type and use of comparators must be reasonable for the specific mutation, not the entire patient population.'[103]

Submissions without a sponsor

4.51 A number of submitters drew the Committee's attention to the problem of how submissions can be facilitated when there is no company willing to sponsor them.

4.52 CFA and the APAA both submitted that 'pathways' should be established where 'benefit and patient need can be demonstrated.'[104] The PFIC Network asked that the rare disease organisations be enabled to work with the Department's HTA Consumer Evidence and Engagement Unit for 'medicines with demonstrated benefit for a rare disease,' a request that was echoed by WMozzies, and by the Metabolic Dietary Diseases Association and Prader-Willi Research Foundation Australia (PWRFA) which both cited

99 CF Pipeline Patient Interest Group, Submission 169, pages 2-3.

100 CF Pipeline Patient Interest Group, Submission 169, pages 1, 3.

101 GUARD, Submission 46, p. 13.

102 Migraine Australia, Submission 24, pages 18-20.

103 CF Pipeline Patient Interest Group, Submission 169, p. 3.

104 CFA, Submission 8, p. [4]; APAA, Submission 67, p. [3].

Action 2.4.3.2 of the *National Strategic Action Plan for Rare Diseases (Action Plan)*.[105]

4.53 Migraine Australia brought up this issue in the specific context of repurposing. It proposed a 'quick and affordable…departmental process' for listed medicines to have their listing altered 'when requested by third parties such as patient bodies.'[106] RVA called for 'a viable pathway for consumers to make an application', particularly for repurposed medicines.[107]

4.54 The Australian and New Zealand Headache Society submitted that it has 'recognised other areas of unmet need in headache over time but has been unable to advocate at any significant level for these changes, since the only avenue is to fund a major submission to PBAC.' It recommended the creation of 'an alternative pathway to PBAC consideration of such agents; the capacity for professional bodies such as ours to make such submissions would be an option.'[108]

4.55 Similarly, the Australian and New Zealand Children's Haematology/Oncology Group submitted that:

> We would also support the development of a streamlined system to allow physician-led applications for registration and reimbursement for rare indications in cases where pharmaceutical companies are not inclined to invest in the registration process.[109]

4.56 A doctor who requested name withheld status, called for the establishment of pathways for 'timely widening of PBS funding of therapies with repurposed use,' arguing that 'these pathways should not be dependent on initiation by drug companies. This has the advantage of removing commercial interests.'[110]

4.57 The Macquarie University Centre for the Health Economy (MUCHE) suggested the introduction of a 'contracted addressment process for listing new orphan and off-patent drugs on the PBS,' which could be modelled on 'the MSAC contracted assessment process whereby the Department

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105 PFIC Network, Submission 19, p. [3]; WMozzies, Submission 165, p. 4; MDDA, Submission 109, p. [8]; PWRFA, Submission 110, p. [4].

106 Migraine Australia, Submission 24, p. 22.

107 RVA, Submission 86, p. 4.

108 Australian and New Zealand Headache Society, Submission 115, p. [2].

109 Australian and New Zealand Children's Haematology/Oncology Group, Submission 120, p. 7.

110 Name withheld, Submission 48, p. [2].

organises, coordinates and covers the costs associated with developing and preparing the necessary MSAC documents for consideration.'[111]

Access for Aboriginal and Torres Strait Islander Australians

4.58 In addition to its comments on the PBAC's membership and its advisory committee proposal discussed above, NACCHO suggested the creation of 'a streamlined pathway to incentivise sponsors to make submissions to PBAC for Aboriginal and Torres Strait Islander populations' and an 'update of PBAC guidelines to emphasise the needs and priorities of Aboriginal and Torres Strait Islander populations.'[112] It argued for these proposals in part because of the stark gap in expenditure per capita between Aboriginal and Torres Strait Islander Australians and the rest of the population, which was found to be $537 per person for the former compared with $891 per person for the latter in 2020.[113]

Broader concept of value

4.59 Another vital issue for patients was the question of how medicines, particularly for rare diseases, are valued. Narcolepsy Australia recommended that 'quality of life assessment considerations be permitted in applications.'[114] ITP Australia recommended that there be 'a restructure of the [economic assessment] of treatments to include not just the immediate costs…but the lifelong economics' especially for rare diseases.[115] SMA Australia suggested 'novel value-based pricing strategies incorporating broad HTA to maximise benefits…could be a way of future access.'[116] The Patient Voice Initiative highlighted the importance of including 'benefits not documented in traditional evidence, including non-health benefits,' as well as 'non-health risks.'[117]

4.60 Migraine Australia insisted that for 'new drugs without comparator' the impact of a potential listing on the health budget should not be considered, but rather the impact on the budget as a whole, thus including factors such

111 Macquarie University Centre for the Health Economy (MUCHE), Submission 62, p. 2.

112 NACCHO, Submission 190, pages 5-6.

113 NACCHO, Submission 190, p. 3.

114 Narcolepsy Australia, Submission 55, p. 6.

115 ITP Australia, Submission 139, p. 5

116 SMA Australia, Submission 37, p. [1].

117 Patient Voice Initiative, Submission 71, p. 2.

as increased tax revenue through patients returning to the workforce. It described this as a 'holistic cost-benefit analysis.'[118] A similar argument was made by HFA, which suggested that the HTA process needs to 'consider the cost benefits to the whole of government of the whole of life benefits that our community experience,' not just the impacts on health budgets. Its examples of 'indirect benefits' included 'children being able to attend school regularly' and relatives being able to spend less time caring for patients and more time working.[119]

4.61 CHF submitted that 'the current PBAC assessment of medicines…inadequately considers the evaluation of social and economic impacts of a particular intervention….Economic evaluation of an intervention must be conducted within a societal perspective and [with a] broader context in mind.'[120] JAFA submitted that '…ultimately most decisions are based on cost. While this remains in place, beneficial therapies either are not funded through the PBS or take an unnecessarily long time to be listed.' It recommended that a formal review of the PBS funding model be undertaken to try to develop a better model.[121]

4.62 GUARD argued that the current approach to 'setting a price according to…the perceived or estimated value of a medicine does not work, in particular for rare diseases.' It suggested that more work is needed on correctly valuing medicines according to the outcomes they produce, and raised the possibility of paying lower prices in return for faster reimbursement of medicines.[122] The PFIC Network submitted that 'rare disease therapies [are] unable to meet the criteria for subsidy under current PBAC …pathways as they were designed for the evaluation of common disease therapies.'[123]

4.63 The A1OA argued that subsidisation of new drugs or technologies should be prioritised 'where a genetic disorder has never had any subsidised

118 Migraine Australia, Submission 24, p. 20.

119 HFA, Submission 119, pages 2, 7.

120 CHF, Submission 205, p. 8.

121 JAFA, Submission 154, p. [4].

122 GUARD, Submission 46, pages 12-13.

123 PFIC Network, Submission 19, p. [9].

treatment.'[124] WMozzies called for 'equity' to be added to the principles underpinning Australia's HTA processes.[125]

The post-Pharmaceutical Benefits Advisory Committee process and price negotiations

4.64 Migraine Australia raised the issue of how post-PBAC pricing negotiations are conducted, suggesting that budgetary concerns are too prominent within the PBAC's decision making and that the Pharmaceutical Benefits Pricing Authority should be re-established to provide independent oversight of the pricing negotiation process.[126] SCN2A Australia requested the Government 'reduce the delay in getting approved medications available to patients', although it used medicinal cannabis as an example of this, so it is unclear to what extent this refers to price.[127]

4.65 A&AA asked for 'a more efficient registration and PBS listing process without jeopardising consumer safety,' giving the example of the atopic eczema treatment dupilumab, which had been recommended by PBAC seven months before the date of submission but had still not been listed.[128] CHF stated that 'improved, streamlined pricing negotiation processes are needed to enable greater transparency of funding arrangements across the health system.'[129] Several submitters raised the issue of the lack of any time limit on price negotiations between the Government and sponsors;[130] MS Australia, for example, argued that imposing such a limit 'would provide some certainty regarding access to treatment and managing consumers' and clinicians' expectations.[131]

Listing update and review process

4.66 PWRFA touched on another common theme in recommending 'that there is a process for timely review and updating of PBS listings to ensure equitable

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124 A1OA, Submission 29, p. 2.

125 WMozzies, Submission 165, p. 3.

126 Migraine Australia, Submission 24, pages 21-22.

127 SCN2A Australia, Submission 127, p. [2].

128 A&AA, Submission 128, p. [5]. Dupilumab was subsequently listed on the PBS on 1 April 2021: Department of Health, *Dupilumab*, Canberra, May 2021, www.pbs.gov.au/medicinestatus/document/331.html , viewed 1 September 2021.

129 CHF, Submission 205, p. 9.

130 Name withheld, Submission 22, p. [2]; APAA, Submission 67, p. [3].

131 MS Australia, Submission 85, p. 11.

and evidenced-based [sic] access to therapies.'[132] ITP Australia suggested that such a process 'utilise evidence from reputable international agencies', while Lymphoma Australia asked that it include 'rigorous patient measures'.[133] The CF Patient Pipeline Interest Group recommended that the Government 'allow more flexible models such as "pipeline agreements" to be considered with a particular sponsor, where new medications are provided and the listing can be expanded to include additional patients without additional PBAC meetings'.[134] Migraine Australia advocated for 'automatically listing alternative preparations and pack sizes'.[135]

The patient voice and the Medical Benefits Advisory Committee

4.67 The Department informed the Committee that 'from 1 July 2021, revised MSAC consultation processes took effect to improve opportunities for stakeholder input, provide procedural fairness and improve transparency.'[136]

Patient views on Medical Services Advisory Committee

Medical Services Advisory Committee's engagement with patients and patient evidence

4.68 Many patients commented on HTA in general rather than one of the specific HTA committees in particular. Nonetheless, the MSAC is an important body for many patients, and some specifically addressed it in their submissions.

4.69 GUARD commented that 'we welcome the review of MSAC Guidelines and the proposed move to include personal utility as part of the decision-making but are concerned that this will further add time and qualitative measures will not be equal in weight to quantitative measures.'[137] The Leukaemia Foundation supported Action 2.2.3.b of the *National Strategic Action Plan for*

132 PWRFA, Submission 110, p. [3].

133 ITP Australia, Submission 139, p. 5; Lymphoma Australia, Submission 143, p. [5].

134 CF Pipeline Patient Interest Group, Submission 169, p. 3.

135 Migraine Australia, Submission 24, p. 22.

136 Department of Health, Submission 15.6, p. [25].

137 GUARD, Submission 46, p.

Blood Cancers, under which the working group the Action Plan establishes should work with the Government and other stakeholders to:

> Continue important reforms to MSAC processes for MBS [Medicare Benefits Schedule] listings, focusing on greater transparency and the rapid adoption of diagnostics…This should include enhancing consumer understanding of and engagement with the MBS listing process, drawing experience from improved consumer engagement in PBS processes.[138]

4.70 RVA likewise submitted that 'the MSAC certainly lacks transparency around timelines and formal consumer engagement' and that 'it is vital that clear timeframes to reach and publish outcomes, similar to the PBAC's timeframes, are implemented and made public.' It reported that some patient organisations have 'a higher level of confidence' in the PBAC than the MSAC due to these differences in transparency.[139]

4.71 Lymphoma Australia supported the publication of 'a more comprehensive summary of submissions' to the MSAC along with the release of the agenda for each meeting.[140] HFA called for the involvement of patient organisations from the beginning of the MSAC process, to assist in 'identifying appropriate evaluation tools, clinical or quality of life outcomes or benchmarks.' It highlighted the need for clinicians with experience of the particular condition in question to be involved in the MSAC's assessment, given how PROMs can vary between different conditions. It explained that:

> …a culture of stoicism and low expectations of treatment benefits has meant that people with haemophilia often have higher mental, psychological and social scores for health-related quality of life than people with similar chronic health conditions, such as arthritis, while their physical functioning scores are actually very low.[141]

Patient comments on other Medical Services Advisory Committee issues

Real world evidence and international cooperation

4.72 There were numerous calls for more use of RWE and international cooperation in HTA generally. Lymphoma Australia addressed the MSAC specifically, suggesting it needs to learn from the example of the TGA and

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138 Leukaemia Foundation, Submission 103, p. [8].

139 RVA, Submission 86, pages 10-11.

140 Lymphoma Australia, Submission 143, p.[5].

141 HFA, Submission 119, p. 9.

how it has increased its international cooperation through initiatives like Project Orbis. In particular it wanted to see the MSAC include 'real-world data that is timely and aligned with approvals from other countries' in its decision-making.[142]

Broader concept of value

4.73 GUARD welcomed the inclusion of 'personal utility' as a consideration in the MSAC's decision-making, although it expressed concern as to how this was to be done. The PFIC Network submitted that the criteria the MSAC uses for its decisions were designed for therapies for common diseases, making it more difficult for rare diseases therapies to be approved for subsidy. In response to this issue it proposed 'broadening' the description and understanding of the principles underpinning Australian HTA processes' and increasing the availability of rare disease expertise in those processes.[143]

Miscellaneous patient comments on Medical Services Advisory Committee

4.74 Patient organisations raised various other concerns about the MSAC. RVA noted that 'while the MSAC can use expedited processes, these processes can only be considered for resubmissions.'[144] HFA asked that the PBS Medicines Status website be expanded to cover technologies being reviewed by the MSAC.[145] Finally, the AFAO recommended that the Government should establish a 'priority track' through the TGA and MSAC for therapies 'needed in the national interest for the protection of the public from health threats.'[146]

The patient voice and the Prostheses List

4.75 The T1DHub was the only patient group to comment on the Prostheses List Advisory Committee (PLAC). It recommended:

> Implement mechanisms for the patient voice to be heard in relation to the Prostheses List approval process. Currently, there is no process to ensure the patient voice is heard and when it is, it may not be the right patient at PLAC

142 Lymphoma Australia, Submission 143, p. [5].

143 PFIC Network, Submission 19, p. [2].

144 RVA, Submission 86, pages 10-11.

145 HFA, Submission 119, p. 10.

146 AFAO, Submission 196, p. 3.

level. Seeking submissions or statements from health consumers with lived experience could assist greatly in understanding the conditions and lived experience health outcomes for patients.[147]

4.76 It proposed reducing PLAC application times by making more use of international approvals. [148]

Other submitters' views on the patient voice

4.77 Many non-patient submitters expressed views on the system's engagement with patients, whether generally or through a specific part of it. Miss Jessica Pace, a pharmacist completing a PhD on regulatory and funding mechanisms, said that her research shows that clinicians and patients largely believe the system uses 'fair procedures', including 'meaningful opportunities for stakeholder participation,' although transparency could be improved.[149]

4.78 BioMarin Pharmaceutical Australia advocated for compulsory consumer hearings, together with 'appropriate processes for local experience from expert clinicians and patients to be considered in the evaluation process.[150] LEO Pharma suggested that 'patient views are currently undervalued as part of the HTA assessment process', and that 'improving the current mechanism to allow for better patient contribution will improve decision-making.'[151]

4.79 Merck Healthcare supported a 'stronger voice' for patients in the system as a whole.[152] Better Access Australia raised a number of questions about how the current system engages with patients, particularly 'grassroots patients groups and individual consumers. These questions reflected Better Access' concerns that the system is much more engaged with industry than with patients, and that patients have to reach out to government rather than vice

147 T1DHub, Submission 192, p. 3.

148 T1DHub, Submission 192, p. 3.

149 Miss Jessica Pace, Submission 40, p. 5.

150 BioMarin Pharmaceutical Australia, Submission 152, pages 3-4.

151 LEO Pharma, Submission 202, p. 3.

152 Merck Healthcare, Submission 34, p. 1.

versa; for example patients who provide feedback on a submission are not notified when a decision is reached on that submission.[153]

4.80 The Australian Cardiovascular Alliance recommended the introduction of 'a consultative process between all HTA committees and researchers, clinicians, patients and patient groups.'[154] Merck Sharp & Dohme Australia submitted that 'a patient-centred approach [to HTA] is required' because patients bring a' unique perspective on disease and the value of potential new treatments.'[155]

4.81 The Australian Healthcare and Hospitals Association submitted that 'it is difficult to balance the type of data and evidence required for current HTAs, which are largely based on clinical outcomes, with patient outcomes or experiences' and that various 'data limitations' exist for patient outcomes. It proposed that the system needs 'to ensure that patient outcomes and experiences are measured and included in datasets through standardised systems or collections.'[156] This recommendation was echoed by Stryker South Pacific, which suggested that funding be adapted 'to enable providers to focus on outcomes that matter to patients as well as cost efficiencies.'[157]

4.82 Medicines Australia expressed concern that patients 'with less common conditions' who do not have access to a patient advocacy group may struggle to contribute to HTA processes. It recommended 'expanded stakeholder involvement in decision-making, before, during and after HTA consideration.' It supported strengthening the patient voice through improving patient input processes' and 'consistent inclusion of PROMs'.[158] It noted that patient involvement in HTA is legislated in Germany, Italy and Taiwan, and suggested that 'there is an argument' for legislating it in Australia.[159]

4.83 ViiV Healthcare Australia (Viiv) submitted that 'stakeholder input into PBAC submissions should be encouraged and valued as meaningful evidence leading to better informed decisions,' and emphasised that 'the

153 Better Access Australia, Submission 160, pages 11-12.

154 Australian Cardiovascular Alliance, Submission 76, p.13.

155 Merck Sharp & Dohme Australia, Submission 63, Appendix A, p. 4.

156 Australian Healthcare and Hospitals Association, Submission 68, pages 1-2.

157 Stryker South Pacific, Submission 28, p. 7.

158 Medicines Australia, Submission 141, p. 8.

159 Medicines Australia, Submission 141, p. 38.

current process is not consistent across submissions.' It noted the legislated requirement of patient input in the aforementioned countries, and the practice in the UK and Canada of identifying interested patient groups and inviting them to make submissions. It praised the Canadian practice of publishing 'the patient document' online in preference to the PBAC selection of 'a sample of patient feedback.'[160]

4.84 The Medical Technology Association of Australia claimed that 'evaluation processes do not sufficiently account for patient input and preference,' with the option for sponsors to arrange for patient input for applications to the MSAC but uncertainty about how it is used by in assessments, and indeed whether it is used at all. It recommended that 'the Department should hold an open workshop on the incorporation of patient input and preference into MSAC evaluations with a commitment to implement aligned recommendations.'[161] It argued that similar issues apply to the PLAC, and that while it includes patient representation the representatives often do not have specific expertise in the condition to which a particular application relates. It advocated for patient input to the PLAC to be considered in its proposed workshop.[162]

4.85 Commenting on the draft MSAC guidelines that were available at the time it made its submission, Edwards Lifesciences praised the proposals for 'looking at outcomes that are important to patients (and sometimes family or carers), and the provision of evidence to support the patient relevance of the chosen outcome.' It supported the proposed inclusion of 'quantitative patient preference data' in applications. It recommended that Taiwan be looked to as a model for patient engagement in HTA, that the Department's HTA Consumer Evidence and Engagement Unit be better resourced and that further clarification be provided about how the MSAC will evaluate patient evidence and what it expects from sponsors in this regard.[163]

4.86 PRISM (Psychedelic Research in Science and Medicine) called for 'improved mechanisms for consumer and stakeholder involvement and engagement in the assessment process for treatments involving psychedelic compounds.'[164] The Australian Antimicrobial Resistance Network recommended the

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160 Viiv Healthcare Australia (Viiv), Submission 80, pages 7-8.

161 Medical Technology Association of Australia (MTAA), Submission 148, pages 49-50.

162 MTAA, Submission 148, pages 53-54.

163 Edwards Lifesciences, Submission 83, pages 31-32.

164 PRISM (Psychedelic Research in Science and Medicine), Submission 161, p. [5].

'leveraging' of the knowledge of patients, along with other research stakeholders, in the development of a better response to the problem of antimicrobial resistance.[165]

Overseas models

The National Institute for Health and Care Excellence

4.87 There was considerable interest throughout the inquiry in the approach of the UK's NICE, which the Macquarie University Centre for the Health Economy submitted 'is often considered best-practice in terms of HTA.'[166] A major focus of that interest was its approach to patient engagement, although other aspects of its operations are discussed in later chapters. Ms Mackechnie of APAA stated that in her view England and Wales (that is, NICE) have the best overall approach to patient engagement, although it is deficient in not providing submission summaries to patients or feedback on their contributions. She described NICE as being 'very proactive in terms of reaching out to patient organisations and mentoring programs.'[167] Ms Simone Leyden, Chief Executive Officer and Co-Founder, NeuroEndocrine Cancer Australia (NECA), likewise praised NICE's approach to educating patients about HTA.[168]

4.88 Ms Leyden told the Committee:

> When a drug or a submission comes up, [NICE] consult the patient organisations that it will affect and they bring them in for a consultation workshop with regard to the submission. They get to see the submission, they get to look at the submission and they get to analyse it before it's even put up for reimbursement….it's something that we should definitely have here.[169]

4.89 The Victorian Comprehensive Cancer Centre indicated its support for such a model.[170] Painaustralia likewise singled out NICE for its 'scoping and consultation workshops,' as well as patient representation on its

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165 Australian Antimicrobial Resistance Network, Submission 53, p. 2.

166 MUCHE, Submission 62, p. 8.

167 *Committee Hansard*, Melbourne, 22 April 2021, pages 2-3.

168 *Committee Hansard,* Melbourne, 23 April 2021, p. 43.

169 *Committee Hansard*, Melbourne, 23 April 2021, p. 45.

170 Professor Grant McArthur, Executive Director, Victorian Comprehensive Cancer Centre, *Committee Hansard*, Melbourne, 23 April 2021, p. 45.

committees.[171] Viiv Healthcare described its scoping process as 'one option to improve the current system,' whereby a scoping document is developed with the input of clinicians and patient groups to determine patient population, place in clinical practice and most appropriate comparator for the therapy.'[172]

4.90 NICE provided evidence to the Committee about how it operates. On the topic of patient engagement and involvement Mr Meindert Boysen, Deputy Chief Executive Officer and Director of the Centre for Health Technology Evaluation, explained to the Committee that:

> It starts when we scope a technology evaluation, so we set the question for the work. That's where patients are involved. When we seek submissions not only are we seeking submissions from the company, but we get them from patients, from patient organisations and from clinicians. When our committees meet there will always be patient experts invited to the meeting to give their feedback, usually on what is currently used within the NHS, so not specifically on the new technology. We have lay members on our committees. We have at least two or three lay members that are part of the committee decision-making. They're standing committee members.
>
> Then when the guidance comes out consultation is a public consultation, so the public patients in a broader sense can respond. And there's a chance to challenge the recommendations at the end when we hold the appeal, so that, I guess, across the board patient organisations are involved. I should also say that patient organisations are very much part of our methods and process development work. When we think about new ways of working—and we're currently in the midst of one of those processes—we very much involve patients in the thinking. They're very active as a group. Also, in one of our recent proposals we have asked our manufacturers to provide a specific, patient-focussed summary of their submission, so that the engagement of those patient experts with the evidence that our committee sees is better managed.[173]

Scotland

4.91 Many submitters highlighted the Scottish system as having a mechanism for providing submission summaries to patients. Ms Mackechnie of APAA told

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171 Ms Carol Bennett, Chief Executive Officer, Painaustralia, *Committee Hansard*, Canberra, 26 March 2021, p. 17.

172 Viiv, Submission 80, p. 7

173 *Committee Hansard*, Canberra, 7 July 2021, p. 3.

the Committee 'we believe that a detailed summary template could be co-developed with patients [in Australia], much as they have done in Scotland.'[174] She said that so far as she is aware the Scottish system is the only one currently providing such summaries.[175]

4.92 Ms Monica Ferrie, Founder, GUARD, was positive about Scotland's approach, stating that 'Scotland does some really terrific things.' She explained:

> So things like the Scottish model of 'We all do things the same' allow groups like RVA and GUARD Collaborative Australia, my organisation, is to understand the process really well for every condition and then be able to assist: 'This is the way that you would go about answering question 1. Let's have a conversation about that, rather than you go away, you do the research, you do all the work yourself and you fill out the form and we'll write a letter to support your submission.'[176]

4.93 In its *Guide for Patient Group Partners*, Scotland's HTA body the Scottish Medicines Consortium (SMC) explains to patients that:

> Most submitting pharmaceutical companies provide us with a completed Summary Information for Submitting Patient Groups Form, which we can email to you. This provides background information about the medicine and the indication, which can help inform your submission.[177]

4.94 The *Summary Information for Submitting Patient Groups Form* template is available online for download.[178]

4.95 As mentioned above, Australia has piloted a scheme for providing submission summaries to patients. Mr Neil MacGregor, Managing Director, Australia-New Zealand, Bristol Myers Squibb, told the Committee:

> We partnered recently with the Department of Health and PBAC in a pilot to enhance consumer engagement through the PBAC decision-making processes. The scope of the pilot saw BMS in concert with the Department of Health develop plain-language executive summaries specific to two of our recent

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174 *Committee Hansard*, Melbourne, 22 April 2021, p. 1.

175 *Committee Hansard*, Melbourne, 22 April 2021, p. 2.

176 *Committee Hansard*, Melbourne, 22 April 2021, p. 23.

177 Scottish Medicines Consortium, 'A guide for patient group partners ', Glasgow, March 2017 (revised August 2017), p. 8, https://www.scottishmedicines.org.uk/media/5616/guide-for-patient-group-partners-2017.pdf, viewed 13 October 2021.

178 www.scottishmedicines.org.uk/media/2775/summary-information-for-patient-groups-form.doc.

> PBAC submissions. These documents were then provided to the relevant patient groups for their review prior to their own submissions to the PBAC. We believe that this pilot initiative benefited all stakeholders and, importantly, added important patient context for that PBAC consideration.[179]

Canada

4.96 Some patient groups praised Canada's approach to patient engagement, specifically its provision of feedback to patient groups who have commented on a HTA submission. Ms Mackechnie, APAA, for example, gave evidence that 'providing feedback on the [patient] submission in terms of what worked, what didn't work and how it could be improved for next time is only done by Canada.'[180] Ms Leyden, NECA, likewise told the Committee that 'the way [Canada and Scotland] involve consumers and upskill consumers and train them in what the HTA system is about is what we should be replicating here.'[181]

Future government engagement with the patient voice

4.97 The Department and its staff were keen to emphasise the progress that has been made in engaging with patients in recent years, although they readily accepted that more work is required. Ms Adriana Platona, First Assistant Secretary, Technology Assessment and Access, Department of Health, who has overall responsibility for the Department's HTA activities, told the Committee that 'the department has been progressively improving the systematic consumer engagement relating to health technology assessment processes, and that will continue.' She noted that the Department is creating 'a new consultation platform' for HTA online.[182]

4.98 Ms Platona commented on the discussion of overseas systems, particularly NICE and the SMC, and was keen to emphasise that 'NICE does not do everything' and does not have all the responsibilities the Department has, such as price negotiation and purchasing.[183] On the issue of supporting submissions without a sponsor, she explained:

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179 *Committee Hansard*, Melbourne, 23 April 2021, pages 6-7

180 *Committee Hansard*, Melbourne, 22 April 2021, p. 2.

181 *Committee Hansard*, Melbourne, 23 April 2021, p. 43.

182 *Committee Hansard*, Canberra, 18 June 2021, p. 18.

183 *Committee Hansard*, Canberra, 18 June 2021, p. 21.

> The reality is that it needs a supplier because, in the end, the agreement to supply the product on the PBS has to be with somebody who has ownership of the product. All the other steps about doing the evidence gathering and preparation of the submissions and waiving fees and charges are all possible with government decision and additional resources. But, to have a product on the PBS, it needs a sponsor.[184]

4.99 The TGA's Adjunct Prof Skerritt noted that the increased publicity the TGA has received due to the COVID-19 pandemic 'brings the expectation that we stand up a lot more education and communication about medicines and products that [patients] use.'[185] On the issue of patient evidence he commented that 'what we are moving towards—and this is part of this work we're doing to look at real-world evidence—is to ensure that consumer patient reported outcomes are reflected more extensively.'[186]

4.100 The PBAC noted the 'need to ensure that further expansion of [its patient engagement] initiatives is adequately resourced.' It expressed a willingness to trial allowing patient representatives to observe some committee deliberations.[187] It submitted that:

> A relatively simple matter that requires industry agreement is to inform clinician and patient groups early in the submission process of the specific indications for which reimbursement is being sought. This includes the clinical claim, intended populations, and details on proposed prescribing and clinician access requirements that the sponsor is proposing to PBAC for consideration.[188]

4.101 The Department reported that the pilot on providing PBAC submission summaries to patients 'is due for evaluation in the final quarter of 2021.'[189]

4.102 The PBAC's Deputy Chair Ms Watson told the Committee:

> We've talked with several patient groups about what some of the potential benefits would be of being able to come in and provide comment earlier on in the cycle—for PBAC, particularly, at the time of submission or at the time of the subcommittee consideration—and have more of a path, if you like, in the

184 *Committee Hansard*, Canberra, 18 June 2021, p. 30.

185 *Committee Hansard*, Canberra, 18 June 2021, p. 26.

186 *Committee Hansard,* Canberra, 18 June 2021, p. 29.

187 Department of Health, Submission 15.3, pages 4-5.

188 Department of Health, Submission 15.3, p. 4.

189 Department of Health, Submission 15.6, p. [25].

> cycles along the way. I think that's something that speaks to the need not only for more resourcing internally with the department and our consumer unit but also to have collaboration with the sponsors about that.[190]

4.103 The MSAC did not itself provide any evidence to the Committee, but the Department told the Committee that 'the Government is committed to continuing to improve MSAC processes, including in respect of stakeholder input, communication and transparency.' It noted that 'from 1 July 2021, revised MSAC consultation processes took effect to improve stakeholder input, provide procedural fairness and improve transparency.'[191]

4.104 As noted in Chapter 2, on 7 September 2021 the Minister announced the signing of five year *Strategic Agreements* with Medicines Australia and the Generic and Biosimilar Medicines Association. These agreements include 'the co-design and implementation of an Enhanced Consumer Engagement Process to better capture the patient voice early in the medicines assessment process,' as well as a comprehensive review of HTA for medicines in general.[192]

Pharmaceutical Benefits Advisory Committee response to other issues

4.105 In response to the concerns discussed above regarding the PBAC's access to expertise, Professor Andrew Wilson, Chair, PBAC, told the Committee:

> In the paper that we've tabled there is an item which is sort of relevant to this, 2.2.6, where I've said:
>
> The PBAC is interested in exploring the mechanisms that might provide greater flexibility in committee membership without increasing what is already a large committee. This might include cross membership with MSAC to facilitate sharing of expertise especially for consideration of co-dependent submissions.
>
> But it may also include situations where we might want to bring in specific experts in relation to it. Having said that, we spend a fair amount of time between sessions meeting with clinical groups and hearing submissions from them. For example, in relation to the new medicines for spinal muscular

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190 *Committee Hansard*, Canberra, 24 June 2021, p. 7.

191 Department of Health, Submission 15.6, p. [13].

192 The Hon Greg Hunt MP, Minister for Health and Aged Care, 'Landmark new medicines agreements to bring significant benefits for Australian patients ', *Media Release*, 7 September 2021.

> atrophy, we have probably had close to 10 meetings with experts in that field over the past 12 months. We do also extensively consult outside, where required, in relation to not just rare diseases but also other diseases.[193]

4.106 On the issue of submissions lacking a commercial sponsor the PBAC submitted:

> The PBAC notes that while PBAC submissions may be made by other parties (e.g., clinical or patient groups) this is challenging given the PBAC requirements particularly without company sponsor engagement.
>
> The PBAC sees benefit in an alternate mechanism to initiate submissions where there is an unmet clinical need and a potentially useful medicine.
>
> Such an alternative pathway may include alternative sourcing arrangements (e.g., calls for submissions for specific medicines) and would require resourcing a capacity to support the preparation of submissions.[194]

Committee Comment

4.107 The Committee is grateful for the time and effort patients, carers and advocacy organisations put into providing evidence to the inquiry. It appears to the Committee that there is a growing understanding among government, industry and others of the importance of the patient voice. It commends the recent efforts of the Department to pay more attention to the views and experiences of patients in its decision-making, and the ongoing work of the patient representatives on the Department's various committees to make sure views and experiences are counted.

4.108 The Committee is adamant that there is a need for patients to participate in the HTA process at an earlier stage, and to be equipped with more information with which to do so. The Committee appreciates that every HTA system is different, and that submissions for reimbursement contain commercially sensitive information which sponsor companies reasonably want to protect. However, the Committee strongly believes that patients should be involved in the process earlier and should be provided with plain English submission summaries. The Committee encourages the Department to give serious consideration to establishing the patient voice in a similar way to that developed in the UK with NICE. The Committee urges the Department to make these patient voice reforms in conjunction with the

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193 *Committee Hansard*, Canberra, 24 June 2021, pages 5-6

194 Department of Health, Submission 15.3, p. 7.

review of the HTA system that was recently flagged to begin in July 2022 in the *Strategic Agreement 2022-27* between the Government and Medicines Australia.

4.109 The Committee considers that it is particularly important that Aboriginal and Torres Strait Islander people are represented on the PBAC and MSAC bodies. While the Committee is greatly concerned with the disparity in access to PBS medicines for Aboriginal and Torres Strait Islander people it does not consider that a separate access pathway is the answer to this problem. Instead the Committee believes that it should be addressed through improvements to patient engagement in the HTA processes. In addition, the review of the HTA system should focus on the assessment of diseases in small patient populations and address equity issues.

4.110 The Committee encourages more formal engagement with clinicians during the HTA processes, as the clinicians will bring with them the patient experience using the medicine or treatment. The Committee sees merit in the consideration of cross-membership for certain applications between the PBAC and MSAC and appointing temporary and ad hoc members to either body. Enhancing clinical engagement should be considered by the independent HTA system review in July 2022.

4.111 Patient feedback on their contributions to the HTA processes should be developed. This will improve their contributions over time and will assist in developing the patient groups understanding of the HTA system. The Committee considers that the Department should provide a tracking system online for patients to see what progress has been made within the HTA system.

4.112 A final difficult issue to emerge from the patient evidence was the problem of how medicines and technologies can be reimbursed when there is no company willing to sponsor them. The Committee notes the Department's evidence that a company is ultimately required to supply the medicine or therapy, and accepts that if the relevant company is resistant to its product being sold in Australia there is little the Government can do. However, often these will be commercial decisions influenced by market size. Alternative pathways and incentives may overcome barriers relating to what could clearly be a market failure due to the limited size of a potential patient cohort in Australia. The Committee believes the Australia Government should establish a fund to support applications by patients, clinicians and others, in the absence of a sponsor company, but that support should be strictly limited to cases of genuine need, to prevent pharmaceutical and

medical technology companies gaming the system to reduce their expenses. The fund should be annually capped with clear eligibility rules. Most instances will be for rare disease medicines and technologies.

5. The Therapeutic Goods Administration

General themes

Positive feedback on the Therapeutic Goods Administration

5.1 Many submitters were complimentary about the Therapeutic Goods Administration 's (TGA's) work in regulating medicines and medical devices. Recordati Rare Diseases Australia stated its belief that 'the TGA is very efficient in registering medical products.'[1] ARCS Australia commented that 'the TGA is an internationally recognised regulator that has been highly beneficial to our sector.'[2] Kyowa Kirin Australia said that it was 'extremely pleased with the accessibility, efficiency, responsiveness and amiability of the TGA' and that its registration processes 'are relatively efficient, if not best practice by global standards.'[3] Biotronik Australia 'commend[ed] the TGA in its desire to continually seek relevance and value' and described it as 'keen to engage with industry in seeking novel solutions.'[4]

5.2 Specialised Therapeutics Australia submitted that substantial improvements to the TGA were made following the 2014 *Expert Panel Review of Medicines and Medical Devices Regulation* (*Sansom Review*), a view echoed by Bayer Australia and New Zealand, Amgen Australia, Edwards Lifesciences and Merck Sharp & Dohme Australia, the last of which described the post-

1 Recordati Rare Diseases Australia , Submission 3, p. [1];

2 ARCS Australia , Submission 41, p. 10.

3 Kyowa Kirin Australia , Submission 87, p. 3.

4 Biotronik Australia , Submission 130, p. [5].

Sansom Review regulatory system as 'world class.'[5] In spite of this, the general view of submitters and particularly those from industry was that further improvements are still needed to enable faster and wider access to medicines and medical devices.

Use of overseas regulators

The case for more alignment with overseas regulators

5.3 The most popular ideas for TGA reform among other submitters was increased international harmonisation and cooperation.[6] Submitters argued that products approved by reputable regulators have already been proven to be effective and safe, so reassessment by the TGA results in duplication and inefficiency.[7] It was claimed that given Australia's small market size, alignment with larger markets is necessary to ensure fast access to medicines and devices, particularly for rare diseases.[8] The small United States (US) company Mirum Pharmaceuticals, for example, explained that it is submitting a rare liver disease medicine to the US Food and Drugs Administration (FDA), then taking it to the European Medicines Agency (EMA), and it would be able to bring it to Australia much faster if Australia's process was aligned with those jurisdictions. [9]

5.4 Submitters emphasised the need for the TGA to harmonise its systems and processes with overseas regulators.[10] It has already made progress on this in

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5 Specialised Therapeutics Australia, Submission 7, p. 3; Bayer Australia and New Zealand, Submission 175, p. 2; Amgen Australia, Submission 82.5, p. 2; Edwards Lifesciences, Submission 83, p. 30; Merck Sharp & Dohme Australia, Submission 63, p. 3.

6 Sanofi, Submission 99, p. 3; Medical Technology Association of Australia (MTAA), Submission 148, p. 35.

7 Australian Association of Medical Research Institutes (AAMRI), Submission 88, p. 8; Australian Amyloidosis Network (AAN), Submission 98, p. [6]; Australasian Leukaemia and Lymphoma Group and Haematology Society of Australia and New Zealand (ALLG and HSANZ), Submission 112, p. 7.

8 ARCS Australia, Submission 41, p. 10; RESULTS International Australia, Submission 106, pp. 5-6; AusBiotech, Submission 114, pp. 11-12; Australian and New Zealand Children's Haematology/Oncology Group (ANZCHOG), Submission 120, p. 6.

9 Mirum Pharmaceuticals, Submission 10, p. 1.

10 Research Australia, Submission 78, p. 11; The George Institute for Global Health, Submission 105, p. 8; University of Melbourne, Submission 133, p. 4.

some areas, such as Project Orbis for cancer medicines which was praised by several submitters.[11]

5.5 AstraZeneca Australia and Bayer Australia and New Zealand commented that 'special registration schemes' like Project Orbis should be introduced or expanded for other diseases besides cancer, with AstraZeneca Australia giving the examples of asthma and cardiovascular diseases.[12] Amgen Australia discussed Project Accumulus, a joint IT framework being developed by the FDA, the EMA, and the Japanese and Singaporean regulators to facilitate better data sharing, and argued that Australia needs to participate.[13]

5.6 In the area of rare diseases, Dr Falk Pharma Australia suggested an 'accelerated pathway' should be available for orphan drugs that have already been registered in certain 'reference markets.'[14] RESULTS International Australia, meanwhile, recommended 'allowing access in Australia to drugs for rare and orphan diseases with WHO [World Health Organization] Prequalification quality approval.' It explained that this would have the added benefit of 'allowing Australia to benefit from low costs negotiated globally for such products' by United Nations agencies.[15]

5.7 Medtronic Australasia was generally strongly supportive of more alignment with international regulators, but argued that in aligning with European Union (EU) devices regulation:

> …there needs to be a balance and caution exercised in areas where the European Regulations are not clear or the guidance documents are not made available. Should the TGA implement the changes in the absence of such explanatory documents, it can create a huge regulatory burden for sponsors.[16]

5.8 Stryker South Pacific recommended 'benchmarking approvals times for TGA against international best practice.'[17] The Medical Technology

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11 Dr Haitham Tuffaha, Submission 72, p. [1]; AAN, Submission 98, p. [5]. ALLG and HSANZ, Submission 112, p. 7.

12 AstraZeneca Australia, Submission 42, p. 2; Bayer Australia and New Zealand, Submission 175, pp. 2-3.

13 Amgen Australia, Submission 82, p. 10.

14 Dr Falk Pharma Australia, Submission 17, p. [3].

15 RESULTS International Australia, Submission 106, p. 3.

16 Medtronic Australasia, Submission 122, p. 21.

17 Stryker South Pacific, Submission 28, p. 6.

Association of Australia (MTAA) likewise recommended 'TGA should continue improving and streamlining its internal processes to deliver consistently quick review times in line with international KPIs.'[18]

5.9 ARCS Australia raised the specific issue of the TGA's regulation of manufacturing, which it said 'seems to be moving away from international harmonisation,' in contrast to the TGA's approach in other areas. It argued that 'this will increasingly present a roadblock for companies, and reverse the progress and advances' made by the other international initiatives. In response to these issues, it recommended that 'the TGA rely more wholly on overseas [Good Manufacturing Practice] accreditation and not insist on additional evaluation of audit reports or request review of technical agreements between manufacturers. '[19]

The case for caution in alignment with overseas regulators

5.10 While a strong majority of submitters who raised the issue of alignment with overseas regulators supported increasing it, some others urged caution. The Western Australian Department of Health (WA Health) highlighted the importance of the TGA being careful in deciding which regulators its designates as Comparable Overseas Regulators (CORs) commenting that:

> It is vital that the TGA regularly monitor any changes to approval processes for research and development across the CORs to ensure that standards remain of a suitable high level. Additionally, robust data capture and post market surveillance across a mandated period should be required.[20]

5.11 The Centre for Law and Genetics, University of Tasmania and Sydney Health Law and Sydney Health Ethics, University of Sydney advocated for careful consideration of any increase in reliance on international regulators, noting 'the current state of flux in acceptable safety and efficacy thresholds, and the recent controversy that has surrounded a number of medical devices approved for use in Australia through these pathways.'[21]

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18 MTAA, Submission 148, p. 7.

19 ARCS Australia, Submission 41, p. 10.

20 Western Australian Department of Health (WA Health), Submission 129, p. [7].

21 Centre for Law and Genetics, University of Tasmania and Sydney Health Law and Sydney Health Ethics, University of Sydney, Submission 179, p. 4.

5.12 The Australian Commission on Safety and Quality in Health Care (ACSQHC) noted the problems with medical devices are heavily dependent on CORs for their approval. They commented:

> … processes which ensure appropriate attention to the quality and relevance of international assessments and approval processes to local circumstances are imperative.[22]

5.13 Dr Bruce Baer Arnold and Dr Wendy Bonython, acting in a private capacity, raised the medical devices issues, and noted that regulatory failures that result in patient injuries place 'avoidable burdens on the public/private health systems.[23]

5.14 The Royal Australian and New Zealand College of Radiologists (RANZCR) drew the Committee's attention to the specific problems that can arise in aligning regulation of software that uses Artificial Intelligence (AI), in its case in clinical radiology. It submitted that:

> It has been well documented that performance of AI systems is related to the population of individuals on which it has been trained.
>
> …there need to be mechanisms in place to ensure that machine learning devices are trained and tested on individuals appropriate for the Australian demographic. Such devices are clearly labelled to ensure that they are able to be used in a clinically appropriate context. When relying on the assessment of overseas regulators, it is also imperative that the TGA has mechanisms in place to be alerted to all changes to AI systems.[24]

The Government's position

5.15 In response to the possibility of the TGA simply 'rubber stamping' decisions by overseas regulators, the Department of Health (the Department) told the Committee:

> A key reason for the Government's decision in 2016 that Australia should continue to make sovereign decisions regarding medicines approvals, rather than 'rubber stamp' decisions of other regulators, was that there was often significant discordance between these decisions. In individual cases, this is thought to be due to differences between regulators in the data submitted by the applicant, differences in clinical practice or risk appetite between countries or differences in opinions between respective advisory committees. There

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22 Australian Commission on Safety and Quality in Health Care (ACSQHC), Submission 207, p. 4.

23 Dr Bruce Baer Arnold and Dr Wendy Bonython, Submission 49, p. 12.

24 Royal Australian and New Zealand College of Radiologists (RANZCR), Submission 204, p. [2].

> have been some cases where absolute differences in regulatory outcome (acceptance versus rejection) occurred but much more common are significant differences in the approved indication (intended use) between regulators for a given medicine.[25]

5.16 On the issue of whether such 'rubber stamping' would be beneficial for provisional approval for rare disease therapies in particular, Adjunct Professor John Skerritt, Deputy Secretary, Health Products Regulation, Department of Health (Adjunct Prof Skerritt), commented:

> In provisional approval, it's even more important to know where the risks and uncertainties are. You could argue that's a case where you actually want to have information on what gaps you need to fill in the coming period. There could be safety issues. Remember, with provisional approval, you're going back to the company and saying, 'We need answers to A, B, C, D, E, F.' In order to shape those questions, you'd argue that the exact opposite should apply; you should actually know more about those drugs and what's not known and what is known about them.[26]

5.17 Adjunct Professor Skerritt argued that the most important consideration is 'to continue to have an environment and actively encourage Australia to be a tier 1 market; in other words, for submissions to Australia to be made as soon as possible after the European and North American submissions.'[27]

Length of review versus risk

5.18 Beyond the issue of alignment with overseas regulators, some submitters insisted on a more general need for caution in speeding up the regulatory process. Miss Jessica Pace emphasised that 'attempts to speed up regulatory processes can have impacts on quality.' She said that her research of the views of clinicians and patients showed that they are 'largely satisfied with our current systems of medicines funding and regulation,' although they acknowledge there are areas that need improvement, particularly for rare diseases.[28] Dr Arnold and Dr Bonython likewise submitted that 'weakening of Australia's therapeutic goods regime will result in harms without substantive benefit to consumers of medical products.'[29]

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25 Department of Health, Submission 15.5, p. [21].

26 *Committee Hansard*, Canberra, 18 June 2021, p. 20.

27 *Committee Hansard*, Canberra, 18 June 2021, p. 20.

28 Miss Jessica Pace, Submission 40, p. 3.

29 Dr Bruce Baer Arnold and Dr Wendy Bonython, Submission 49, p. 4.

5.19 *Australian Prescriber* submitted that 'it is important that rapid approval does not compromise the safety of new drugs.' It noted that 'adverse effects tend to emerge over time and, if efficacy has been based on surrogate outcomes, there can be uncertainty whether new drugs will be effective in practice.' It nonetheless did not call for any reduction in the current use of accelerated approval pathways.[30] The Sydney Children's Hospital Network and Children's Medical Research Institute kept their submission on this issue general, stating that 'the focus must be on patient safety' but that 'risks should be balanced against potential benefits of early access to novel therapeutics.'[31] The ACSQHC made the same broad point, submitting:

> The argument is sometimes made, that assessment and approval processes are extended and more immediate availability of a new drug or device would save lives. The risks and benefits of 'early' introduction should always be considered objectively.[32]

Resourcing

5.20 Currently, the TGA's activities are primarily cost recovered from industry fees and charges, however, a small amount of appropriation funding is provided for other activities. For example, in the 2019-20 Mid-Year Economic and Financial Outlook statement, the Government provided $33 million over four years (including $6.6 million in 2020-21) for work on improvement of patient safety through regulatory measures for opioids and to partially defray the costs of the TGA Special Access Scheme, Orphan Drugs Program and mandatory reporting of shortages of critical medicines.[33]

5.21 A related concern was the question of whether the TGA is adequately resourced. The Association of Australian Medical Research Institutes (AAMRI) submitted that '[approval] timelines could be decreased by increasing the TGA budget so expedited reviews can be implemented.'[34] Novo Nordisk Australia recommended that there be 'additional funding allocated to the TGA to strengthen the TGA's ability to regulate new and innovative therapeutic goods, ensuring that sufficient effort is directed to the

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30 Australian Prescriber, Submission 94, pp. [1]-[2]

31 SCHN and CMRI, Submission 185, p. 17.

32 ACSQHC, Submission 207, p. 4.

33 Department of Health, Submission 15.6, p. 1.

34 AAMRI, Submission 88, p. 8.

development and exploration of novel therapeutic classes.'[35] Dr Arnold and Dr Bonython argued that the TGA's current funding is insufficient for it to 'fulfil its responsibilities on a timely proactive basis' and 'attract and retain expertise.'[36]

5.22 *Australian Prescriber* explained that the TGA publishes Australian Public Assessment Reports (AusPARs) to provide information about newly approved medicines, but that there is often a delay between the registration of medicines and their publication. It submitted that:

> The rapid approval of new drugs in Australia must be accompanied by a rapid release of the information supporting those approvals. The TGA should be given the resources to ensure that an AusPAR is available at the same time a new drug is launched.[37]

5.23 ARCS Australia emphasised the need for 'resources at the TGA being sufficient to maintain a strong focus on continuous improvement and strategic focus.' It added that 'initiatives such as the Advanced Therapies Unit are critical to success' and 'IT infrastructure at the TGA needs a major overhaul.'[38] Johnson & Johnson seconded this, and stated:

> We would advocate for sufficient resources to address current TGA limitations, including in relation to information technology. In that regard, we support the recent announcement in the Federal Government's October Budget to provide additional resources to the TGA.[39]

5.24 Medicines Australia submitted that the TGA's IT infrastructure is not 'fit-for-purpose' and it welcomed the funding announced in the 2020 Federal Budget.[40] It further asserted that, in contrast to the FDA and European EMA, the TGA does not have sufficient internal resources to conduct its clinical evaluations and consequently must rely on external evaluators.[41] It explained that:

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35 Novo Nordisk Australia, Submission 151, p. 4.

36 Dr Bruce Baer Arnold and Dr Wendy Bonython, Submission 49, p. 8.

37 Australian Prescriber, Submission 94, p. [2].

38 ARCS Australia, Submission 41, p. 3.

39 Johnson & Johnson, Submission 134, p. 14.

40 Medicines Australia, Submission 141, p. 32.

41 Medicines Australia, Submission 141, p. 31.

> Quality of external clinical evaluations can be poor due to lack of experience …or failure to understand regulatory requirements. This creates additional burden for Sponsors in having to address erroneous requests…and can result in delays to approval.[42]

5.25 The MTAA raised concerns about the TGA's resources, and specifically its IT resources, asserting that 'long review timelines are often caused by a lack of specialist reviewers and outdated IT systems.'[43] It recommended that the Government 'ensure that TGA has the human and IT infrastructure resources to fulfil its mission.'[44] Pathology Technology Australia delivered one of the most forthright criticisms of the TGA's resourcing, submitting that:

> Improvement in the TGA is more likely to come from changing either the resourcing or the funding model. The current fee-for-service model is a nonsense when the TGA cannot staff to workload (under the public service staffing limits imposed by the Department of Finance). If this service is to remain fully fee-for-service, then it needs to be free to staff-to-workload. If TGA remains tethered to public service staffing ratios, then product assessment and registration services need to be federally funded.[45]

5.26 When asked by the Committee about whether the TGA's current cost recovery model provides it with sufficient resources, Adjunct Prof Skerritt replied:

> So consumer expectations have changed. Profiles have changed. There's a list of other things and services that we provide that can't be attributed to an individual company. There's also a greater expectation on compliance…So the dilemma I have, as I've seen the nature of expectations of regulators change, is whether we have the model that can actually service that.[46]

5.27 The Department added:

> While the TGA's activities are primarily cost recovered from industry fees and charges, a small amount of appropriation funding is provided for other activities. For example in the 2019/20 Mid-Year Economic and Financial Outlook statement, the Government provided $33 million over four years

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42 Medicines Australia, Submission 141, p. 56.

43 MTAA, Submission 148, p. 37.

44 MTAA, Submission 148, p. 7.

45 Pathology Technology Australia , Submission 178, p. [3].

46 *Committee Hansard*, Canberra, 18 June 2021, p. 26.

> (including $6.6 million in 2020/21) for work on improvement of patient safety through regulatory measures for opioids and to partially defray the costs of the TGA Special Access Scheme, Orphan Drugs Program and mandatory reporting of shortages of critical medicines.
>
> There are some activities that may not be appropriately cost recovered under *Australian Government Cost Recovery Guidelines*[47] because they cannot be attributed to individual TGA sponsors, or it would be unreasonable or inefficient to cost recover (e.g. from individual terminally ill patients in the case of SAS A).[48]

5.28 The Department went on to note a series of examples of costs that may not be appropriately cost recovered, some of which are of particular interest to this inquiry. These include 'horizon scanning on new medicines and medical technologies,' 'provision of early scientific advice for new and emerging technologies,' 'regulatory policy development for new and emerging technologies,' 'community and healthcare practitioner education and communications' and the 'Orphan Drugs Scheme.' It concluded by stating that:

> It would be a decision for government, and not for officials, to determine whether changes to TGA's funding model are appropriate, and if so how these activities should be funded.[49]

Technical aspects of regulation

Molecular indications

5.29 One overseas development that attracted particular interest from some submitters was the recent FDA approval of larotrectinib, an NTRK inhibitor. AAMRI explained that this drug:

> …was recently approved in the US for a molecular indication rather than the more usual disease indication. This means that rather than a drug being approved for the treatment of a specific cancer, such as breast, lung, bowel

47 Department of Finance, 'Australian Government cost recovery guidelines RMG304', Canberra, July 2014, www.finance.gov.au/publications/resource-management-guides/australian-government-cost-recovery-guidelines-rmg-304, viewed 14 October 2021.

48 Department of Health, Submission 15.6, p. [1].

49 Department of Health, Submission 15.6, p. [2].

> etc., it is approved for every cancer where an NTRK fusion is found, and in both adult and paediatric populations.[50]

5.30 AAMRI suggested that a 'routine, standard approval pathway for therapeutics with molecular indications' be created.[51] It did however acknowledge that:

> Safety risks need to be considered due to cross reactions and dose alterations, or administration route in conjunction with pharmaceutical development requirements. However, if these considerations are considered, expedited approvals should be possible.[52]

5.31 The Luminesce Alliance and one of its members, the Children's Cancer Institute, both noted the US example. They argued that 'in an era of molecularly and genetically targeted drugs [the current system] creates artificial access restrictions,' and that it should be changed 'to facilitate the broadening of indications sharing the same molecular and genetic drivers of disease.' [53] The Victorian Comprehensive Cancer Centre referred to the US example above in the context of rare cancers, and put forward a proposal for a 'fast-tracked approval program for tumour agnostic treatment of rare cancers.'[54]

5.32 Similarly, the Australian and New Zealand Children's Haematology/Oncology Group submitted that:

> …increasingly it is recognised that there may be rare childhood cancers which are driven by the same molecular mechanisms as more common adult cancers and that the drugs developed to treat those adult cancers may be effective in childhood cancers.[55]

5.33 Accordingly it suggested that a 'mechanism of action approach should…be extended to the conduct of clinical trials and the registration and approval process for new drugs and other novel therapies.'[56]

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50 AAMRI, Submission 88, p. 9.

51 AAMRI, Submission 88, p. 9.

52 AAMRI, Submission 88.1, p. 3.

53 Luminesce Alliance, Submission 32, p. 20; Children's Cancer Institute, Submission 84, p. [3].

54 Victorian Comprehensive Cancer Centre, Submission 61, p. 1.

55 ANZCHOG, Submission 120, p. 3.

56 ANZCHOG, Submission 120, p. 4.

Advisory Committee on Medicines

5.34 ARCS Australia raised concerns about the TGA's Advisory Committee on Medicines (ACM), submitting that there is 'a trend in significant divergence between ACM positions and that of local medical practice.' It went on to say:

> TGA has recognised that the constitution of the ACM could benefit from renewal or the TGA could further explore other mechanisms for obtaining independent scientific/medical advice (as is routinely done for oncology products for instance). We acknowledge this plan which needs to be adequately funded and be agile in responsiveness for specific expertise to support new product registration.[57]

5.35 Medicines Australia commented on the ACM that there should be 'alignment of committee membership with therapeutic area of relevance.'[58] Novartis Australia and New Zealand noted that while the Pharmaceutical Benefits Advisory Committee (PBAC) requires a positive Delegate's Overview from the ACM before it will recommend an application for reimbursement through the parallel process, the meetings schedules of the ACM and PBAC are not coordinated.[59]

Communication with sponsors

5.36 Bristol Myers Squibb (BMS) proposed that the 'TGA should provide early guidance on major and minor issues raised during the regulatory review, to enable Sponsors to begin to respond sooner, expediting the regulatory process.'[60] Medicines Australia provided a list of seven aspects of the TGA's 'Evaluation Process' and four aspects of its 'Advisory/Expert Committee Process' that it said are out of step with the equivalent processes of the FDA and EMA, together with recommendations to resolve these discrepancies.[61]

5.37 Many of these suggestions were technical issues relating to improving communication between the TGA and sponsor, although they did include their concern with the ACM membership. The MTAA raised similar concerns, commenting that questions are issued to the sponsor from different review sections at different times rather than all at once, and the

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57 ARCS Australia Submission 41, p. 11.

58 Medicines Australia, Submission 141, p. 59.

59 Novartis Australia and New Zealand , Submission 138, p. [11].

60 BMS, Submission 118, p. [16].

61 Medicines Australia, Submission 141, pp. 56-59.

process for requests for additional information means different sections often request the same information.[62]

Status of real world evidence

5.38 Submitters had various comments to make relating to the TGA's approach to evidence. The Australasian Sleep Association recommended that the TGA 'use a case-based rather than formulaic approach to making decisions' where a medicine has been shown to be effective in comparison to a placebo, even if its efficacy against comparator medicines has not yet been established.[63]

5.39 ARCS Australia noted that the TGA already accepts real world evidence (RWE) as part of application dossiers, but submitted that it should develop 'guidance for accepting and evaluating' dossiers that contain such evidence, aligned with guidance from overseas regulators such as the TGA.[64] BMS made a similar recommendation, saying that this would 'improve predictability and transparency for the sponsor, reducing the need for resubmissions and delays in patient access.'[65] Roche Australia stated 'there is a lack of formal guidance on how sponsors should develop and frame this type of evidence.' It noted that such guidance had been developed by the FDA and EMA.[66]

5.40 Medical technology submissions touching on the TGA's approach to evidence were strongly supportive of a greater role for RWE. Stryker South Pacific commented that:

> In relation to the introduction of innovative technology (without adequate clinical evidence or potentially without an adequate comparator) the ability to commit to an ongoing post-market clinical follow-up in lieu of excessive pre-market evidence generation is important to enable access in both the public and private sectors. This should include maintaining reporting requirements and the ability to halt access should early issues be identified.[67]

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62 MTAA, Submission 148, p. 36.

63 Australasian Sleep Association, Submission 16, p. 5.

64 ARCS Australia, Submission 41, p. 5.

65 BMS, Submission 118, pp. 21-22.

66 Roche Australia, Submission 92, p. 20.

67 Stryker South Pacific, Submission 28, p. 15.

5.41 Abbott Diabetes Care suggested that the TGA's evidence guidelines are 'focused and heavily weighted on double-blind randomised controlled trials, in which patients do not know what therapy they are receiving, but that while these work well for drugs they these are impossible to run for many devices. It emphasised that the shorter 'product cycles' for devices compared to drugs – meaning they are developed and made outmoded more quickly - together with 'ethics issues in device trials' and smaller patient populations mean that RWE is even more important than it is for medicines.[68]

5.42 The TGA acknowledged the thrust of these comments on its approach to evidence, as Adjunct Prof Skerritt told the Committee:

> …a number of the submissions also said that we should provide clearer regulatory guidance around the use of real-world evidence in submissions. We've actually commenced a project and public consultations which will probably lead to more specific and detailed guidance and engagement with patient groups in the industry about how we can better incorporate real-world evidence.[69]

Post-market surveillance

5.43 The potential of more use of RWE in TGA decision-making was discussed in the context of post-market surveillance, as noted by Stryker South Pacific which suggested:

> …utilising the early adoption of medical technology in Australia's private health sector to collect post-market surveillance and performance data to inform policy, regulatory and funding decisions.[70]

5.44 The MTAA suggested that lessons can be learned from the TGA's response to the COVID-19 pandemic in this respect, and that the TGA's Priority Review pathway could be improved by 'combining a fast-track premarket review with a rigorous post-market oversight to ensure both fast access and patient safety.'[71]

5.45 WA Health argued for the need for post-market surveillance where the efficacy or safety (particularly long-term safety) of a new therapeutic good is uncertain. It used the UK and EU's black triangle scheme as an example of a

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68 Abbott Diabetes Care, Submission 191, p. 2.

69 *Committee Hansard*, Canberra, 18 June 2021, p. 16.

70 Stryker South Pacific, Submission 28, p. 6.

71 MTAA, Submission 148, p. 35.

surveillance system, and suggested that surveillance could be incorporated into the TGA's schemes for access to medicines and devices not on the ARTG.[72]

5.46 Miss Pace reported that the clinicians and patients participating in her research 'expressed a desire for greater use of post-market data collection in order to provide faster access to new medicines,' but she cautioned that such collection is 'more difficult than many imagine.' She said this was because of difficulty in collecting the raw data and insufficient funding or expertise for the regulator to analyse it properly.[73] Drs Arnold and Bonython criticised Australia's current approach to post-market surveillance and called for the introduction of 'a mandatory public fault reporting scheme for therapeutic goods,' including prompt publication of information about faults by the TGA.[74]

5.47 Both the Pharmacy Guild of Australia and the Pharmaceutical Society of Australia advocated for enhanced post-market surveillance for medicines, which they described as pharmacovigilance;[75] the latter stated that 'it is important that a holistic, nationally-coordinated and outcomes-focussed approach to undertaking pharmacovigilance activities is implemented.'[76] They emphasised the important role that pharmacists play in pharmacovigilance currently and the scope for it to be increased;[77] the Guild, for example, proposed 'a standardised service model in community pharmacy that fitted in with the re-supply (repeat) arrangements for new and novel medicines.'[78]

Other areas of interest

General engagement with industry

72 WA Health, Submission 129, p. [7].

73 Miss Jessica Pace, Submission 40, p. 4.

74 Dr Bruce Baer Arnold and Dr Wendy Bonython, Submission 49, p. 13.

75 Pharmacy Guild of Australia, Submission 108, p. 3; Pharmaceutical Society of Australia (PSA), Submission 203, p. 5.

76 PSA, Submission 203, p. 5.

77 Pharmacy Guild of Australia Submission 108, p. 3; PSA, Submission 203, p. 5.

78 Pharmacy Guild of Australia, Submission 108, p. 3.

5.48 AstraZeneca Australia recommended that the TGA promote its Priority Review pathway to industry 'for other disease indications in addition to oncology.' It suggested that this would be assisted 'by periodic reporting of the number of applications' rather than the current practice of only publishing successful applications.[79] The MTAA suggested that the pathway be the subject of 'a sustained, dedicated education and training program aimed at Australian MedTech companies developing or aiming to distribute novel/ breakthrough technologies.[80] The TGA's Priority Review pathway is discussed further in Chapter 3.

The role of the states and territories

5.49 The MTAA expressed unhappiness with the role that state and territory governments currently play in the regulation of medical devices, stating that:

> State and Territory governments need to eliminate red tape and duplicative requirements for medical devices that increase the cost and burden to industry with no added benefit to patient safety, such as compulsory registration to commercial databases Recall Health and National Product Catalogue. TGA regulations, systems and processes should be adopted uniformly across Australia without duplication by State and Territory departments of health.[81]

5.50 It welcomed the 'recognition of the need for change' in the 2020 Addendum to the *National Health Reform Agreement*, and recommended that to implement that change 'a national list of novel health technologies recently approved should be created' and:

> State and territory governments should be required under their reporting responsibilities for the National Health Reform Agreements to transparently outline their processes for evaluating and funding new technologies included in the novel list, what decisions have been taken and progress in uptake of the new technology.[82]

The independence of the regulator

5.51 Dr Arnold and Dr Bonython recommended that consideration should be given to 're-establishing it as an independent body that reports direct to

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79 AstraZeneca Australia, Submission 42, p. 2.

80 MTAA, Submission 148, p. 58.

81 MTAA, Submission 148, p. 6.

82 MTAA, Submission 148, p. 59.

Parliament,' as opposed to the current model under which it forms part of the Department.[83]

Breakthrough status

5.52 There was strong interest from the medical devices industry in the FDA's Breakthrough Devices Program. Edwards Lifesciences submitted that:

> The goal of the Breakthrough Devices Program is to provide patients and health care providers with timely access to these medical devices by speeding up their development, assessment, and review, while preserving the statutory standards for premarket approval.
>
> The Breakthrough Devices Program offers manufacturers an opportunity to interact with the FDA's experts through several different program options to efficiently address topics as they arise during the premarket review phase, which can help manufacturers receive feedback from the FDA and identify areas of agreement in a timely way.[84]

5.53 Edwards Lifesciences suggested that the Government establish a similar program.[85] Medtronic Australasia explained the difference between this program and the TGA's existing Priority Review in the following terms:

> The priority review designation criteria that the TGA has established is different to that of the [FDA] and requires the requisite evidence to be available at the time of the submission rather than working in a partnership approach modelled by the [FDA], who get involved from the early stages of design, development and evidence gathering requirements such as design of clinical trials.[86]

5.54 Medtronic Australasia argued that establishing a similar program in Australia would particularly assist in ensuring breakthrough devices are quickly adopted in public hospitals, by establishing 'a more strategic national approach to patient access.'[87] It recommended 'enabling the TGA to have market entry discussions and better alignment to accept [FDA] breakthrough designations.'[88]

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83 Dr Bruce Baer Arnold and Dr Wendy Bonython, Submission 49, p. 9.

84 Edwards Lifesciences, Submission 83, p. 26.

85 Edwards Lifesciences, Submission 83, p. 27.

86 Medtronic Australasia, Submission 122, p. 15.

87 Medtronic Australasia, Submission 122, p. 17.

88 Medtronic Australasia, Submission 122, p. 26.

5.55 AusBiotech discussed the FDA program, in which it said 'the regulator effectively works with companies at early development stage to co-design study protocols and requirements, and undertakes real time assessment of manufacturing quality, effectively leading to approval at the time of reporting of trials.[89] BioScience Managers meanwhile submitted that 'breakthrough device or similar designation would help development of [digital therapeutics] in Australia.'

5.56 The MTAA submitted that the criteria for the TGA's Priority Review are 'similar to criteria used by other regulatory agencies such as the US FDA and its Breakthrough Devices Program.'[90] When asked about the difference between this program and the TGA's Priority Review, it told the Committee that:

> TGA insists on evidence up-front, whereas the FDA is more inclined to look at real-world evidence. We believe this is important…you can glean a lot of clinical evidence up-front. You would still be on top of it to make sure, but you have equity of the availability.[91]

5.57 MTAA commented that 'we hope to see…continued alignment between the TGA priority review pathway and the US FDA Breakthrough Devices Program.'[92]

5.58 Noxopharm Limited drew the Committee's attention to similar FDA initiatives for medicines. It submitted that 'providing the equivalent of the FDA breakthrough (fast-track) approval, especially for orphan drugs would bring forward revenues, again making investment in Australian drug development a more attractive option.'[93]

The Special Access Scheme

5.59 RESULTS International Australia informed the Committee that 'important drugs' for the treatment of tuberculosis currently have to be accessed through the Special Access Scheme (SAS).[94] It explained that the process for

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89 AusBiotech, Submission 114, pp. 8-9.

90 MTAA, Submission 148, p. 35.

91 Mr George Faithfull, Advisor and Vice-Chair, Regulatory Affairs Strategic Committee, MTAA, *Committee Hansard*, Canberra, 11 March 2021, p. 13.

92 MTAA, Submission 148, p. 35.

93 Noxopharm Ltd, Submission 70, p. [3].

94 RESULTS International Australia, Submission 106, p. 1.

a doctor to get approval under the SAS is 'long and cumbersome,' and if not treated as soon as possible the patient can infect others, develop drug-resistant tuberculosis, and even die.[95]

5.60 The Australasian Association of Nuclear Medicine Specialists and the Australian and New Zealand Society of Nuclear Medicine (AANMS and ANZSNM) drew attention to another problem relating to the SAS, writing that:

> The difficulty and expense of change of sponsor of an existing listed drug should be minimised. In recent years, existing listed drugs have dropped off the ARTG when a new sponsor elects not to seek change of registration. This results in nuclear medicine practices having to use the SAS pathway to use a proven drug which was once, but is no longer, on the ARTG.[96]

Nuclear medicine

5.61 The AANMS and ANZSNM made the following suggestions for radiopharmaceuticals:

> … a separate ARTG class should be created for them given their unique nature; evidence requirements for diagnostic radiopharmaceuticals should be reduced commensurate with the extremely low safety threat they pose; application costs should be reduced in recognition of the level of evidence required and the lack of commercial sponsors under the current pricing; and restrictions on interstate and intrastate supply of radiopharmaceuticals manufactured under exemption from TGA manufacturing regulation should be lifted given the low safety risk they pose.[97]

Digital technology

5.62 One area of emerging technology that attracted submitter attention was digital technology. Sleepfit Solutions focused its submission on digital therapeutics (DTx), which it described as 'evidence-based behavioural treatments delivered online that can increase accessibility and effectiveness of health care.' It noted that these differ from '"consumer grade" health-related software applications' as they 'deliver defined therapeutic interventions rather than general wellness tracking services,' and are distinct

95 RESULTS International Australia, Submission 106, p. 2.

96 AANMS and ANZSNM, Submission 95, p. 6.

97 AANMS and ANZSNM, Submission 95, p. 6.

from other 'digital healthcare services' such as 'adherence, diagnostics tools or telemedicine platforms.'[98]

5.63 Sleepfit Solutions explained that current medical device regulation is 'ill-suited to DTx innovations' as 'digital therapies can be developed more quickly than pharmacological products, and benefit from agile development practices with ever faster feedback loops.' It called for the creation of a 'formal approval pathway' for DTx, and suggested that it could be modelled on one introduced by Germany in 2020.[99] BioScience Managers addressed DTx, submitting that:

> By their very nature, DTx are "data-intensive"….Artificial intelligence, machine learning and novel algorithms are now and will continue to be central to DTx. It is imperative that regulatory agencies like TGA build internal data science and software coding skills to evaluate and approve DTx.[100]

5.64 As mentioned above in the discussion of alignment with overseas regulators, the RANZCR commented on some of the issues surrounding AI. It explained that it has been 'working on AI in radiology since 2016,' and outlined eight 'Regulatory Principles' it has developed for the regulation of such AI.[101] It noted that unlike traditional medical devices 'machine learning systems and artificial intelligence tools are not static and can learn post release and change.'[102] It therefore argued that they should be regulated 'more robustly', with a level of evidence required commensurate with the level of risk of the particular device, and it noted the problem with alignment with overseas regulators discussed above. The RANZCR argued that any substantial modifications to the AI model must require fresh authorisation from the TGA, and that ongoing monitoring of AI devices is even more important than for regular devices.[103]

Patient-matched medical devices

98 Sleepfit Solutions, Submission 198, p. [1].

99 Sleepfit Solutions, Submission 198, p. [4].

100 BioScience Managers, Submission 206, p. [2].

101 RANZCR, Submission 204, pp. [1]-[2].

102 RANZCR, Submission 204, p.

103 RANZCR, Submission 204, p. [2].

5.65 3DMediTech, a manufacturer of 'patient-matched 3D printed devices,' made a submission on the regulation of patient-matched devices.[104] These devices, known as personalised medical devices, are defined as a device that:

> (a) is manufactured by the manufacturer, within a specified design envelope, to match:
>
> (i) either or both of the anatomical and physiological features of a particular individual; or
>
> (ii) a pathological condition of a particular individual; and
>
> (b) is designed by the manufacturer (even if the design is developed in consultation with a health professional); and
>
> (c) is manufactured using production processes that are capable of being:
>
> (i) either or both validated and verified; and
>
> (ii) reproduced.[105]

5.66 3DMediTech noted the TGA's creation of a new registration pathway for such devices, implemented in 2021, which separates them out from the 'custom-made' medical device category which is 'subject to a less prescriptive regulatory regime.' It praised this reform as striking 'an extremely effective balance' between protecting patient safety and supporting business.[106] It asked however that the transitional arrangements for the new pathway be altered so that the list of devices allowed to remain regulated as custom-made devices as a transitional measure be made public, to allow for more transparency.[107]

Medicinal cannabis

5.67 MedReleaf Australia submitted that the current regulatory system 'does not have the structure or ability to appropriately review medicines that are whole plant cannabis,' as opposed to a single chemical compound. It

104 3DMediTech, Submission 111, p. 2.

105 *Therapeutic Goods (Medical Devices) Regulations 2002* (Cth), Dictionary.

106 3DMediTech, Submission 111, p. 3.

107 3DMediTech, Submission 111, pp. 4-5.

recommended that 'a new class of registration be implemented that is more appropriate in assessing medical cannabis.'[108]

Committee Comment

5.68 The Committee wishes to record its appreciation for the work the TGA and its staff have undertaken during the COVID-19 pandemic, and thanks it for continuing to engage well with the inquiry despite being under increased pressure.

5.69 The Committee is satisfied that the TGA is performing well in many aspects of its regulatory role, particularly those relating to medicines and medical devices for common diseases. The Committee believes that this is in large part due to two factors: the reforms made following the *Sansom Review*, and a proactive approach to reforming itself further, including actively seeking the views of those affected by its regulatory activities.

5.70 The Committee agrees that the TGA should not adopt a whole scale 'rubber stamping' approach to overseas regulatory approvals. While the Committee acknowledges that much of the evidence it received supported much greater alignment with overseas regulators, it believes that the risks of major change need to be weighed against the potential benefits. However, the Committee does see scope for increased collaboration with Comparable Overseas Regulators (CORs) and an expansion of Project Orbis arrangements for disease consortiums other than cancer.

5.71 The Committee acknowledges that there are access problems for many rare diseases, and encourages the TGA to work on improving those through increasing its alignment with international regulators where relevant. In particular, the Committee believes it is an unsatisfactory situation that cancer patients have the benefit of Project Orbis, but patients of non-cancer rare diseases have no equivalent. It therefore urges the TGA to try to remedy this disparity.

5.72 The Committee urges the Australian Government to reconsider the current cost recovery funding model for the TGA within the Department of Health. The Committee sees merit in increasing funding for staffing levels and expertise within the Department of Health to ensure the TGA can manage an increasing number of submissions in the near future and to expand competencies in horizon scanning for new medicines and technologies. The

108 MedReleaf Australia, Submission 189, pp. [3]-[4].

Committee welcomes the extra funding the Australian Government has recently provided, and urges the Australian Government to provide further funding to ensure that the TGA's workforce is staffed sufficiently to meet workloads and that the IT system is able to deal with an increased number of submissions in the future.

5.73 The Committee acknowledges that the cost recovery model works well for therapies for more common diseases, and believes that it could be used to support the publication of Australian Public Assessment Reports (AusPARs) at the same time as the launch of a medicine, to ensure clinicians and their patients are as well informed as possible.

5.74 The Committee believes there is merit in the suggestions that the TGA should adapt its processes to enable the approval of therapeutic goods by molecular indication as well as by disease indication. The Committee acknowledges that this is a highly complex issue, that such a change may involve substantial effort on the part of the TGA and that it will have repercussions for other elements of the development and approval process such as clinical trials and reimbursement. However, the Committee believes this will be an area of growing importance into the future and the TGA should adapt its processes accordingly.

5.75 The Committee recommends the TGA aligns its processes with the PBAC's for parallel processing purposes and its communication with sponsors during the assessment process. The Committee notes the growing importance of Real World Evidence (RWE) and welcomes the TGA's commitment to produce more detailed guidance on its use.

5.76 The Committee noted the FDA's Breakthrough Devices Program. The Committee supports this idea and recommends that the Australian Government establish a similar program in Australia to support the domestic medical technology sector.

6. Health Technology Assessment and the Pharmaceutical Benefits Advisory Committee

Introduction

6.1 Once a medicine or medical device is granted regulatory approval by the Therapeutic Goods Administration (TGA) it can be marketed by the sponsor, and purchased by patients. However since most patients cannot afford the expense of many new medicines and devices, they must wait until it is reimbursed by the Government, which requires it to undergo Health Technology Assessment (HTA).

6.2 HTA process is conducted by a number of bodies, the most prominent being the Pharmaceutical Benefits Advisory Committee (PBAC) and Medical Services Advisory Committee (MSAC). Many submitters discussed HTA in general rather than one specific body, although their focus was directed towards medicines, and the PBAC was the individual body that attracted the most attention. Consequently this chapter addresses both the evidence concerning the PBAC and that concerning HTA in general, while the following chapter focuses on the evidence concerning the MSAC and related matters, such as the Prostheses List Advisory Committee (PLAC).

Overall performance of the Health Technology Assessment system

6.3 The Committee heard a wide range of views on the performance of the current HTA system, ranging from academics who claimed there is, for the

most part, no problem with access to medicines[1] to pharmaceutical companies who asserted that the system's poor performance is preventing some medicines from being available in Australia at all.[2]

6.4 Nonetheless submitters raised more issues about the performance of the HTA system in general and the PBAC in particular, than the TGA. BioMarin Pharmaceutical Australia (BioMarin) commented that:

> BioMarin's experience, like many sponsors, has demonstrated that the bottle neck for access to new drugs and novel medical technologies in Australia rests not with initial approval by the [TGA], but with the subsequent approval processes for reimbursement, and therefore should be the focus of the inquiry.[3]

6.5 BioMarin argued that 'the expedited processes for TGA registration have not been replicated across the respective reimbursement pathways for human therapeutics in Australia.'[4] Novo Nordisk Oceania (Novo Nordisk) similarly submitted that 'the benefits from these shorter regulatory pathways will continue to go unrealised without PBAC and MSAC pathways also being improved to match the expedited TGA pathways'.[5]

6.6 LEO Pharma had a somewhat different view, namely that the HTA system works well for some types of conditions but not for others. It suggested that certain conditions such as dermatological diseases have been neglected by the Pharmaceutical Benefits Scheme (PBS) while cancer therapies

> …have in the last decade garnered more attention and public interest with sustained advocacy by the patients, clinicians, and the pharmaceutical industry. This has resulted in better support by decision makers and key stakeholders for reimbursement on the PBS.[6]

6.7 BioMarin submitted that:

> Apart from the ability to make a 'parallel' submission to PBAC whilst the sponsor anticipates registration approval from TGA, the Australian registration and reimbursement processes are entirely separate with virtually no alignment of evaluations and approvals. Indeed, even though the TGA

1 Miss Jessica Pace, Submission 40, p. 3.

2 Amgen Australia (Amgen), Submission 82, p. 3.

3 BioMarin Pharmaceutical Australia (BioMarin), Submission 152, p. 1.

4 BioMarin, Submission 152, p. 1.

5 Novo Nordisk Oceania (Novo Nordisk), Submission 151, p. 3.

6 LEO Pharma, Submission 202, p. 2.

> assesses the safety and clinical efficacy of a medicine for the purposes of registration, the PBAC performs yet another evaluation of safety and efficacy.[7]

6.8 Novartis Australia and New Zealand (Novartis) recommended the creation of:

> …a forum for parallel and/or joint pre-submission consultation between sponsors and with all key decision-makers (regulators and payers) and a single format and point of entry for the subsequent submission covering all evidentiary requirements for novel therapies in areas of urgent clinical need.[8]

Length of review for assessment and resubmissions

6.9 There was a widely held view among submitters that the HTA system currently takes too long to provide access to medicines.[9] Many submitted that there is a need to 'streamline' the HTA system, before going on to make more specific recommendations.[10] A particular concern was how often multiple submissions are required for a medicine to receive a positive recommendation from the PBAC.[11] In the words of Professor John Zalcberg OAM, Chair, Australian Clinical Trials Alliance (ACTA):

> …we face sometimes years of backwards and forwards with resubmissions, minor submissions and major submissions going on. In the period when these resubmissions are occurring, sometimes over a year or two or more, patients don't have access, and that is a problem.[12]

6.10 The Department of Health (the Department) advised that 29 per cent (38 out of 132) of first time submissions considered by the PBAC between its March

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7 BioMarin, Submission 152, p. 5.

8 Novartis Australia and New Zealand (Novartis), Submission 138, p. [11].

9 For example: Merck Sharp & Dohme Australia (MSD), Submission 63, p. 2; Better Access Australia (Better Access), Submission 160, p. 4.

10 AstraZeneca Australia (AstraZeneca), Submission 42, p. 2; Medical Oncology Group of Australia and Private Cancer Physicians of Australia (MOGA and PCPA), Submission 50, p. 4; Albireo Pharma, Submission 59, p. [2]; Gene Therapy Advisory Steering Group, Sydney Children's Hospital Network, Submission 102, p. [3]; Western Australian Department of Health, Submission 129, p. [7]; Novartis, Submission 138, p. [11]; Bayer Australia and New Zealand (Bayer), Submission 175, p. 3.

11 Specialised Therapeutics Australia (STA), Submission 7, p. 20; Mr Michael Smith, Submission 13, p. 6; MOGA and PCPA, Submission 50, p. 1; Rare Disease Industry Working Group (RDIWG), Submission 51, p. 5; UCB Australia (UCB), Submission 74, p. 4; BioMarin, Submission 152, p. 1.

12 *Committee Hansard*, Sydney, 7 May 2021, p. 46.

2020 and March 2021 meetings inclusive received a 'not recommended' outcome.[13] Dr Haitham Tuffaha explained that his research on the 179 new cancer drug submissions between 2010 and 2018 showed that positive recommendations were made for only 37 per cent of submissions, with drugs taking an average of 2.1 submissions for approval.[14]

6.11 The Department noted that there has been a substantial reduction in processing times since the implementation of various process reforms from 1 July 2019.[15]

6.12 The PBAC itself provided a suggestion to further streamline the process, which it explained as follows:

> The establishment of the PBAC Executive consisting of the Chair, Deputy Chair and Chairs of the Drug Utilisation and Economic sub-committees provides an opportunity for further efficiency in PBS processes.
>
> This would be enhanced if decisions around some matters could be formally delegated to the PBAC Executive.
>
> This could include approvals for Section 19A exemptions for medicine shortages, changes in dispensed amounts, and changes to doses or minor changes to product content in the case of nutritional food products.[16]

Flexibility

6.13 A related criticism was that the current system lacks flexibility, particularly in the face of increasingly advanced medicines and technologies. Pfizer Australia (Pfizer) explained that:

> The emergence of innovative, targeted therapies has tested the limits of our [HTA] process and created tension between assessors, industry and patients. Attempts to address this have led to increasing layers of red tape. The result is a system that is increasingly complex, rigid and costly.[17]

6.14 Medicines Australia similarly submitted that:

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13 Department of Health, Submission 15.4, p. [1].

14 Dr Haitham Tuffaha, Submission 72, p. [1].

15 Department of Health, Submission 15, pages 31-32.

16 Department of Health, Submission 15.3, p. 6. The section in question is apparently s 19A *Therapeutic Goods Act 1989* (Cth).

17 Pfizer Australia (Pfizer), Submission 137, p. [2].

> An emerging issue relates to the lack of flexibility in funding assessment pathways. For some medicines, there appears to be no pathway at all, which acts as a brake on both innovation and access. For others, even as approaching regulatory approval, there is no clarity on the funding pathway.[18]

6.15 The Australian Cardiovascular Alliance (ACvA) recommended that the 'flexibility of processes' be improved to 'fast-track urgent medicines and devices,' a sentiment echoed by AstraZeneca.[19] Bayer Australia and New Zealand (Bayer) put forward a similar view, and submitted that 'no truly innovative medicines can receive a positive recommendation from reimbursement agencies without more flexibility in assessment methodologies.'[20] Novo Nordisk likewise suggested there is a need for 'flexibility, transparency and adaptability in assessment and funding for new technologies where there are no defined pathways.'[21]

6.16 Albireo Pharma identified flexibility as a particular requirement for assessment of rare diseases.[22] Merck Sharp and Dohme Australia (MSD) suggested that the PBAC is less flexible than equivalent overseas bodies, but emphasised that flexibility 'needs to be built around clear and transparent processes backed by independent scientific method.'[23] Mr Stuart Knight, General Manager, Roche Australia (Roche), told the Committee:

> If I could leave you with one word, I think it would be just to make our system more flexible so that the processes that we have are more capable of dealing with uncertainty. For the data that is not perfect, how are we going to deal with that? How do we work through that together? Where there's inflexibility is where we are having problems.[24]

Interaction with hospitals

18 Medicines Australia, Submission 141, p. 37

19 Australian Cardiovascular Alliance (ACvA), Submission 76, p. 13; AstraZeneca, Submission 42, p. 4.

20 Bayer, Submission 175, p. 6.

21 Novo Nordisk, Submission 151, p. 4.

22 Albireo Pharma, Submission 59, p. [2].

23 MSD, Submission 63, p. 3.

24 *Committee Hansard*, Sydney, 7 May 2021, p. 27.

6.17 Another difficulty that was raised with the current system was the potential for inconsistency where hospitals are involved. Alexion Pharmaceuticals Australasia (Alexion) submitted that:

> …there is no clear assessment or funding pathway for Rare Disease treatments that need to be initiated as inpatient supply at the time of diagnosis but transition to chronic management in the outpatient setting post the acute event. It is recommended to have a clear and transparent pathway documented for highly specialised drugs that are initiated in tertiary hospitals, but the patient's chronic management continues in an outpatient setting.[25]

6.18 The Medical Oncology Group of Australia and Private Cancer Physicians of Australia (MOGA and PCPA) likewise expressed concern that 'the different coverage of on-label and off-label indications in hospital and PBS formularies may affect the continuity and affordability of treatment for patients.'[26]Meanwhile Amgen Australia (Amgen) wrote that:

> Many new cancer medicines are very effective, very quickly, in reducing the size of a tumour, or the number of tumorous cells. These medicines have potential side effects such as Tumour Lysis Syndrome (TLS) or Cytokine Release Syndrome (CRS) which have symptoms which may need treatment in hospital.
>
> Therefore, it is appropriate for these patients to have their first treatment in hospital. As hospitals are state run, the patient may not be eligible for PBS-subsidised medicines. Amgen recommends that the federal and state governments work together to ensure equitable access to these new and highly efficacious medicines in an appropriate clinical setting.[27]

6.19 The evidence received by the Committee was limited but not particularly positive on the issue of how HTA or HTA-like processes are conducted for the hospitals themselves. The Australian Healthcare and Hospitals Association (AHHA), which represents public and non-profit hospitals amongst others, submitted:

> Currently in Australia, processes differ across jurisdictions and public hospitals in relation to how new technologies are assessed and implemented, making it difficult to know if the technology leads to better patient outcomes at an efficient cost.

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25 Alexion Pharmaceuticals Australasia (Alexion), Submission 30, p. 9.

26 MOGA and PCPA, Submission 50, p. 3.

27 Amgen, Submission 82, p. 8.

> As noted in the Addendum to the *National Health Reform Agreement 2020-2025*…the current approach to health technology assessment to inform investment and disinvestment decisions in Australia is fragmented and does not facilitate coordinated and timely responses to rapidly changing technologies. Separate processes exist across all levels of the health system, which has the potential to duplicate effort, create inefficiencies and inconsistent advice, and delay access to innovative and emerging technologies.[28]

6.20 This view was supported by the private sector. Stryker South Pacific argued that:

> The current processes for assessing new health technologies in public hospitals differ vastly across states, territories and public and private health systems, leading to inequities in access between the public and private health systems...
>
> There needs to be a clear and consistent approach across governments, health services and clinicians to ensure that evidence to support the value of new technologies can be demonstrated in terms of both costs and patient outcomes.[29]

6.21 Edwards Lifesciences added that:

> Separate processes exist across all levels of the health system, which has the potential to duplicate effort, create inefficiencies and inconsistent advice, and delay access to innovative and emerging technologies. We would welcome a coordinated national approach but not at the expense of speed to market.
>
> Currently the ability to provide new technology to the public hospital system is more flexible and not exclusively dependent on MSAC approval. However, we would be concerned if a national coordinated HTA process meant that state hospital systems stop purchasing new technology unless it had an MBS item. This could potentially further slow access of new technology to Australian patients.[30]

Coordination within Government

6.22 The complexity of the Government's system for providing access to medicines and medical devices was reflected in the fact that many submitters felt that the different parts of the system need to coordinate better

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28 Australian Healthcare and Hospitals Association (AHHA), Submission 68, p. 1.

29 Stryker South Pacific (Stryker), Submission 28, p. 5.

30 Edwards Lifesciences, Submission 83, p. 34.

with each other, and indeed the fact that many nominated different parts to be involved in this coordination. AstraZeneca, for example, addressed the 'HTA committees,' recommending 'improvements to the cross-talk/ coordination' between them.[31]

6.23 AbbVie submitted that:

> Early dialogue between the TGA and PBAC, for orphan medicines, paediatric oncology medicines and advanced therapies for rare diseases where the patient population is small, would be particularly beneficial.[32]

6.24 The Victorian Comprehensive Cancer Centre recommended 'dialogues between market authorisation and Health Technology Assessment, so the clinical evidence is efficiently used by regulatory and reimbursement agencies' and 'early dialogue and alignment' between the TGA and PBAC.[33] Alexion commented:

> As TGA assesses safety, efficacy and quality, the existing PBAC/MSAC evaluation process for drugs/therapies duplicates that assessment. The PBAC/MSAC could have their roles changed to determining:
>
> - Restriction criteria; and
> - Managed entry requirements[34]

6.25 AusBiotech made its submission in more general terms, and called for:

> Alignment and harmonisation of registration and reimbursement frameworks and better connection within the…TGA and Health Assessment workforces to expedite approvals for therapeutic products that cut across a number of disciplinary practices.[35]

6.26 Dr Tuffaha argued that 'better alignment is required between the registration and reimbursement processes. Parallel submissions to TGA and PBAC should be encouraged and facilitated through active engagement

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31 AstraZeneca, Submission 42, p. 4.

32 AbbVie, Submission 180, p. [4].

33 Victorian Comprehensive Cancer Centre, Submission 61, p. 5.

34 Alexion, Submission 30.1, p. [2].

35 AusBiotech, Submission 114, p. 3.

between sponsors and PBAC.'[36] The ACvA supported more use of parallel processing.[37]

6.27 The MOGA and PCPA made one of the strongest submissions on this issue, writing that:

> The governance culture and silo-approach within various authorities and government departments need to be challenged and a single, coordinated agency and decision-making process with supporting legislation is required to achieve greater process efficiency. We strongly recommend legislative reform that combines the TGA and PBAC process and MSAC process when appropriate.[38]

It recommended 'harmonisation of evidentiary requirements between regulatory and reimbursement authorities.'[39]

6.28 Better Access Australia (Better Access) criticised the 'assessment of subsidies by different committees noting the convergence of technologies is confounding the arbitrary placement in the subsidy assessment process.' It suggested the system be reviewed to investigate 'the viability of creating a single assessment system combining the skills and expertise of the various committees to be deployed as needed for the technology or treatment.'[40] ARCS Australia likewise claimed that 'another area where barriers to accessing new medicines and devices exist is in the separation of funding pathways between the PBS (PBAC) and MSAC.'[41]

6.29 Medicines Australia commented that 'the lack of integration and predictability across the regulatory and reimbursement processes involving multiple bodies extends timelines needed to reach an outcome that enables patient access.' In response it recommended the creation of a 'joint [(TGA]; [PBAC]; [MSAC], Australian Technical Advisory Group on Immunisation (ATAGI) pre-submission advice framework to improve alignment of end-to-end processes.'[42]

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36 Dr Tuffaha, Submission 72, p. [1].

37 ACvA, Submission 76, p. 6.

38 MOGA and PCPA, Submission 50, p. 4.

39 MOGA and PCPA, Submission 50, p. 4.

40 Better Access, Submission 160, pages 6-7.

41 ARCS Australia, Submission 41, p. 5.

42 Medicines Australia, Submission 141, p. 32.

6.30 Novo Nordisk supported a 'joint [TGA]/[PBAC] pre-submission advice framework to ensure alignment of end to end processes' as well as 'collaborative meetings between TGA, PBAC and MSAC becoming the standard approach for bringing new or novel technologies to Australia.'[43]

6.31 Specialised Therapeutics Australia (STA) made the same point about the need for 'collaborative meetings' between the HTA bodies. It argued:

> That Australia's subsidy systems need to be aligned with the same timeframes of certainty and transparency as the TGA, and further, that the role of the TGA in determining safety and efficacy should be given higher weighting by the MSAC and PBAC.[44]

6.32 In its submission the Department highlighted its new Health Products Portal (HPP), which it suggested would greatly assist with coordination within Government:

> The HPP Program vision is to realise a single, secure and easy to use place where industry can interact with Government to apply, track, pay and manage listings for regulated and subsidised health-related goods and services. The aim of the HPP is to create consistent and simplified business processes through a digital solution that supports legislative compliance and evidence-based policy and decision making.
>
> This digital solution will provide a consistent user experience for sponsors and other stakeholders, reducing duplication of effort and enabling a single, digital and trackable user journey through the regulatory and subsidisation lifecycle. Further, it will create a cohesive end-to-end HTA process, where information is gathered at any stage of the process with a view to its purpose, its use and reuse throughout, and availability at the right time. This will streamline and improve the process and efficiency in which medicines and medical devices enter the Australian market. The HPP has already enabled a streamlined approach for PBAC submissions, and over time, will link data and services to include other areas including TGA, PLAC and MSAC.[45]

International cooperation and harmonisation

6.33 Many submitters recommended that international cooperation and harmonisation should be increased in Australia's HTA system more

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43 Novo Nordisk, Submission 151, p. 3.

44 STA, Submission 7, p. 5.

45 Department of Health, Submission 15, p. 33.

generally.[46] The MOGA and PCPA submitted that 'the national approval process for new drugs and novel medical technologies must be made more efficient and responsive to international best practice' and recommended 'continuing to align the Australian system with international approval processes where possible.'[47] Medicines Australia encouraged the Government to 'modernise and improve HTA evaluation processes in line with international best practice HTA.'[48]

6.34 Many submitters drew a contrast between what they viewed as the significant progress the TGA has made in improving its cooperation with international regulators in recent years, and the lack of comparable progress by the PBAC and other HTA bodies, and urged the latter to learn from the former.[49] AbbVie, for example, after praising the work the TGA has done to improve its international cooperation recently, noted that this has lagged for HTA, submitting that 'there is an opportunity for the reimbursement pathway to adopt similar concepts to Project Orbis to accelerate access to medicines.'[50]

6.35 The Macquarie University Centre for the Health Economy (MUCHE) explained that currently 'submissions to the PBAC must report relevant published economic evaluations involving the proposed drug or similar drugs, including those considered by other HTA agencies or committees.'[51] It stated that the benefits of the PBAC considering other HTA bodies' decisions include 'insights into whether the drug was approved only in sub-populations due to greater efficacy or safety concerns, economic model structure and inputs, and key drivers of the results.' It noted however that there are various differences between countries that need to be taken into consideration, including in population characteristics, comparators, clinical practices, health system costs, cost effectiveness thresholds and weighting between cost effectiveness and other criteria. It recommended that 'PBAC

46 Mirum Pharmaceuticals, Submission 10, p. [1]; Australasian Sleep Association, Submission 16, p. 5; Sanofi, Submission 99, pages 4-5; The George Institute for Global Health, Submission 105, p. 8; Johnson & Johnson, Submission 134, p. 13; Eli Lilly Australia (Eli Lilly), Submission 140, p. [2].

47 MOGA and PCPA, Submission 50, p. 3.

48 Medicines Australia, Submission 141, p. 41.

49 STA, Submission 7, p. 5; BioMarin, Submission 152, p. 5.

50 AbbVie, Submission 180, p. [3].

51 Macquarie University Centre for the Health Economy (MUCHE), Submission 62, p. 6.

should consider, but not rely on, the deliberations made by other HTA agencies or committees if available, including funding recommendations.'[52]

6.36 The MUCHE noted that benefits of increased collaboration between HTA agencies include 'reduced resources incurred by sponsors/applications to generate evidence and HTA agencies in assessing evidence; and improved transparency and timeliness in decision making,' and that such collaboration is already occurring overseas, such as through the European Network for Health Technology Assessment. It recommended the Committee 'consider the need for increased collaboration between PBAC and other HTA agencies, including the harmonisation of PBAC methods guidelines.'[53]

6.37 Dr Falk Pharma Australia commented that for medicines for rare diseases:

> …it would simplify PBS submissions if economic modelling used in other countries could be used here, rather than creating Australian-specific ones. Naturally, it is accepted that these models would need updating with local population and prevalence data (if separately available). These models are expensive to create and are generally a re-configuration of data previously reviewed in these other markets.[54]

6.38 Medicinal Cannabis Industry Australia (MCIA) explained that Australia has 'lagged some other parts of the world' in the approval and use of medicinal cannabis, and consequently recommended 'enabling drugs that have been given approval overseas in jurisdictions equivalent to Australia to be fast-tracked for approval in Australia.'[55]

6.39 The PBAC noted in its submission to the inquiry that the TGA's work in this area in recent times, and commented that:

> The PBAC is interested in examining how similar types of sharing of health technology assessments could be implemented with other reimbursement authorities. Health technology assessments require more inputs that are country specific, such as local clinical practice, costs and availability of other therapies and supports, so there will always be a need for Australian specific assessments. However, there are elements that are likely to be very similar across countries.

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52 MUCHE, Submission 62, p. 7.

53 MUCHE, Submission 62, pages 7-8.

54 Dr Falk Pharma Australia, Submission 17, p. [2].

55 Medicinal Cannabis Industry Australia (MCIA), Submission 75, pages 1, 3.

> A barrier to this is the confidentiality arrangements that companies have with different countries. While the PBAC understands the sponsor's reason for this in relation to pricing aspects, there would still seem to be substantial room for sharing of other aspects including economic modelling. Economic inputs would need to be adjusted to reflect country specific clinical practice, comparators and healthcare resource costs. It would appear to the PBAC that some global sponsors sometimes already use common models in their submissions to the PBAC.[56]

Measuring Pharmaceutical Benefits Advisory Committee performance

6.40 Amgen asserted that 'unlike other major areas of public health expenditure (Commonwealth or State), no data are currently collected and published by the Government' on how long medicines are taking to be listed on the PBS after their registration on the Australian Register of Therapeutic Goods or on the broader performance of the PBS. It argued that:

> Australian patients and taxpayers need to know how long they are waiting for access to the safe and effective medicines that they need. Data against a well-defined set of metrics are fundamental to both good and accountable government and well managed businesses. The collection and publication of such performance data would bring the PBS in line with other major areas of healthcare expenditure and delivery.[57]

6.41 Sanofi similarly recommended the implementation of 'an open and transparent tracking system designed to measure speed to access from registration to reimbursement for new therapies.' It suggested that 'this system should include benchmarks to other comparable countries and healthcare systems.'[58]

Reviewing the system

6.42 Many stakeholders called for a wide-ranging review into the HTA system. Roche stated that 'a review of the HTA processes and methods that will be challenged by precision medicine technologies is required.'[59] Biotronik submitted that:

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56 Department of Health, Submission 15.3, p. 6.

57 Amgen, Submission 82.5, p. 2.

58 Sanofi, Submission 99, p. 5.

59 Roche Australia (Roche), Submission 92, p. 13.

> …the Health Technology Assessment resources within Australia [are] in need of a whole of health review. This process was last under review back in 2011 and one of its key recommendations was to revisit the landscape every three years, which the government of the day remained silent about.[60]

6.43 Sanofi argued that:

> …the review of the National Medicines Policy (NMP) provides the ideal mechanism to achieve the integrated and comprehensive reform required to ensure Australia's approval processes remain efficient, fit-for-purpose and equipped to appropriately inform decision-making about how best to allocate investment to optimise health outcomes for all Australians.[61]

6.44 LEO Pharma and Better Access made similar comments on the need for the review of the National Medicines Policy to be used for such a purpose, with the latter suggesting lessons should be learned from the review announced in November 2020 by the United Kingdom's (UK) National Institute for Health and Care Excellence (NICE).[62]

6.45 Amgen reiterated the claim that there is an incongruity between the performance of the TGA and that of the HTA system in its argument that:

> The Australian Government recently implemented reforms to the TGA based on recommendations made by an Independent Expert Panel Review. Many of these reforms are explicitly designed to speed up access to medicines. Nonetheless, the TGA is effectively only one-half of the access system in Australia and therefore the reform of its processes has achieved only half the job. Amgen believes that a companion Independent Expert review focussed on the PBS … is required.[63]

6.46 Amgen emphasised that a review should look not just at 'the HTA methods used by the PBAC in its evaluation and decision-making' but include the 'processes, timelines and the relationship between patient access and finalisation of PBS listing terms with sponsors.'[64]

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60 Biotronik Australia (Biotronik), Submission 130, p. [5].

61 Sanofi, Submission 99, p. 1.

62 LEO Pharma, Submission 202, p. 6; Better Access, Submission 160, pages 21-22.

63 Amgen, Submission 82.5, p. 2.

64 Amgen, Submission 82.5, p. 2.

6.47 The Department advised that the review of the NMP was to commence in August 2021, chaired by Professor Michael Kidd AM.[65] On 7 September 2021 the Government announced that it would conduct a comprehensive Health Technology Assessment Policy and Methods Review as part of its new five year Strategic Agreements with Medicines Australia and the Generic and Biosimilar Medicines Association, discussed in Chapter 2.[66]

The application process

Engagement with sponsors

6.48 There were strong views among submitters that more pre-submission engagement is required, both from the HTA system in general and the PBAC in particular. The Rare Disease Industry Working Group (RDIWG) submitted that:

> Earlier engagement with the Department of Health would be welcomed by Industry in order to be able to identify the appropriate reimbursement pathway, provide the patient voice and establish clinical need so that all parties facilitate the path to access without increasing submission churn.[67]

6.49 It called for the PBAC pre-submission process for rare and ultra-rare diseases to be 'enhanced,' including by involvement of a Life Saving Drugs Program (LSDP) representative in the case of medicines that may be eligible for the LSDP.[68]

6.50 Noting the complexity of HTA for rare disease medicines in particular, Takeda Pharmaceuticals Australia (Takeda) argued that 'a central entry point for discussions on HTA for rare diseases could improve the efficiency of review by initiating a discussion on the current standard of care and the unmet medical need earlier in the review process.'[69]

6.51 MSD took a more general view, stating that:

> Those with a stake in HTA should be involved, including industry, to develop a collaborative approach to assessment. In particular, broad involvement can

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65 Department of Health, Submission 15.6, p. [5].

66 The Hon Greg Hunt MP, Minister for Health and Aged Care, 'Landmark new medicines agreements to bring significant benefits for Australian patients', *Media Release*, 7 September 2021.

67 RDIWG, Submission 51, p. 5.

68 RDIWG, Submission 51, p. 6.

69 Takeda Pharmaceuticals Australia (Takeda), Submission 66, p. 5.

> facilitate the exchange of information in confidence to ensure the reviewer has complete clinical, epidemiologic, and economic information to formulate a review.[70]

6.52 LEO Pharma argued that 'genuine and active engagement between the PBAC and companies to better understand the requirements and expectations would lead to fewer first time rejections and better informed decision making by the PBAC.' It stated that the current hour-long pre-submission meeting (for which the PBAC charges $15,800) does not provide 'sufficient clarity' and does not include PBAC decision-makers. It recommended that the Chair of the PBAC attend the meeting 'for therapy areas where the PBAC lacks understanding of new treatments and where an area of disease has not had a recommendation in the last 5 years.'[71]

6.53 STA described the current rules governing engagement between a sponsor and the PBAC as 'very strict,' which it said 'effectively prevents open, frank dialogue with evaluators once a submission has been lodged.' To remedy this it recommended the provision of 'additional opportunities for rigorous and robust engagement prior to the submission decision (rather than at a post-decision meeting).'[72]

6.54 There were concerns about the general tenor of the relationship between industry and the Department. Shawview Consulting submitted that:

> The level of constructive engagement, or 'vibe', in the dialogue has waxed and waned over the years. My sense is that the day-to-day relationship between government officials and industry on PBS policy and process issues is today more transactional and less solution-focussed than in the past. This may be an understandable response to budgetary pressures, industry and business changes and the changing dynamics of Australia's place in the global health landscape, but the result is that many interactions between government and industry are short-term exercises in cost-saving and damage control. I encourage government officials to embrace a more cooperative, solution-focussed, appropriate, professional, long-term relationship with industry.[73]

6.55 This was a view shared by Omico, which argued:

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70 MSD, Submission 63, Appendix A, p. 4.

71 LEO Pharma, Submission 202, p. 5.

72 STA, Submission 7, p. 18

73 Shawview Consulting, Submission 181, p. 9.

> It is also envisioned that greater public sector-industry collaboration based around coordinated, mutually compatible areas of expertise and investment, could give rise to enhanced opportunities for negotiations between government and industry in regard to drug pricing, compared to the current adversarial model. Since there is no future for therapeutics in general that will not depend on industry for drug development, a collaborative rather than adversarial model for innovative health systems is both logical and desirable.[74]

6.56 The MUCHE, meanwhile, suggested that smaller pharmaceutical companies in particular 'may also see the process as combative rather than one of information seeking and negotiation.'[75]

6.57 Novo Nordisk took a broader view, and recommended 'dialogue between industry and the PBAC to consider future policy issues to guide the HTA process.'[76] Medicines Australia submitted that:

> In the past, there was regular dialogue between Medicines Australia and the PBAC on issues of importance to the HTA process. Medicines Australia believes the re-introduction of such a dialogue would be beneficial, given the lack of certainty for new therapies in terms of HTA assessment.[77]

6.58 Biotronik made a similar proposal:

> A government instigated regular 'Innovation Forum' to bring manufacturers, payers and providers together could act as a platform for dialogue, mutual understanding and sensible decision making to bring clinical innovations with health-economic benefit to market sooner.[78]

Fees

6.59 Many pharmaceutical companies expressed unhappiness with the current PBAC fee regime, particularly as it applies to medicines for rare diseases. Novartis explained that while medicines granted an orphan drug designation by the TGA are fee exempt for their first submission to the PBAC, since June 2019 full fees have been payable for any subsequent resubmission; it is possible to request further exemptions but 'this is uncertain as it can only be requested during the process.' It submitted that

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74 Omico: Australian Genomic Cancer Medicine Centre, Submission 184, pages [1]-[2].

75 MUCHE, Submission 62, p. 2.

76 Novo Nordisk, Submission 151, p. 3.

77 Medicines Australia, Submission 141, p. 40.

78 Biotronik, Submission 130.1, p. [1].

'an expansion of the criteria for a fee exemption, which is known in advance of the PBAC submission would provide certainty and clarity for sponsors about the potential costs.'[79]

6.60 UCB Australia (UCB) and MSD merely raised the limited exemption as a problem, but other companies wanted it expanded:[80] Recordati Rare Diseases Australia (RRDA) proposed the first two to three applications;[81] STA nominated two major and one minor submissions;[82] and Amicus Therapeutics recommended five years.[83]

6.61 Bayer proposed extended the exemptions beyond designated orphan drugs to drugs that treat 'diseases affecting small populations (but not given orphan designation).'[84]

6.62 Novartis expressed concern that the current orphan definition does not capture 'personalised medicines' that 'breakdown a previously large homogenised patient population for say lung cancer, into smaller population subsets that are heterogeneous,' and accordingly recommended 'a review of the existing criteria or new fee exemption criteria for personalised innovative medicine.'[85]

6.63 There was discussion of the possibility of deferring payment of fees until after reimbursement is granted, which was supported by BioMarin for all applications for orphan medicines.[86] STA suggested this should apply for 'at least the first two applications' for any medicine for companies with revenue less than $50 million per annum, with fees to be paid in instalments once the PBS expenditure on the medicine exceeds $3 million per annum.[87]

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79 Novartis, Submission 138, p. [8]-[9].

80 MSD, Submission 63, p. 4; UCB, Submission 74, p. 4.

81 Recordati Rare Diseases Australia (RRDA), Submission 3, p. [2].

82 STA, Submission 7, p. 21.

83 Amicus Therapeutics, Submission 31, p. 4.

84 Bayer, Submission 175, p. 4.

85 Novartis, Submission 138, p. [8].

86 BioMarin, Submission 152, p. [2].

87 STA, Submission 7, p. 20.

6.64 Noxopharm Limited proposed 'deferral of payment of fees for Australian-owned companies would permit early-stage companies to begin to receive revenue before paying off the balance of submission fees.'[88]

6.65 Better Access recommended 'considering fee processes and payment plans commensurate with the size of the company.'[89] RRDA argued that fee relief should be 'means tested,' meaning companies below a certain annual turnover – it nominated $50 million – would receive the exemptions, but larger companies would not. It based this argument on the claims that this would provide more resources to the PBAC and TGA, larger companies do not need the exemptions, and they would not be deterred from making orphan drug applications because 'their business now depends on sales of orphan drugs.'[90]

6.66 By contrast the RDIWG suggested that this be done on a medicine by medicine basis, arguing 'consideration of implementation of a fee structure based on budget impact would increase access to treatments for very small populations.'[91]

6.67 The Department advised that it 'previously has been asked to consider a sliding scale of fees based on company size,' although it is bound by the Government's cost recovery policies. It noted that 'the fees charged reflect the costs and efforts undertaken by the Department, commensurate with each submission type, regardless of company size.'[92] On the question of fee waivers, it commented:

> Fee exemptions apply to all applications that meet the criteria set out in the Regulations. Fee waivers are granted at the discretion of the Secretary or a delegate where an applicant demonstrates that their application is in the public interest and that cost recovery fees would genuinely make the application financially unviable.[93]

6.68 Professor Andrew Wilson (Prof Wilson), Chair of the PBAC, made the following comments on the issue of the PBAC's fees and resourcing:

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88 Noxopharm Limited, Submission 70, p. 3.

89 Better Access, Submission 160, p. 7.

90 RRDA, Submission 3, p. [2].

91 RDIWG, Submission 51, p. 5.

92 Department of Health, Submission 15.6, p. [8].

93 Department of Health, Submission 15.6, p. [9].

> I think it's inappropriate for me to comment on whether cost recovery, which is a government policy, is working. It depends a little bit on what you mean by 'working'. It has little impact on what we receive…I've already flagged that, if we want to enhance our consumer-involvement processes even further, that is an area where I think we need to think about resourcing. As flagged…if we want to allow for some alternative pathways for submission and some more active surveillance of need, there would need to be additional capacity to do that. The existing system keeps up with the submissions based process, but, if you were to add additional, unfunded applications to be made, that would be very challenging.[94]

Provisional access

The existing system and opportunities for change

6.69 A significant issue raised by the pharmaceutical sector was the need to strengthen and expand provisional subsidised access to medicines, currently provided through managed access programs.

6.70 Bristol Myers Squibb Australia (BMS) explained that these programs '…are intended to allow listing when the clinical data remains incomplete, potentially speeding up the approval process; however, agreeing [one] can be complex and does not necessarily address the gap between regulatory and reimbursement approvals.'[95] Johnson & Johnson stated that:

> The 'Managed Access Program' (MAP) framework is an existing mechanism which is intended to facilitate access to new therapies in areas of high clinical need. Whilst the uptake of this mechanism has been very limited, there is an opportunity for this framework to be re-invigorated and adapted to better support the medicines access needs of patients. Therefore, it is recommended that the existing MAP framework be formally reviewed in consultation with relevant stakeholders.[96]

6.71 Novartis noted the problem that evidence that is sufficient for a medicine to receive provisional approval from the TGA is often insufficient for it to receive a positive recommendation from the PBAC, including for a MAP,

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94 *Committee Hansard*, Canberra, 24 June 2021, p. 9.

95 Bristol Myers Squibb Australia (BMS), Submission 118, p. [18].

96 Johnson & Johnson, Submission 134, p. 10.

meaning the provisional approval does not actually provide access any faster.[97] Similarly, Roche submitted that:

> There is similar need for alignment for the managed entry scheme [i.e. MAPs] to be more flexible with parallel filing to the TGA and PBAC for new medicines that have substantial benefit based on early data. With the TGA provisional pathway fast tracking evaluations, alignment of these timeframes with the PBAC processes will reduce delays and provide greater certainty for sponsors. This will help sponsors to navigate the regulatory and reimbursement processes in the most efficient way possible.[98]

6.72 The RDIWG argued that many new technologies may provide long-term benefits for patients and consequently 'may have limited data at the time of assessment.' It urged that 'there should be a focus on the development of innovative access mechanisms to ensure patients have the advantage of being able to access treatment in parallel to the long-term collection of [RWE].'[99] Many other submitters were supportive of similar ideas, emphasising the opportunity for RWE collection.[100] BMS, which claimed that it had participated in Australia's 'first significant managed access scheme' for a melanoma drug, submitted that:

> …conditional approvals could reference, rather than duplicate, the post-marketing studies being conducted in other countries….In some cases, it may be necessary to conduct local studies, although this should be justified on an exceptional basis, rather than assumed for all cases.[101]

6.73 It argued that relying on overseas studies would reduce the administrative burden on doctors and lower costs for sponsors, thereby encouraging them to bring their medicines to Australia.[102]

6.74 Companies such as AstraZeneca, MSD, Johnson & Johnson, Pfizer, Novartis and LEO Pharma stressed the importance of the risk of this type of

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97 Novartis, Submission 138, pages [11]-[12].

98 Roche, Submission 92, p. 16.

99 RDIWG, Submission 51, p. 3.

100 Albireo Pharma, Submission 59, p. [2]; Stryker, Submission 28, p. 15; Alexion, Submission 30, p. 5; Association of Australian Medical Research Institutes (AAMRI), Submission 88, pages 8-9; Australasian Leukaemia and Lymphoma Group and Haematology Society of Australia and New Zealand (ALLG and HSANZ), Submission 112, p. [7]; Pfizer, Submission 137, p. [5]; Novartis Pharmaceuticals, Submission 138, pp, [11]-[12]. LEO Pharma, Submission 202, p. 6.

101 BMS, Submission 118, p. [22].

102 BMS, Submission 118, p. [22].

arrangement being appropriately shared between the sponsor and the Commonwealth.[103] The New South Wales Government (NSW Government) argued the arrangements need to include 'agreed timelines for evaluation post implementation with a focus on disinvestment, or renegotiation on price for therapies that do not meet expected value to patients and/or the health system. 'It also argued that they should only be put in place where the medicine meets 'baseline safety and efficacy.'[104] The Australasian Sleep Association recommended that these programs be designed in consultation with 'patients and clinical stakeholders (e.g. not for profit clinical or patient support organisations).'[105]

6.75 Dr Tuffaha recommended that the utilisation of Managed Access Programs be increased, and noted:

- It is vital to engage major stakeholders, including patient representatives, in the development and implementation of managed access schemes.
- Objective criteria and methods (e.g., Value of Information analysis) are required to systematically examine the need for, and the value of, these schemes.
- The conditions governing the implementation of the schemes should be clear, transparent and balanced to address the expectations of various stakeholders.
- The scheme should be continuously evaluated and improved to ensure that it serves its purpose.
- The consequences of any potential delisting decisions on stakeholders, should be carefully considered and managed, possibly through certain managed exit schemes (MEXITS).[106]

Overseas examples

103 AstraZeneca, Submission 42, p. 2; MSD, Submission 63, Appendix A, p. 4; Johnson & Johnson, Submission 134, p. 9; Pfizer, Submission 137, p. [5]; Novartis, Submission 138, pages [11]-[12]; Submission 202, p. 6.

104 New South Wales Government, (NSW Government), Submission 93, p. 19.

105 Australasian Sleep Association, Submission 16, p. 5.

106 Dr Tuffaha, Submission 72, p. [2].

6.76 One model for provisional access that drew particular support was that used by Germany.[107] Medicines Australia explained 'the German model' as follows:

> On market entry, a new medicine is reimbursed at its launch price for the first year, pending the completion of an early benefit assessment. In the second year of launch, depending on the outcome of the early benefit assessment, the reimbursement price is determined either by:
>
> - Compulsory rebate negotiations…for medicines with an additional benefit versus a competitor.
> - Reference price system where medicines with no additional benefit are reimbursed at the reference price[108]

Figure 6.1 The German Model

Source: Better Access Australia, Submission 160, p. 17.

6.77 Better Access provided a diagram illustrating the German model, *Figure 6.1*. It claimed that 'the German reimbursement process does not come at the cost of a rigorous value assessment,' it merely means that 'the negotiation of

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107 STA, Submission 7, p. 6; BioMarin, Submission 152, p. 5.

108 Medicines Australia, Submission 141, p. 54.

prices does not… stand in the way of access for patients.' It did however concede that the system 'has faced fiscal challenges with some companies overpricing and failure of the system to clawback excess from use beyond indication, or failure to achieve health outcomes in real world application versus clinical trials.' It argued that :

> Australia's experience in the utilisation of Risk Share Arrangements and improving data accessibility through electronic health records places us well to modify a system with suitable 'carrots and sticks' to get the balance of access, affordability and transparency right.[109]

6.78 BMS outlined France's Temporary Authorisation for Use program. It is available where a drug meets three criteria:

- The drug must be intended for a serious or rare indication
- There must be no other appropriate therapies available for this indication in France
- The drug must have presumed efficacy and safety in light of the available scientific data, and the treatment cannot be delayed for patients[110]

6.79 The drug price is 'set freely', but subject to an annual cap. Data is collected while the temporary authorisation is in force.[111]

6.80 Medicines Australia stated that in the UK 'the Cancer Drugs Fund (CDF) acts as a managed access pathway for new cancer medicines.'[112] MSD suggested that this has been more successful than its Australian equivalent, as it has been used for considerably more medicines.[113] BMS described the CDF as having two roles: funding managed access arrangements, and providing 'interim funding for all newly recommended cancer drugs.'[114]

6.81 The Committee's UK witness Mr Meindert Boysen, Deputy Chief Executive Officer and Director of the Centre for Health Technology Evaluation, NICE, explained that:

> We have a specific fund—it's called the Cancer Drugs Fund at the moment, but there are plans to expand that. That fund is used to allow companies to

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109 Better Access, Submission 160, p. 18.

110 BMS, Submission 118, p. [20].

111 BMS, Submission 118, p. [20].

112 Medicines Australia, Submission 141, p. 54.

113 MSD, Submission 63, Appendix A, p. 2.

114 BMS, Submission 118, p. [24].

> bring a drug to market at an earlier stage while data is collected. The one test we apply in the Cancer Drugs Fund is to make sure these products have a plausible potential for being cost effective. So we do our work. If the Cancer Drugs Fund wasn't available, our committees probably would not have supported the technology. But, because there is a Cancer Drugs Fund, they can recognise the uncertainty that is inherent in the evidence base, often for rare cancers in particular, and allow a period of what we call 'managed access'—two to three years of use in the NHS—combined with data collection.[115]

Proposed models

6.82 Mr Michael Smith, an industry consultant, put forward a detailed model for an interim access scheme, which is illustrated by *Figure 6.2.* Noteworthy features of his proposal include:

- It would be available for technologies (medicines or devices) considered to meet a high and unmet need by the PBAC or MSAC, eligible for the TGA's orphan drug designation or registered through its provisional or priority approval pathways, or for a 'special population' (such as paediatric or Indigenous) [116]
- The PBAC or MSAC would recommend that the technology is suitable for interim access, but the rest of the process would then be left up to the Sponsor and the Government[117]
- The duration of the interim access period would be agreed between the Sponsor and the Government, and could be extended by mutual agreement[118]
- The price would be divided into two components: the price the PBAC or MSAC considers reasonable on the basis of the available evidence, which would be paid immediately, and the difference between that price and the price requested by the Sponsor in its submission, which would be deferred[119]
- If the technology is not listed on the PBS or MBS at the end of the interim period, the Sponsor would not receive any of the deferred

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115 *Committee Hansard*, Canberra, 7 July 2021, p. 4.

116 Mr Smith, Submission 13, p. 9.

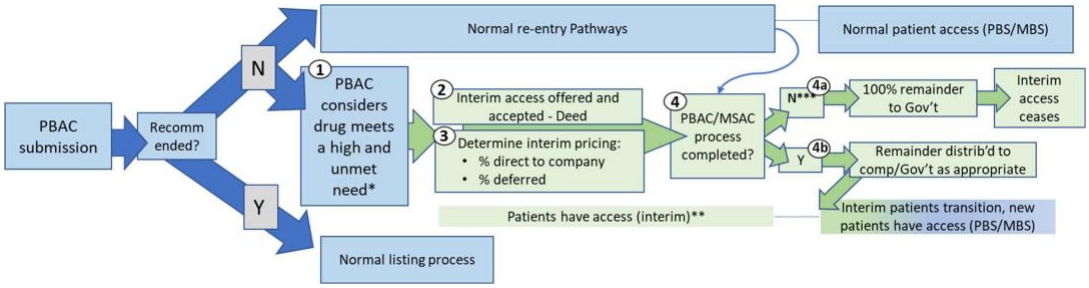
117 Mr Smith, Submission 13, p. 9.

118 Mr Smith, Submission 13, p. 10

119 Mr Smith, Submission 13, p. 10.

payment. Access would continue for existing patients but would not be available for new patients.[120]

Figure 6.2 Proposed Interim Access Scheme



Source: Mr Michael Smith, Submission 13, p. 8.

6.83 The MUCHE likewise recommended that the PBAC be allowed to recommend a MAP for any submission, even if it has not been requested by the Sponsor. It also included MAPs for LSDP listing in this recommendation – it was unclear on the evidence whether any form of managed access is currently available for the LSDP.[121]

6.84 The University of Melbourne suggested that standard 'post market surveillance mechanisms…are often unreliable.' On that basis it proposed a more restricted form of provisional access to medicines:

> The creation of nationally accredited centres for early, proactive assessment of a new innovation's efficacy, safety and health economic outcomes to provide the evidence to support broader dissemination (or not), and disinvestment from existing, ineffective health care practices.[122]

6.85 The ACTA recommended that the Government:

> Establish a rigorous pathway for treatments, services and technologies that are unproven in the real world to enter practice as quickly as possible through a conditional scheme. This scheme would require participation in either a trial conducted by Clinical Trial Networks (CTNs) and/or Clinical Quality Registries (CQRs) capable of generating important real-world data about the clinical effectiveness and value of the intervention in the real-world context.[123]

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120 Mr Smith, Submission 13, p. 11.

121 MUCHE, Submission 62, p. 12.

122 University of Melbourne, Submission 133, p. 3.

123 Australian Clinical Trials Alliance (ACTA), Submission 149, p. 5.

6.86 It pointed out that such a pathway would provide three benefits: earlier access for patients, better data for the Government, and more experience with the new therapies for doctors.[124]

6.87 The MOGA and PCPA submitted that the evidence base for cancer medicines often has more uncertainty than the current system is willing to accept, and consequently:

> For diseases with significant unmet clinical need and technologies that have proven to be efficacious and safe, making decisions based on surrogate endpoints may be appropriate, on the condition that the sponsor is obliged to undertake post-marketing evaluation.[125]

6.88 The Pharmacy Guild of Australia argued for the implementation of 'a standardised protocol-based post-market pharmacovigilance consultation in community pharmacy to enable earlier and reliable access to and ongoing clinical support for new and novel treatments.'[126]

The Pharmaceutical Benefits Advisory Committee's View

6.89 When asked by the Committee how many MAPs are in place currently, Prof Wilson replied:

> A very small number. I think in practice we have two which are still operational at the moment. The challenges in the managed access program, which I talk about in the paper, are that you have to have the right sort of question; you've got to be able to actually answer the uncertainty that you want to address; you've got to have the capacity to collect the data; and all parties need to be willing to submit the data. That requires resources to be able to do that, and there is expertise involved in doing it. It's sometimes easier. There's a registry that sometimes makes it a lot easier, from our perspective. But we would certainly be much more comfortable if it also had a specific legislative basis.[127]

6.90 In the paper, Prof Wilson refers the PBAC's submission that:

> …based on our observations in countries with similar health systems to ours, such a program should have a legislated framework which is binding on sponsors in relation to negotiated entry price, the period and requirements for

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124 ACTA, Submission 149, p. 5.

125 MOGA and PCPA, Submission 50, p. 2.

126 Pharmacy Guild of Australia, Submission 108, p. 6.

127 *Committee Hansard*, Canberra, 24 June 2021, p. 6.

> establishing a cost-effective price as determined by the PBAC, and agreement to continuation [sic] of supply for existing patients for free in the event that the cost-effective price is not agreed between the parties….Legislated frameworks will enable requirements for data collection and patient participation to be reasonable, relevant and mandated for the PBAC purposes. Such a program would require resourcing for clinical and patient participation, as well as the oversight of access protocols….
>
> The PBAC strongly believes an early access program should not be limited to a specific disease or condition although the eligibility criteria of a medicine for such a program should refer to high unmet need and disease severity/prognosis.[128]

Real World Evidence

6.91 While the central role that real world evidence (RWE) plays in provisional access schemes has just been discussed, submitters commented on various other issues relating to it. Takeda stated that RWE 'offers additional insights into the value of treatments' when used alongside clinical trial data and 'has the potential to reduce uncertainty and enable more informed decision-making' when generated through 'appropriate methodologies.' It noted that there will be particular opportunities to gather RWE when 'patients will be followed up for a long time for safety monitoring,' as will be the case for many gene and cell therapies, and emphasised the importance of disease registries for this purpose.[129]

6.92 Medicines Australia submitted that there are two 'major challenges' to more use of RWE in Australia's HTA system:

- Methodological challenges – where the lack of a specific framework and language for provision of real-world evidence leads to under-generation and under-acceptance.
- Procedural challenges – where the pre-reimbursement process is not conducive to the generation of real-world evidence for inclusion in HTA submissions[130]

6.93 It noted that there is a 'comparatively low ability in Australia to link datasets (compared with the rest of the world).' It noted that patients expect more use

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128 Department of Health, Submission 15.3, p. 4.

129 Takeda, Submission 66, p. 4.

130 Medicines Australia, Submission 141, p. 35.

of RWE in HTA than currently occurs, and highlighted the importance of including Patient Reported Outcome Measures (PROMs) in such evidence.

6.94 In the context of a discussion of the PBAC's approach to assessing medicines for rare paediatric diseases, the Luminesce Alliance explained that:

> …there is an opportunity to change the onus of approval to include different levels of evidence required for approval, such as the inclusion of real world evidence outside the gold standard of randomised controlled trials, such as observation in clinical practice and the use of clinical quality registries for a staged approval of drugs for paediatric indications.[131]

6.95 It called for better collection of data on compassionate access schemes - under which pharmaceutical companies provide their medicines to patients for free in certain circumstances, such as before reimbursement[132] - in order to generate more RWE, including through use of registries.[133] Both these points were echoed by the Children's Cancer Institute, one of the Alliance's members.[134]

6.96 The Australasian Leukaemia and Lymphoma Group and Haematology Society of Australia and New Zealand recommended a 'commitment to fully mobilise real-world data programs' for HTA purposes through better data collection, particularly 'increased focus and funding' for 'clinical quality registries.'[135]

6.97 Roche submitted that 'there has been a growing recognition of the value of RWE in making regulatory and reimbursement decisions, but the way in which RWE is being used in these processes is unclear.' It explained that the ability to capture RWE is growing thanks to technological advances, and that this growth offers increased opportunities for it to be used to mitigate uncertainty in assessment of therapies for small patient populations, as well as to assess repurposed medicines better. It noted the importance of improving 'data infrastructure' for the potential of RWE to be fulfilled.[136]

6.98 Sanofi recommended the development of 'clear and more inclusive processes, including the acceptability of several sources of scientific

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131 Luminesce Alliance, Submission 32, p. 21.

132 Medicines Australia, Submission 141, p. 12.

133 Luminesce Alliance, Submission 32, p. 21.

134 Children's Cancer Institute, Submission 84, p. [3].

135 ALLG and HSANZ, Submission 112, p. [7].

136 Roche, Submission 92, pages 19-20.

evidence, such as [RWE] to capture the value for patients and their families.'[137] Novartis similarly asked for 'a consistent approach, supported by Government, for the generation of [RWE] via registries to address evidence gaps in economic evaluations.'[138] AbbVie submitted that :

> …when unmet medical need is high and where randomisation is not ethical or feasible, other options such as [RWE] could be a viable option to provide pivotal evidence of the benefit of new medications. Fit for purpose HTA processes which allow more flexible evidentiary requirements which take into account the clinical and ethical complexity will need to be developed.[139]

6.99 BMS noted that HTA bodies in the UK (NICE) and Canada have 'signalled their intent to formally incorporate RWE into HTA guidance.' It stated that:

> PBAC and MSAC do currently consider RWE in evaluating medicines and RWE is referred to in the HTA Guidelines for both agencies. The guidelines do not, however, give sufficient details about how RWE will be considered. Further guidance would provide greater clarity to sponsors.[140]

6.100 It suggested the guidelines should address the same matters NICE is considering, namely types of RWE accepted, required quality and 'detailed methodological framework for best practice for consideration and use of data analytics.'[141]

6.101 UCB called for more use of RWE in assessment of 'drug device mechanisms' (that is, a form of combination therapy).[142] MCIA advocated for the use of both 'inclusion of large-scale observational studies as supporting evidence' and 'allowing the use of the TGA's SAS data as an observational instrument' in assessment of medicinal cannabis products.[143] Alexion argued that 'there is a need to develop detailed and transparent explanatory notes' for the LSDP's eligibility criteria to 'allow the company to make a more accurate assessment of [RWE] needs.'[144]

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137 Sanofi, Submission 99, p. 3.

138 Novartis, Submission 138, p. [11].

139 AbbVie, Submission 180, p. [4].

140 BMS, Submission 118, p. [21].

141 BMS, Submission 118, p. [21].

142 UCB, Submission 74, p. 3.

143 MCIA, Submission 75, p. 3.

144 Alexion, Submission 30, p. 9.

6.102 The MUCHE, while supportive of a role for RWE in the context of provisional access, commented that 'it should be noted that [randomised controlled trials] are considered to be the gold standard, and real world, observational data is often subject to confounding.'[145]

The valuation process

A broader concept of value

6.103 The valuation process was of particular interest to industry. Better Access submitted that value should be ascribed to factors such as economic productivity, workforce participation, 'a sense of self' and 'a sense of confidence and opportunity.'[146] It suggested it was telling that the Government has bypassed the HTA process entirely in its funding decisions regarding COVID-19 vaccines, particularly in regard to how that process values other vaccines.[147]

6.104 The AHHA noted:

> To demonstrate value, health technology assessments must also include consideration of equity. Are the right patients receiving the right treatment? Value is only achieved across the whole health system if everyone that needs it can access it.[148]

6.105 Viiv Healthcare Australia (Viiv) similarly argued that the current approach:

> …doesn't recognise that some individual consumers may have better or worse outcomes from medicines that are considered clinically equivalent on average across the whole target. For example, a patient may have side effects from the old medicine but not from the new one. So, patient choice is also important at a personalised level.[149]

6.106 STA provided one of the most concise comments on this issue, proposing the introduction of 'new objectives for subsidy processes aligned to patient need.'[150]

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145 MUCHE, Submission 62, p. 11.

146 Better Access, Submission 160, p. 22.

147 Better Access, Submission 160, p. 21.

148 AHHA, Submission 68, p. 2.

149 Viiv Healthcare Australia (Viiv), Submission 80, p. 7.

150 STA, Submission 7, p. 6.

6.107 Medicines Australia argued that 'the current PBAC evaluation of medicines, inadequately considers the evaluation of social and economic impacts of a particular medicine or intervention.' It claimed that 'there are validated methodologies for assessing many of the key determinants of success, used often and with useful context in other areas of health and social research,' citing studies on the valuation of treatments for osteoarthritis and haemophilia.[151] It suggested that the problems with the current system are particularly acute for vaccines, 'preventative medicine approaches' and other therapies with particularly long-term benefits.

6.108 In its submission Eli Lilly Australia (Eli Lilly) asserted there is a problem with the valuation of 'innovative medicines,' and made two recommendations to address this:

- Development of a more comprehensive assessment of value when it comes to innovative medicines that takes into account the second-round effects of keeping people well and productive members of society.
- Inclusion of a data-based matrix that considers and measures the long-term benefits of listing innovative medicines on the PBS. Data should include not just dollar savings to the health system more broadly, but also the financial and associated socioeconomic benefits of improved workforce productivity and reducing disability.[152]

6.109 Roche likewise argued that, particularly in face of 'new precision medicine technologies,' 'broader dimensions of value, including societal value, need to be included, and encouraged, in assessments for reimbursement.'[153] It went on to explain:

> While both the MSAC and PBAC Guidelines state that they do consider the value of societal outcomes, they do not do so in a quantitative manner - i.e societal outcomes are not included in the cost-effectiveness calculation. It would be valuable for the Government to provide transparency and clarity

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151 Medicines Australia, Submission 141, pages 33-34, 39, citing D Schofield et al., 'Measuring labour productivity and the benefits of interventions for osteoarthritis ', Project report for Medicines Australia, Sydney, September 2016, www.medicinesaustralia.com.au/wp-content/uploads/2020/11/20160905-rpt-FINAL-Schofield-OA_productivity-final-report.pdf , viewed 18 October 2021; and L Brown et al., 'The societal burden of haemophilia A. III - the potential impact of emicizumab on costs of haemophilia A in Australia', *Haemophilia*, August 2020.

152 Eli Lilly, Submission 140, p. [4].

153 Roche, Submission 92, p. 18.

> around how opportunities for more formal inclusion of societal benefits in cost-effectiveness calculations can be undertaken.[154]

6.110 Johnson & Johnson was also critical of the view of value taken by both the PBAC and MSAC, which it described as 'narrow.'[155] It recommended 'greater recognition' in the Guidelines 'of societal value to reflect the overall benefit to the Australian Government and the Australian people of new and innovative therapies,' as well as 'consideration of leveraging innovative international assessment model…such as a Value Appraisal System or Multi-Criteria Decision Analysis tool designed specifically for the Australian environment.'[156]

6.111 UCB raised the valuation of innovation in a more specific context, the valuation of 'drug device mechanisms,' stating it wished to see 'a broadening of the criteria (e.g. acceptance of real-world data) for the cost effectiveness assessment of drug device mechanisms, to appropriately recognise the value of the delivery device to the holistic treatment.'[157] MSD likewise argued the current system does not 'appropriately value' diagnostic devices.[158] The ACvA argued that:

> Widespread adoption of digital health technologies is inhibited by the lack of a coordinated framework for assessing the value of digital technologies and incorporating such value assessments into reimbursement mechanisms.[159]

6.112 Some submitters focused particularly on the question of valuing products for rare diseases. CSL Behring, for example, called for 'allowing a broader consideration of value' for blood products and therapies for rare diseases.[160]

6.113 Alexion Pharmaceuticals made the following point:

> There is a need when considering the value of medicines for rare diseases to consider matters beyond cost-effectiveness such as these broader societal impacts i.e. impact on carers, broader community care and economic costs. A fit for purpose process to assess rare disease treatment should also consider

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154 Roche, Submission 92, p. 19.

155 Johnson & Johnson, Submission 134, p. 10.

156 Johnson & Johnson, Submission 134, p. 13.

157 UCB, Submission 74, p. 3.

158 MSD, Submission 63, Appendix A, p. 2.

159 ACvA, Submission 76, p. 11.

160 CSL Behring, Submission 145, p. 15.

> the use of multi-criteria decision-making (MCDM) to incorporate all relevant elements of the rare disease treatment value into a funding decision. Any future modification to the review system for rare disease treatment should limit the use of cost-effective ratios to allow broader assessment of value for pragmatic decision-making or allow for more flexibility in dealing with uncertainty.[161]

6.114 In contrast to the focus of many submitters on rare diseases, the Australian and New Zealand Headache Society commented that the PBAC is rejecting migraine medications on cost effectiveness grounds when 'the same pharmaceutical companies succeed in approval for similar medications at much greater individual costs for rare diseases.' It recommended that:

> …for common conditions such as migraine, the broad economic benefits of treatments under consideration such as productivity, avoidance of absenteeism and ability to engage in the workforce or in productive but unpaid domestic and community activities be given greater emphasis by PBAC.[162]

6.115 The sentiment was echoed by LEO Pharma, which stated 'not all diseases are viewed equally by the PBS….this has been particularly obvious in the space of dermatology…where the societal and economic contributions a treated patients can add are often overlooked.'[163] It argued that better horizon-scanning would make the PBAC 'better equipped to assess the value of new and innovative medicines for Australians.'[164]

6.116 MSD did not raise the issue of rare versus common diseases, but submitted that:

> The assessment of treatment value must be kept separate and apart from considerations of affordability. Accepting budget impact as a component of treatment value wrongly suggests that curtailing pharmaceutical spending will solve system affordability issues and ignores the existence of numerous inefficiencies throughout health systems.[165]

Valuing future benefits and vaccines

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161 Alexion, Submission 30, p. 9.

162 Australian and New Zealand Headache Society, Submission 115, p. [2].

163 LEO Pharma, Submission 202, p. 2.

164 LEO Pharma, Submission 202, p. 5.

165 MSD, Submission 63, Appendix A, p. 4.

6.117 One particular criticism that submitters had of the current valuation system was its approach to valuing longer-term benefits. Mr Ian Noble, Director, Value, Access and Policy, Amgen, explained the problem as follows:

> In an economic evaluation you model things out into the future and you have a discount rate which you apply, because values in 10 years' time aren't the same as values now; it's like interest rate. In Australia, we have a five per cent discount rate. At five per cent every year, by 10 years you've discounted the benefits quite a lot. For a medicine like a gene therapy or a vaccine, where all the cost is today but the benefit is over the lifetime for those children, you're discounting all their benefits away, then you're looking at the costs undiscounted. I know that in the UK and Canada they're looking at three per cent and 1½ per cent discount rates. Why we've got five per cent in these modern times I do not know at all. That's a practical thing that has a massive impact on those sorts of technologies.[166]

6.118 Novartis similarly noted that:

> The current HTA evaluation process is limited in its ability to allocate value to single-use products with the potential for long-term patient benefit given the constraints of evaluating costs and benefits within the 'health care' budget only and heavily discounting future benefits to patients.[167]

6.119 Medicines Australia said of the current approach:

> The resultant impact on pricing is that it may not accurately reflect a treatment's value. Key examples include vaccines and other preventative medicines approaches, where the outcome may be distant to the intervention. There are simple means to address these issues methodologically, even adjusting discount rates in economic modelling; the system ought to be sufficiently flexible to ensure accurate and appropriate valuation.
>
> The issue of appropriate valuation is particularly acute where the value of health benefits and healthcare savings accrue over many years. Future benefits and costs are discounted to reflect society's time preference for benefits now over benefits in the future or the cost of capital. Australia appears to apply one of the highest discount rates in the world to the assessment of future healthcare benefits and costs.[168]

6.120 It illustrated the effect the difference in discount rates makes with the example of a treatment 'preventing a death in a child with a life-expectancy

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166 *Committee Hansard*, Sydney, 12 March 2021, p. 21.

167 Novartis, Submission 138, p. [12].

168 Medicines Australia, Submission 141, p. 33.

of 80 years:' Australia would value this at 20.5 life years, compared to the UK and New Zealand at 27.7 life years and Canada at 47 life years. It concluded:

> [a]t a time when healthcare systems worldwide are calling for a rebalance of effort towards prevention, Australia's discount rate risks pulling resource allocation in precisely the opposite direction.[169]

6.121 MSD submitted that a study has shown the PBAC to underestimate survival benefits and that 'the underestimation of survival benefits and the relatively short time horizon preferred by the PBAC for economic analyses suggest that that the value of medicines with longer-term benefits may be underestimated.'[170] It argued that there are four problems with how vaccines in particular are valued: the high discount rate for future benefits; the narrow 'healthcare system perspective' used in assessment of costs and benefits (discussed in the previous section); a low tolerance for uncertainty; and 'the lower cost-effectiveness threshold (willingness to pay per unit of health gained) applied for preventive interventions like vaccines as compared to therapeutic medicines.'[171] It suggested that none of these issues is unsolvable, and proposed 'establishing pathways for vaccines to make them accessible for public health issues with high unmet need.'[172]

6.122 Pfizer echoed these concerns, and similarly identified the high discount rate, the 'narrow assessment scope' and 'the lower cost-effectiveness threshold applied for preventative interventions like vaccines as compared to therapeutic medicines.'[173] It recommended applying a lower discount rate, using a broader perspective on costs and benefits and removing the cost-effectiveness disadvantage.[174] Ms Anne Harris, Country Manager, Pfizer, told the Committee:

> …for vaccines, in particular, we do have some challenges currently with the process for vaccine evaluation. We know vaccines have a huge public health benefit, but it does take time for those benefits to come through. The current

169 Medicines Australia, Submission 141, p. 35.

170 MSD, Submission 63, Appendix A, p. 2, citing K Phan K. et al.,'Comparison of long-term overall survival with extrapolated overall survival for pembrolizumab assessed by Australian reimbursement authorities', *ISPOR Asia*, 14-16 Sept 2020.

171 MSD, Submission 63, Appendix C, pages [1]–[2].

172 MSD, Submission 63, Appendix C, p. [1].

173 Pfizer, Submission 137, p. [5].

174 Pfizer, Submission 137, p. [6].

> system devalues—the benefit upfront is valued more than the benefit later on. As you say, there are these broader benefits. I would say that one example would be meningococcal B vaccines. We have struggled to get that through evaluation, not just with Pfizer, but across the industry. There've been several attempts, but it has not been able to be demonstrated, to get a positive recommendation. If there were an approach which truly valued preventative treatments differently to how they are valued against medicines, we would be able to have further access.[175]

6.123 Ms Harris noted that the assessment process is longer and more expensive for vaccines than for therapeutic medicines, as they must be assessed by the ATAGI before going to the PBAC, which requires a separate submission and $180,000 fee, and must go through a tender process after approval by the PBAC.[176]

6.124 Ms Vanessa Xavier, Head, Market Access, Australia and New Zealand, Sanofi told the Committee:

> …with a vaccine, it is not possible to conduct a clinical trial that will capture every potential benefit of that vaccine. If we look at influenza specifically, there's something called seasonal variation….Our recent experience with flu vaccination was that we submitted 16 years' worth of seasonal data to show that, on average, the vaccine was highly cost effective. But, during the evaluation process, the focus was on, 'Okay, what is that one year in 14 where you're not matched and your efficacy is not as high as the other years?' Our response to that is that value has to be determined by the overall benefit that the product is going to deliver. So that's the first issue. You can never conduct a 16-year trial across all different seasonal variations to calculate efficacy.
>
> The second issue is then the broader benefit….Under the current evaluation process it is actually not possible as part of your base case to include broader societal benefits. You must limit your economic evaluation to healthcare costs only….So, if you have to curtail the number of benefits that you're allowed to include in your evaluation, clearly what that means is that the price or the value that is attributed to your vaccine is significantly lower than other jurisdictions where you may be able to consider those broader societal benefits.
>
> What that means specifically for us in Australia is also—I'm sure you've heard through this inquiry about ICER [incremental cost effectiveness ratio] thresholds. It's the willingness to pay for different types of interventions. We

175 *Committee Hansard*, Sydney, 12 March 2021, p. 9.

176 *Committee Hansard*, Sydney, 12 March 2021, p. 11.

> don't have specific thresholds, but you can see in the decision-making that there are different ICERs that are recommended for different types of therapeutics, which relate specifically to unmet need. So you'll see that oncology life-saving drugs generally accept listings at higher ICER thresholds. Vaccines have the lowest ICER threshold of all interventions. So not only are you not allowed to include the full scope of the benefits; the willingness to pay is significantly lower. This is why, unfortunately for us, we have been through quite a few processes for vaccines and we've actually not been able to bring our vaccines to the market in Australia.[177]

6.125 Sanofi submitted that 'no review of the National Immunisation Program has occurred since the Program was created in 1997.' It suggested that 'a review into the evaluation processes for vaccines should be conducted.'[178] The University of Melbourne similarly commented that:

> We also note the opportunity, driven by COVID-19, to review the PBAC assessment process for publicly funded vaccines. The current assessment, which is designed for drug assessment, should consider the societal, health and economic benefits of vaccines that offer future reductions in mortality/mobility.[179]

The use of comparators

6.126 Another aspect of the PBAC's valuation method that submitters supported reforming was the use of comparators. These are defined in the Department's HTA glossary as simply 'the existing health technology (or other current clinical management) that most health care practitioners will replace in practice should the proposed health technology be implemented as proposed.'[180] The Department told the Committee that 'the comparator really is meant to capture a new proposal in comparison with the existing state of play.'[181]

6.127 Viiv explained that:

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177 *Committee Hansard*, Sydney, 7 May 2021, p. 16.

178 Sanofi, Submission 99.1, p. [1].

179 University of Melbourne, Submission 133, p. 4.

180 Department of Health, 'Glossary: key terms for preparing submissions to a health technology assessment (HTA) advisory committee for funding of a medicine, medical service or prosthesis', Canberra, February 2013, https://www.pbs.gov.au/industry/useful-resources/glossary/Glossary-of-Terms_Final-15Apr-13.pdf, viewed 19 October 2021.

181 Ms Adriana Platona, First Assistant Secretary, Technology Assessment and Access, Department of Health, *Committee Hansard*, Canberra, 18 June 2021, p. 24.

> The *National Health Act 1953* (Cth) requires the PBAC to assess cost-effectiveness of a medicine relative to an alternate therapy or therapies. This is referred to as the comparator. This is to ensure the listing of new medicines represent a value for money investment in the PBS. New medicines can face challenges in demonstrating cost-effectiveness when compared to older medicines, whose prices have been significantly eroded over time through statutory pricing cuts. Even in cases where the new medicine is safer or more effective than the older medicine, it can be difficult to justify an appropriate price where the older medicine is very inexpensive. As more medicine patents continue to expire and Government generic savings are achieved, the impact of this 'comparator price erosion' will increase.[182]

6.128 Viiv noted that this is closely linked to the policy of reference pricing, which it described as:

> …a policy that applies when drugs considered to be of similar safety and efficacy for pricing purposes are linked and recommended by the PBAC as cost minimised. The lowest priced brand or drug (i.e. the lowest cost comparator) sets a benchmark price for either the other brands of that drug or the other drugs within the same sub-group of therapeutically related drugs.[183]

6.129 It went on to say:

> In many cases, the lowest cost comparator has limited use. This will often result in there being a clinical comparator defined by the market leader with high quality evidence supporting the relative efficacy and safety being different to the price comparator with limited evidence of relative benefit.[184]

It suggested this problem could be solved by the institution of a system similar to that used by the UK's NICE, under which 'a scoping document is developed with the input of clinicians and patient groups to determine patient population, place in clinical practice and most appropriate comparator for the therapy.'[185]

6.130 In his appearance before the Committee Mr Meindert Boysen, Deputy Chief Executive Officer and Director of the Centre for Health Technology Evaluation, NICE, did not comment directly on NICE's approach to

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182 Viiv, Submission 80, p. 6.

183 Viiv, Submission 80, p. 6.

184 Viiv, Submission 80, p. 7.

185 Viiv, Submission 80, p. 7.

comparators, but when asked about the involvement of patients in NICE's processes said:

> It starts when we scope a technology evaluation, so we set the question for the work. That's where patients are involved. When we seek submissions not only are we seeking submissions from the company, but we get them from patients, from patient organisations and from clinicians.[186]

6.131 When asked about NICE's approach to health economics in general Mr Boysen replied:

> My experience is that there are always two versions of what might be considered as the truth, although it's really difficult to establish what the true value of a technology is, because all the research is short term. It's all about modelling. Modelling—and we know this from COVID, of course—is all to do with managing uncertainty. I don't think the complexities of the health economics is the point. It's: How do you manage uncertainty? How do you deal with risk? That, I think, is the big question for HTA agencies: Do you deal with risk by saying no? Do you deal with it by having an arrangement in which you manage risk together and you collect the evidence? Health economics ought to be about uncertainty and risk and not about just one number. That's where I am at.[187]

6.132 Viiv's concerns were shared by other submitters, including MSD, UCB, AbbVie and LEO Pharma.[188] Gilead Sciences gave an example of a hepatitis B treatment that the PBAC rejected by comparing it to the 'lowest cost comparator' even though the PBAC agreed another drug was the 'appropriate clinical comparator.' Gilead submitted that

> Australians are missing out on new medicines as a result of a policy that seeks to anchor the cost of new drugs to the lowest cost drug (including generics) and not the price of the medicine it will replace.
>
> Changes to this process should urgently be considered to ensure the independent PBAC is selecting comparators that reflect current clinical practice, in preference to defaulting to a comparator with the lowest cost. This

186 *Committee Hansard*, Canberra, 7 July 2021, p. 3.

187 *Committee Hansard*, Canberra, 7 July 2021, p. 9.

188 MSD, Submission 63, p. 3; UCB, Submission 74, p. 4; AbbVie, Submission 180, p. [4]; LEO Pharma, Submission 202, p. 4.

> may include, if necessary, amending the legislative powers under which the PBAC operates.[189]

6.133 Pfizer drew attention to the problems of 'comparator price erosion' and the 'application of "lowest cost comparator."'[190] Its recommended response was:

> Resolution of the comparator selection issue as agreed in the current Strategic Agreement between Medicines Australia and Government without further delay. This could include establishing a clinical and pricing comparator before lodgement of a PBAC submission and the application of shadow pricing to allow F1-like price for F2 medicines that have undergone significant price reduction.[191]

6.134 The second part of the recommendation is targeted at the comparator price erosion problem, and refers to the mechanics of how that erosion occurs through the PBS formularies. As Pfizer explained earlier in its submission: 'in general, on-patent medicines sit in the F1 formulary and off-patent medicines sit in the F2 formulary, and the prices of medicines in the F2 formulary reflect competition in the market.'[192]

International reference pricing

6.135 Another dimension of the valuation issue that several submitters were keen to emphasise is the comparison between Australia's pricing and other countries.[193] They argued that this is important because of 'international reference pricing.' As Pfizer explained:

> The relatively low prices…can also impact on other markets, due to international reference pricing of PBS list prices. Australian PBS prices are referenced by numerous other countries. Ultimately, this can result in medicines not being PBS listed in Australia.[194]

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189 Gilead Sciences, Submission 101, p. 3.

190 Pfizer, Submission 137, p. [10].

191 Pfizer, Submission 137, p. [11].

192 Pfizer, Submission 137, p. [10].

193 Mr Chris Stemple, Vice President and General Manager, Australia and New Zealand, AbbVie, *Committee Hansard*, Sydney, 12 March 2021, p. 13; Shawview Consulting, Submission 181, p. 7.

194 Pfizer, Submission 137, p. [11]

6.136 Mr Benjamin Basil, President and General Manager, Australia, New Zealand and North Asia-Pacific, Eli Lilly responded to the suggestion that more pricing transparency would benefit Australia patients by saying:

> One of the obvious threats is around the international reference pricing and visibility. These special pricing arrangements that we have with 80 per cent of our overall revenue doesn't make that visible. If that becomes visible, then we're going to face more and more situations where we have an innovation and, in order to dispense it and make it available to Australians or in any particular market, it would be a loss for the company.[195]

6.137 Johnson & Johnson submitted that:

> In the context of an increasing global focus on International Reference Pricing, including the potential for its adoption in major pharmaceutical markets like the United States, it is becoming increasingly difficult for us to justify to our global organisation why a lower value for the novel therapy should be accepted. Consequently, there is a real risk that Australian patients will miss out on receiving treatment with new and novel therapies as product sponsors will not be able to accept the terms for reimbursement in Australia.[196]

6.138 Johnson & Johnson's particular concern about the situation in the United States (US) was shared by other submitters such as STA.[197] This situation was explained by Medicines Australia as follows:

> US President Donald Trump has signed the 'Most Favored Nation' executive order (EO), which aims to introduce international reference pricing (IRP) into the Medicare pharmaceutical drug programmes (Part B and Part D) to lower drug prices in the United States. The new order calls on the Secretary for Health and Human Services (HHS) to test a new payment model for which Medicare would pay no more than the most-favoured-nation price for "certain high-cost" physician-administered Part B drugs, as well as Part D pharmacy drugs with "insufficient competition". According to the federal administration, the most-favoured-nation price would be calculated as the lowest price for a particular prescription drug or biologic that is sold in another Organisation for Economic Cooperation and Development (OECD) country with a "comparable" per capita GDP to the US.[198]

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195 *Committee Hansard*, Sydney, 7 May 2021, p. 28.

196 Johnson & Johnson, Submission 134, p. 10.

197 STA, Submission 7, p. 5.

198 Medicines Australia, Submission 141, p. 15. The Executive Order is Exec. Order No. 13947, 85 Fed. Reg. 59171 (24 July 2020).

6.139 Medicines Australia submitted that Australia's prices for the relevant medicines are up to 81 per cent lower than those in the US, equal lowest in the OECD with France and slightly than Norway at 80 per cent.[199] Amgen submitted that 'potential US brand market sales losses based on Australian prices could be nearly 50 times the total Australian market,' namely $338 billion in losses compared to the $7 billion Australian market.[200]

6.140 Ms Leah Goodman, Managing Director, Australia and New Zealand, Merck Healthcare, similarly told the Committee:

> The problem is that Australia is actually a price reference country. I can give you an example from 2018 when I had to decide—not me but my global company—not to bring a product into Australia for a very rare type of head and neck cancer, because the value given by the Australian system was so low that it would have impacted China.
>
> It's going to get worse, because the US is now talking about, through the executive order and the Pelosi bill, price referencing Australia. As soon as that happens - …the US revenue pays for the majority of the global research and development costs. You can imagine me…saying: 'Give us priority. Make us make a wave 1 country at an intensely low value that puts at threat both China and the US.' It's not going to happen….the end result will be that Australia is left behind in access to innovative medicine.[201]

6.141 The 'Pelosi bill' is a reference to the Elijah E. Cummings Lower Drug Costs Now Act, HR Res 3 117th Congress (2021). That Bill refers to an 'average international market price' calculated by reference to prices in Australia, the UK, Canada, France, Germany and Japan.[202]

6.142 Medicines Australia stressed the important of 'confidentiality of pricing to ensure that other markets do not either reference or apply Australian-derived pricing to their markets' – a reference to the Special Pricing Arrangements discussed above by Mr Basil– and recommended that 'there should be a regular forum established to consider global actions and policies that may impact on both Australia's health outcomes and competitive position.'[203]

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199 Medicines Australia, Submission 141, p. 15.

200 Amgen, Submission 82.4, p. [4].

201 *Committee Hansard*, Sydney, 12 March 2021, p. 7.

202 s 1191(3)(B).

203 Medicines Australia, Submission 141, p. 15.

6.143 Mr Michael Smith was more hesitant than other submitters to predict what the impact of American developments on Australia will be. He submitted that:

> One view is that this represents a challenge to access in Australia because, if medicines' prices in Australia are referenced by the USA (i.e. prices in Australia are used as reference points to adjust prices in the USA), it will delay or prevent availability in Australia. This is because Australia is a small market globally, and in comparison to the USA.
>
> While this potential development is beyond the scope of this Inquiry, it will be seen by some submitters as impactful. Because reference pricing by the USA would be a new development, its impact remains to be seen.[204]

6.144 The Trump Administration Executive Order faced multiple court challenges, which suspended its implementation. As of September 2021 the current US Administration had indicated that it intended to revoke the Order, but was still pursing options to lower medicine prices in the US. The HHS stated that:

> On July 9, 2021, President Biden signed an Executive Order on Promoting Competition in the American Economy that, in part, directs the Secretary of HHS to take steps to lower the prices of and improve access to prescription drugs and biologicals. HHS is exploring opportunities to promote value-based care for our beneficiaries; to address the high cost of Medicare Part B drugs, manufacturers' pricing, and the resulting growth in Medicare Part B drug spending; and to modernize the Medicare program to improve the quality and cost of care for beneficiaries. We will continue to carefully consider the comments we received on the November 2020 interim final rule as we explore all options to incorporate value into payments for Medicare Part B drugs and improve beneficiaries' access to evidence-based care.[205]

6.145 The 'November 2020 interim rule' is a reference to the previous Executive Order. Meanwhile *H.R. 3, Elijah E. Cummings Lower Drug Costs Now Act* passed the US House of Representatives on 12 December 2019, but as of October 2021 still had not passed the US Senate.[206] It is therefore unclear

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204 Mr Smith, Submission 13, p. 5.

205 US Department of Health and Human Services, 'Most Favored Nation (MFN) Model', Washington D.C., August 2021, https://public-inspection.federalregister.gov/2021-16886.pdf, viewed 19 October 2021.

206 US Congress, 'H.R.3 - Elijah E. Cummings Lower Drug Costs Now Act', Washington D.C., undated, https://www.congress.gov/bill/116th-congress/house-bill/3/all-actions?overview=closed#tabs, viewed 19 October 2021.

what role, if any, international reference pricing will play in US pricing in the future.

The Pharmaceutical Benefits Advisory Committee's response

6.146 The PBAC noted many of the issues just discussed, which it suggested were too technical to be considered in depth by this inquiry. Instead it proposed they be considered in a review of the PBAC Submissions Guidelines:

> The PBAC notes that a number of submissions to the Inquiry raise issues about the extent to which the committee takes into account non-health care benefits and costs in assessing cost-effectiveness; the discount rates applied in economic analyses especially in relation to vaccines and preventive medicines; the choice of comparator; and the use of real-world data. These, and other methodological issues would be better considered as part of a broader PBAC Submissions Guidelines review and the PBAC would be happy to do so. As with previous reviews, there would be wide consultation and an industry liaison working group and any changes to Government policy parameters would be taken into account.[207]

Pricing

The Pharmaceutical Benefits Advisory Committee and pricing

6.147 After a medicine is given a positive recommendation by the PBAC, the sponsor must then negotiate a price and other matters with the Department before it can be listed on the PBS.[208] However concerns were raised that the PBAC itself is effectively becoming involved in price negotiation, and that this helps explain why multiple submissions are often required for a medicine to receive a positive recommendation. Kyowa Kirin Australia, speaking as a new entrant to the Australian market, submitted that while it was generally impressed with the PBAC:

> Where we have some initial concerns is in the potential use of submission and evaluation processes for primarily pricing and commercial negotiation

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207 Department of Health, Submission 15.3, pages 7-8.

208 Department of Health, *8 Procedures for a Positive Recommendation to List*, Department of Health, undated, www.pbs.gov.au/info/industry/listing/procedure-guidance/8-procedures-positive-recommendation-list/8-procedures-for-a-positive-recommendation-to-list, viewed 26 September 2021.

> purposes. However, we will reserve further comment and suggestions about this issue until our current engagement with the system is complete.[209]

6.148 MSD claimed that this tendency is real and has already had negative effects on medicine access:

> PBAC decisions are increasingly creeping beyond the scope of cost-effectiveness assessment to include budgetary cost containment considerations. These types of conservative HTA decisions, conflated with budgetary concerns, have resulted in Australian patients missing out on medicines.[210]

6.149 Mr Ian Noble of Amgen told the Committee about an Amgen drug for familial hypercholesterolaemia, which was approved for one patient population (homozygous patients) almost five years before it was approved for another (patients needing secondary protection). He explained the gap in the following terms:

> There's a budgetary consideration in this process. The homozygous population is an extremely rare patient group—we're looking at 20 or 30 patients—and it's not a big cost. Whereas when you talk about patients needing secondary prevention—so they're already on lipid-lowering therapy; they've already had heart attacks—that is still a relatively large population and potentially a larger financial impact.[211]

6.150 He went on to say:

> This is the problem I'm trying to illustrate. It's a very inefficient process to have what is effectively a negotiation via a committee that meets three times a year. It's just not geared up for negotiation. We need to have…one submission do a good job, say yes to that and then have a negotiation. But that negotiation post-PBAC doesn't really happen. It's: 'You've got a rejection. You've got to go back to the start again.'[212]

6.151 BioMarin did not go into the same degree of detail, but recommended:

> development of a policy framework to deal with pricing negotiations and funding arrangements, to enable evaluation of new drugs and emerging medical technologies to be delinked from the funding decisions, and the selection of the appropriate evaluation pathway for human therapeutics to be

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209 Kyowa Kirin Australia, Submission 87, p. [3].

210 MSD, Submission 63, p. 3.

211 *Committee Hansard*, Sydney, 12 March 2021, p. 17.

212 *Committee Hansard*, Sydney, 12 March 2021, p. 18.

made in accordance with evaluation expertise rather than the funding mechanism.[213]

6.152 LEO Pharma did not directly raise the issue of whether pricing considerations are contributing to rejections, but commented that:

> The current approach of rejecting a submission due to the need for more information rather than having ongoing dialogue between the payers, sponsors and clinicians meant the reimbursement process is taking longer, with a higher overall cost to sponsors.[214]

Price negotiations

6.153 Turning to medicines that do receive a positive recommendation from the PBAC, several submissions expressed unhappiness with the price negotiation process and how long it often takes.[215] The MOGA and PCPA submitted that:

> …over the last decade some important new oncology drugs and therapies in areas of high unmet clinical need in Australia have received positive recommendations from the PBAC, however the decisions have been followed by delays due to Government fiscal considerations, or prolonged and often unsuccessful negotiations between the sponsor and Government regarding price. These delays to PBS listing have negatively impacted on timely access to key oncology treatments in Australia.[216]

6.154 The MOGA and PCPA recommended that 'the delay between PBAC approval and PBS listing be reviewed. Efforts should be made to reduce the time from PBAC recommendation to PBS listing as this is likely to have a positive impact on patient care.' However when asked how negotiations could be shortened its Deputy Chair, Dr Deme Karikios replied:

> It's very practically challenging. My understanding—I'm not in the room with drug companies when they figure out the prices—is that someone from global says what they need to go in at, and it's always above; they're not going to shoot low. I don't know. If we could somehow put to them what's appropriate. It probably does get put to them; I don't know. I did some research on this during my PhD. The biggest factor that leads to rejection is price. But if a

213 BioMarin, Submission 152, p. 3.

214 LEO Pharma, Submission 202, p. 6.

215 MSD, Submission 63, p. 2; Gilead Sciences, Submission 101, p. 2; Eli Lilly, Submission 140, p. [2]; Better Access, Submission 160, p. 6; LEO Pharma, Submission 202, p. 2.

216 MOGA and PCPA, Submission 50, p. 1.

> global company is saying to the Australian subsidiary, 'You've got to go in at that price first; we're not going to accept anything lower,' then what can we do? I don't know the answer to that.[217]

6.155 Medicines Australia offered a potential solution that it claimed would both improve the negotiation process and address the problem of the PBAC's potential involvement with negotiation discussed above. It submitted that:

> Since the abolition of the Pharmaceutical Benefits Pricing Authority (PBPA) in 2014, the PBAC has arguably taken a more active role in considering not just the cost-effectiveness of medicines but also budgetary impacts, deeds of agreements, net prices, and risk sharing…
>
> In Medicines Australia's view...questions of funding, pricing, business viability and investment should be the remit of a separate body.
>
> The former PBPA had as its objective "to secure a reliable supply of pharmaceutical products at the most reasonable cost to Australian taxpayers and consumers, consistent with maintaining a sustainable pharmaceutical industry in Australia". It provided some semi-independent oversight of the Department of Health's administration of the post-PBAC price negotiation process, where many medicines struggle to achieve PBS listing.
>
> Medicines Australia believes the system would benefit from the introduction of a new oversight committee, which could add value to governmental processes, improve decision making and accountability, and assist in achieving the appropriate balance between value-for-money reimbursement and ensuring sustainable supply.[218]

Value-based payment models

6.156 There was considerable interest in the potential of 'value-based payment models,' particularly for the supply of antimicrobials. The AHHA commented that:

> From a funding perspective, while fee-for-service or activity-based funding models have provided greater transparency in terms of variation of costs in the public hospital system, many commentators are seeing a 'value' based approach as better suited to drive overall improvements in patient outcomes as well as cost efficiency. The discussion around value-based health care to

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217 *Committee Hansard*, Sydney, 11 March 2021, p. 66.

218 Medicines Australia, Submission 141, p. 40.

> date has largely been around organisational transformation and system design, with limited consideration of the impact of new technologies. Ultimately, new technologies are only useful if they provide better patient outcomes at an efficient cost, and this may not be easy to demonstrate in the short-term.[219]

6.157 AHHA emphasised that data on patient outcomes and experiences will have to be collected and utilised effectively and consistently for any move towards value-based payment to be a success.[220] This point was echoed by Takeda, which described it as essential for the use of performance-based contracts. It stated that 'performance-based contracts (where payments are linked to clinical outcomes) are a formal arrangement to jointly address an identified risk in the expected outcomes of treatment,' and suggested that they are 'likely to be required to bring high cost innovative therapies to Australia.'[221]

6.158 The ACvA likewise submitted that the payment system should 'reward technologies for their clinical and economic value.' It then elaborated what it believes this should involve:

> Promote and incentivise Innovative Payment Schemes, namely value-based healthcare (Defined by the World Economic Forum and used by NSW Health: The health outcomes that matter to patients relative to the resources or costs required), to foster early coverage of innovation, and help subsequent evaluation by relevant payers and authorities. For example, novel devices such as wearables are often fitted in the hospital on the day of discharge and worn by the patient in the community. This could be regarded as a service to the hospital (freeing up beds), the patient (allowing them to recover at home) and the physician (giving them flexibility in patient treatment).[222]

6.159 Johnson & Johnson similarly suggested that 'value-based healthcare….has the potential to improve system outcomes.' It identified two varieties: value-based contracting, 'where drug or device contracts are negotiated directly with payers based on the achievement of desired outcomes;' and value-based procurement, where 'tenders reflect consideration of broader outcomes beyond the lowest price.' It was particularly supportive of the use of the latter 'to guide investment decisions in public hospitals,' arguing that

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219 AHHA, Submission 68, p. 1.

220 AHHA, Submission 68, p. 2.

221 Takeda, Submission 66, p. 6.

222 ACvA, Submission 76, p. 10.

the Commonwealth and state and territory government should incorporate it into their reform activities under the *National Health Reform Agreement*.[223]

6.160 The issue of the development, approval and funding of antimicrobials is discussed in Chapter 10.

Post-assessment matters

The appeal process

6.161 STA submitted that the PBAC's independent review (that is, appeal) process should be 'extended to independent review of positive recommendations where that recommendation is not consistent with the original application.' It also recommended that 'fees for independent review be removed so that individual patients and patient groups can seek an independent review.' [224] Better Access similarly suggested 'include options for introducing independent review and appeals processes accessible to the community and individual consumers not just the sponsors of medicines and technologies.'[225]

Delisting and ensuring supply

6.162 The process by which medicines are delisted, or removed from the PBS, received comparatively little attention during the inquiry. The NSW Government submitted that:

> Currently, regulation in Australia is too focused on the approval process. Early access could be further expedited if a robust process for renegotiation of prices, disinvestment and delisting was developed by the Commonwealth in collaboration with States and Territories. This could lower the threshold for approval and simplify economic analysis if approvals were initially time-limited to enable local collection of data.[226]

6.163 The Department told the Committee:

> From time to time, medicines are delisted from the PBS Schedule. This generally occurs at the request of the company responsible for the supply of the medicine in Australia. Reasons vary, but may include the listing of newer,

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223 Johnson & Johnson, Submission 134, pages 13-14.

224 STA, Submission 7, p. 6.

225 Better Access, Submission 160, p. 7.

226 NSW Government, Submission 93, p. 16.

> more advanced alternatives or changes in clinical practice that reduce market share, supply issues, a material change in the cost of imported supply, that the medicine is now available without a prescription (over-the-counter), or it is discontinued for other commercial reasons.
>
> When a sponsor submits a request to remove a medicine from the PBS Schedule, advice is often sought from the PBAC. In these instances, one of the matters which the PBAC provides advice on is whether the delist will result in an unmet clinical need for patients. If the PBAC notes the potential for an unmet clinical need, then it may ask the Department to investigate alternative arrangements.
>
> Ultimately, pharmaceutical companies make their own decisions about whether they intend to delist a medicine from the PBS Schedule and cannot be compelled by the Government to keep supplying a medicine on the PBS.[227]

6.164 It noted that as of July 2021 there were 905 drugs in 2428 forms, marketed as 5401 brands, so 'collating statistics on the average time a medicine stays listed on the PBS would represent a substantive resource investment by the Department.'[228]

6.165 The Association of Australian Medical Research Institutes argued that where a sponsor decides to delist a medicine on commercial grounds but a patient population relies on that medicine:

> A funding mechanism needs to be developed to continue the treatment for those patients…as well as to allow others to acquire the necessary intellectual property or licencing to allow broader continued production and supply.[229]

6.166 This was a view echoed by the PBAC itself, which submitted:

> The PBAC is aware of a number of situations where requests for deletion of medicines from the PBS have been driven by small demand for a product even though it may have an important place in current clinical practice. It is also aware of repeat requests for deletion, in effect repeated requests for price increases, claiming that the PBS price is not financially viable but where the size of the requested price increase is poorly justified.
>
> The PBAC notes there may be a need for last resort mechanism to directly source providers for such medicines including potentially from suppliers not

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227 Department of Health, Submission 15.7, pages [4]-[5].

228 Department of Health, Submission 15.7, p. [3].

229 AAMRI, Submission 88.1, p. 3.

currently active in Australia to keep essential medicines available on the PBS.[230]

Committee Comment

6.167 The Committee thanks the members of the PBAC and the staff of the Department's Technology Assessment and Access Division for their information sharing and assistance with this inquiry.

6.168 The Committee believes that the volume and variety of evidence it received on the HTA system in general and the PBAC in particular, is a testament to the pressure on the system, and the various interests it must try to balance. The Committee's overall view is that the PBAC is generally performing well in coping with that pressure and balancing those interests.

6.169 The Committee agrees with the widely held view among submitters that after the reforms that were made to the TGA following the *Sansom Review* the other aspects of the HTA system are lagging behind. Consequently the Committee welcomes the Australian Government's announcement of the forthcoming independent Health Technology Assessment Review (the HTA Review), which it hopes will finish the job the *Sansom Review* started in improving patients' access to medicines.

6.170 While the Committee recognises that many of the issues raised in this inquiry will be considered as part of the HTA Review, it believes that there are reforms that can be implemented now for the benefit of patients.

6.171 The Committee welcomes the development of the role of the PBAC Executive, and believes its role should be expanded and formalised to fast track specific assessment processes. The Committee believes that the PBAC and Department of Health (the Department) should determine what the scope of the Executive's role should be and what changes are required to legislation.

6.172 While it was understandable that concerns were raised regarding the interaction of the PBS with hospitals and the conduct of HTA by hospitals, some of these concerns were more relevant to the structural organisation of the health system than the subject matter of the current inquiry. The Committee notes that it has long been common for patients to receive some of their treatment in a hospital setting and some as outpatients, although this may become more frequent as medical technology advances. The Committee

230 Department of Health, Submission 15.3, p. 7.

urges the Australian Government to continue to work with the states and territories to ensure that patients receive treatment where it is safest and most efficacious for them, and that there are no gaps in continuity of care.

6.173 The Committee acknowledges that the HTA system is complex and sometimes confusing. The Committee welcomes the Department's creation of the Health Products Portal (HPP) for PBAC applications and the plans to expand it to cover the TGA and other HTA bodies. The Committee believes there is potential for a 'pre-submission advice framework,' as was proposed by some submitters, to be made available to users of the HPP. In the Committee's view, the broader relationship of the TGA, PBAC and MSAC should be considered as part of the independent HTA Review. On the evidence before this inquiry – including on the unique role of the MSAC, discussed in Chapter 7 – the Committee regards them all as fulfilling important and distinct roles and does not support any proposed merger, but recognises the value of enhanced integration and an increased harmonisation of evidentiary requirements.

6.174 The Committee believes that there is an opportunity for more international cooperation and alignment in HTA. This is such a broad subject that the Committee considers it should be left up to the HTA Review to finalise what approach Australia should take in this area, but in the meantime it encourages the Department to work to strengthen relationships with comparable HTA bodies overseas. The Committee believes that a formal arrangement similar to the Access Consortium of which the TGA is a member would be the best mechanism to facilitate this.

6.175 The Committee believes that the HTA system should be as transparent as possible, and that the performance of the PBAC and other HTA bodies should be measured just like other elements of the health system. The Committee notes that the PBAC is in a unique position, in that its performance is heavily dependent on the quality of submissions made to it by sponsors, and is closely tied to the conduct of price negotiations between sponsors and the Department of Health. Nonetheless, the Committee believes that the international benchmarking would increase the transparency of the HTA system. In addition, the Committee believes the Department of Health should table an annual update of its KPIs regarding TGA regulation and HTA processing times in Parliament.

6.176 The Committee received mixed evidence on the state of communications between industry and the Government, both during the HTA process and more generally. On communication during the HTA process, the Committee

is hopeful that the joint pre-submission advice and HPP's will go some way to steering sponsors in the right direction to submitting successful first-time applications. The Committee encourages the Department of Health and PBAC to be as communicative as possible during the HTA process and that this should be further refined during the HTA Review.

6.177 In relation to the PBAC's fee regime, the Committee believes that a different approach is needed. Instead of trying to define the appropriate scope of a fee waiver, the Committee supports a move to a HECS-style system providing application fee waivers for Australian start-up companies, orphan drugs and companies with revenue of under $50 million per annum. Submission fees would only be payable for successful submissions once the drug has been listed and has earned a specified amount of revenue in the Australian market to promote innovation. In addition, the Committee believes a sliding scale of fees should be considered for resubmissions, with fees being lower for resubmissions.

6.178 It was clear to the Committee that there was great interest in the potential of Managed Access Programs (MAPs), although this was very much out of step with the low uptake of such programs currently. The Committee shares the optimism of many submitters about these programs, and believes that should form a key feature of the system in the future. However, the Committee is mindful that they come with considerable risks for the Australian Government, both in terms of managing patient expectations and understanding and preventing industry from exploiting them in price negotiations. Given the importance of these programs and the difficult balance to be struck, the Committee considers that these should be considered more fully by the independent HTA Review, together with the closely related issue of the collection and use of Real World Evidence (RWE). In the meantime, the Committee believes that the Australian Government should do what it can to encourage uptake of these programs, specifically providing for them in legislation, as was requested by the PBAC.

6.179 The Committee agrees with the PBAC that the issues raised about how it values medicines are for the most part too technical to be considered properly in the context of this inquiry. The Committee's opinion is that the HTA process should take a broader view of the costs and benefits associated with a particular submission, and that it should ascribe more value to long-term benefits, including lifestyle benefits, and the long-term benefits of preventative medicines such as vaccines in particular. The Committee believes these matters should be considered as part of the independent HTA Review.

6.180 The Committee notes that there has not been a review of the National Immunisation Program since its establishment over 20 years ago. The Committee recognises the vital role that vaccines play in addressing many diseases, including its importance in providing protection against COVID-19, and therefore recommends that the Department of Health conduct a review of the National Immunisation Program. The Committee believes it is important that this review reform existing approaches used to value vaccines to facilitate early and rapid deployment of vaccines in Australia.

6.181 The Committee is particularly concerned by what appears to be a narrow focus on other medicines or devices in the selection of comparators, rather than a more holistic consideration of what other treatments might benefit the patient. On the evidence provided to the Committee, the current system depends almost entirely on the commercial initiative of sponsors. While there is a mechanism for the sponsor of a new medicine, drug A, to have that medicine displace an old medicine, drug B, in clinical practice, there is no mechanism to compare how another modality such as a lifestyle therapy performs against drugs A and B. The Committee believes that the PBAC process would benefit from adopting elements of NICE system in the UK, in which comprehensive scoping is conducted with input from patients and clinicians. The Committee hopes that this may assist in the problem identified by some sponsors of comparators being chosen on cost rather than clinical efficacy. The Committee recommends the establishment of an Office of Clinical Evaluation within the Department of Health to assess the best and most effective care for patients in the context of new and emerging health technologies.

6.182 The Committee notes the concerns expressed by many pharmaceutical companies about the use of international reference pricing overseas, particularly its potential use in the United States. This could have profound impacts on the Australian HTA system and access to medicines and technologies by Australian patients. The Australian Government must therefore maintain a watching brief on this issue and be prepared for changers that may be required to our own reimbursement system.

6.183 The Committee was interested to hear about the experiments that are being conducted in value-based payment models, including in Australia, and believes that there is much potential for these in the future. Accordingly, the Committee considers that their potential should be evaluated by the independent HTA Review.

6.184 If an application has been rejected by the HTA process, the Committee is supportive of the need for an independent review process. Such a process should be triggered where there is agreement between the Department and the applicant, sponsor, clinician and or patient advocacy group, that there is a clear case for a review.

6.185 The issues of delisting of medicines from the PBS and securing ongoing supply are further problems on which the Committee heard relatively little evidence. The Committee however, believes that there is considerable merit in the PBAC's suggestion of a last resort mechanism to secure supply of necessary medicines that are in danger of being delisted.

7. The Medical Services Advisory Committee

Introduction

7.1 While the Pharmaceutical Benefits Advisory Committee (PBAC) was the element of Australia's Health Technology Assessment (HTA) system that received the most attention from submitters, many discussed the Medical Services Advisory Committee (MSAC), and to a lesser extent the Prostheses List Advisory Committee (PLAC). Most of the major issues raised were similar to those discussed in the previous chapter in the context of the PBAC and the HTA in general, although there were some important differences. Indeed, the extent to which the same principles can be applied to the HTA of medicines and that of devices was itself a controversial issue.

7.2 As outlined in Chapter 3, there are some major differences between the PBAC and MSAC in terms of structure, process, and even authority, with the PBAC being legislated while the MSAC is non-statutory. Importantly, while the MSAC's primary role is to assess services for inclusion in the Medicare Benefits Schedule (MBS), it serves as a fall-back option for HTA of technologies that do not otherwise have a clear pathway through the system.[1]

7.3 It should be noted that a new version of the *Guidelines for Preparing Assessments for the Medical Services Advisory Committee* (MSAC Guidelines) were published in May 2021, after submissions to the inquiry had closed and

[1] Department of Health, Submission 15.6, p. 12.

most public hearings had been concluded.[2] This means that some of the evidence provided to the Committee may have been superseded, and given the highly technical nature of the MSAC Guidelines it has not been possible for the Committee to compare them comprehensively against that evidence. A brief discussion of the new MSAC Guidelines is included at the end of this chapter.

Length of review

7.4 The general view of submitters on the MSAC was that it has various features that require improvement. As was the case for other elements of the regulatory and reimbursement system, there was a common view among submitters that the current MSAC processes take too long to be completed and need to be accelerated.[3] The Medical Technology Association of Australia (MTAA) stated that 'industry's experience is that the entire process for undergoing MSAC review and getting access following this review is very lengthy, frequently two years or more.'[4] BioMarin Pharmacetical Australia was critical:

> Similarly, the…MSAC representatives recently stated that the length of the process was about 12 months and that typically, applications were not successful, and that it is not humanly possible to have a faster process. This accepted paradigm results in significant stakeholder frustration, long delays for patients, and significant resource burden for the Department of Health and sponsors. This may even make it less viable for many smaller companies to bring new treatments for rare diseases to Australia in a timely manner.[5]

Resourcing

Funding

[2] Department of Health, 'Guidelines for preparing assessments for the Medical Services Advisory Committee', Canberra, May 2021, www.msac.gov.au/internet/msac/publishing.nsf/Content/4599F67A7A885C2DCA2586E000799979/$File/MSACGUIDE-Summary%20for%20stakeholders-08-FINAL(18May21).pdf, viewed 15 October 2021.

[3] Specialised Therapeutics Australia (STA), Submission 7, p. 5; Australian Cardiovascular Alliance (ACvA), Submission 76, p. 5; Edwards Lifesciences, Submission 83, p. 2; Dr Sue O'Malley, Submission 150, p. [3].

[4] Medical Technology Association of Australia (MTAA), Submission 148, p. 46.

[5] BioMarin Pharmaceutical Australia, Submission 152, p. 1.

7.5 Unlike the Therapeutic Goods Administration (TGA) and the PBAC, the MSAC does not currently operate on a cost recovery model.[6] Specialised Therapeutics Australia (STA) acknowledged that the extra resourcing that would be involved in the implementation of its recommendations (discussed below) 'may require consideration of cost-recovery.'[7] Better Access Australia (Better Access) similarly recommended that the Committee 'consider expanding fees to other parts of the system [beyond PBAC] to support more robust timeframes and transparency of processes in all subsidy assessment committees.'[8]

Expertise

7.6 There was some commentary on the expertise currently available to the MSAC. STA suggested there is 'a potential lack of expertise in assessing novel personalised/genomic medicine and technologies.'[9] The MTAA was much stronger in its criticism, stating:

> MSAC consists of many high-quality experts from a range of fields, including health economics. However, it must cover a wide spectrum of technologies and disease states….the types of medical devices that will need to be evaluated in the future will be diverse. Of particular note is the central role of information and digital technology in many of these future devices. MSAC appointees already have limited knowledge of bioengineering in comparison to genetics or immunology for example. The growth of digital health will likely require the addition of expertise in this area as well. Heavy reliance on TGA will be important in the future but this should be augmented with other expertise within MSAC and, potentially, the secretariat, in order for medical devices to be properly assessed.
>
> Even with the spread of expertise within MSAC, specialised knowledge in a particular procedure or device can be lacking. For instance, an interventional cardiologist may not be experienced as an electrophysiologist and have no direct experience in the use of technologies for these purposes, even though broadly they are in the same specialty. This direct experience is even more important with medical devices than biopharmaceuticals, because the clinician

6 Better Access Australia (Better Access), Submission 160, p. 20.

7 STA, Submission 7, p. 6.

8 Better Access, Submission 160, p. 7.

9 STA, Submission 7, p. 13.

> is often physically manipulating the device in question and the interrelationship between device and user is much closer.[10]

7.7 It expressed concern with the 'variable' quality of assessments performed by external contractors, which it suggested could be due to the fact that most expertise in health economics is in pharmaceuticals, rather than devices.[11]

7.8 AusBiotech noted the challenges novel technologies will pose to the MSAC and the system in general, and called for:

> Broad education of regulatory and reimbursement health workforces to build understanding of the benefits, limitations and risks as well as the ethical, legal and societal impacts of emerging areas. Better connection within these workforces to expedite approvals for therapeutic products that cut across a number of disciplinary practices.[12]

International cooperation and harmonisation

7.9 The need for alignment with international processes was a theme that ran throughout the inquiry, and while most submitters spoke in general terms or focused on PBAC, as discussed in Chapter 6, there was clearly much that was applicable to MSAC. The University of Melbourne, for example, commented that:

> Australia's approval process for new drugs and medical technologies should continue to look to international best practices and innovations with a view to ensuring all Australians can access new therapies wherever an available, high-quality evidence base can support it.[13]

7.10 STA stated that 'MSAC is "out-of-step" with equivalent global decision-makers.' It argued that 'taking into account the decisions of internationally equivalent decision makers would alleviate the burden' on MSAC, as well as providing a 'level playing field' for patients.[14] Dr Sue O'Malley claimed that the MSAC 'is "out of step" with clinical evidence requirements in the rest of the world.'[15]

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10 MTAA, Submission 148, pp. 47-48.

11 MTAA, Submission 148, p. 48.

12 AusBiotech, Submission 114, p. 14.

13 University of Melbourne, Submission 133, p. 3.

14 STA, Submission 7, pp. 15-16

15 Dr O'Malley, Submission 150, p. [3].

7.11 Medicines Australia likewise argued that 'MSAC decision-making does not show the same flexibility towards newer technologies when compared with other countries with similar national health systems, for example the United Kingdom (UK), Canada, and France.'[16] Myriad Genetics made a similar case:

> We propose that MSAC recognise validity of international guidelines and more cost-effective methods of clinical validation that provide sufficient evidence of safety and efficacy. At present, Australia will continue to be left behind in the implementation of novel, personalised diagnostics as the costs to meet the criteria expected is beyond most IVD [*in vitro* diagnostics] companies.[17]

The application process

Consistency and transparency of processes

7.12 In the opinion of some stakeholders the MSAC lags behind the PBAC in the consistency and transparency of its processes. Medicines Australia, for example, submitted that:

> Through years of refining and feedbacks, the…PBAC processes are better established, where milestones and deadlines are clearly laid out for companies seeking PBAC consideration of a submission. This is not the case for the…MSAC.
>
> ….
>
> As such, it is proposed that there is greater transparency and alignment of the MSAC and PBAC processes and guidelines, including (but not limited to) the publication of MSAC calendar, detailing milestones such as the availability of ratified MSAC Minutes and timing of Public Summary Documents, publication of MSAC agenda and outcomes at specified times (comparable to the long-standing practice of the PBAC).[18]

7.13 Better Access similarly argued that:

> …timing and access points [for PBAC] are standardised and clear and provide for detailed analysis of their recommendations and processes albeit not reporting statistically on them themselves.

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16 Medicines Australia, Submission 141, p.

17 Myriad Genetics, Submission 47, p. [2].

18 Medicines Australia, Submission 141, p. 36.

> Conversely, the processes of MSAC including referrals from other bodies such as the [National Blood Authority] represent considerable uncertainty and vaguery [sic] in process and timeframes for access and subsidy.[19]

7.14 Other submitters simply focused on the MSAC's own processes. Novo Nordisk Oceania called for 'MSAC processes and timeframes [to] be standardised and expedited.'[20] Bayer Australia and New Zealand recommended:

> Greater transparency and consistency of MSAC processes, such as:
>
> - Publication of MSAC calendar which details key milestones such as ratified MSAC minutes and timing of Public Summary Documents
> - Publication of MSAC agenda and outcomes at specified time[21]

7.15 AstraZeneca Australia (AstraZeneca) similarly proposed:

> …greater transparency and consistency of MSAC processes, including (but not limited to):
>
> - Publication of MSAC calendar (comparable to the long-standing practice of the PBAC agenda), detailing milestones such as availability of ratified MSAC Minutes and timing of Public Summary Documents
> - Publication of MSAC agenda and outcomes at specified times (as with PBAC)[22]

7.16 STA criticised the transparency of the MSAC's decision-making. It suggested:

> That the MSAC processes and timeframes be standardised and expedited, including significant improvement in transparency of external data and contributions and time to provision of minutes from the meeting to allow sponsors to re-submit as quickly as possible and improve patient access.[23]

7.17 It proposed 'that an independent review process for MSAC decisions be introduced similar to that of the PBAC.'[24] BXTAccelyon similarly indicated that when it requested the MSAC to review a decision 'after several months

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19 Better Access, Submission 160, p. 20.

20 Novo Nordisk Oceania, Submission 151, p. 4.

21 Bayer Australia and New Zealand, Submission 175, p. 7.

22 AstraZeneca Australia (AstraZeneca), Submission 42, p. 5.

23 STA, Submission 7, p. 6

24 STA, Submission 7, p. 6.

we received a one line response refusing any reconsideration without addressing any of the concerns raised in the submission.'[25]

7.18 The Australian Cardiovascular Alliance (ACvA) demanded 'greater transparency and consistency in MSAC processes.' It submitted that this should involve:

> Publication of an MSAC calendar (comparable to the long-standing practice of the PBAC agenda), detailing milestones such as availability of ratified MSAC Minutes and timing of Public Summary Documents is strongly recommended, as is publication of an MSAC agenda and outcomes at specified times (as with PBAC).[26]

7.19 Edwards Lifesciences had some general criticisms of the MSAC's 'accountability and transparency.' Its solutions to these issues included having the MSAC (and PLAC) appear before a Parliamentary Committee once a year, and for each body to publish 'the number of applications submitted, approved and refused' annually.[27]

Engagement with sponsors

7.20 Some submitters were unhappy with how the MSAC engages with sponsors, in line with many of the concerns just discussed, as well as those raised in relation to other elements of the regulatory and reimbursement system. Myriad Genetics stated that:

> We believe all interested parties would be better served through a more collaborative and consultative approach. This would improve the evidence collected and needed to satisfy MSAC's requirement, as well as decrease the repeat submissions received and evaluated. Japan's MHLW is an example of this collaborative approach. Consultation happens prior to a submission, before extensive evaluation implemented. Delivering novel personalised diagnostics to its population is significantly more efficient than in Australia.[28]

7.21 The MTAA noted some deficiencies in this area compared with the PBAC's processes:

> Pre-submission meetings for MSAC are possible but not openly advertised on the MSAC website as they are for the…PBAC process. In fact, a number of our

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25 BXTAccelyon, Submission 164, p. [3].

26 ACvA, Submission 76, p. 13.

27 Edwards Lifesciences, Submission 83, p. 37.

28 Myriad Genetics, Submission 47, p. [2].

> members were not even aware that they were possible when MTAA raised this. This opportunity should be clearly spelled out including the ability to bring subject matter experts into the discussion.
>
> Furthermore, unlike major submissions for the PBAC, there is no opportunity for sponsors or their invited experts to address the MSAC meeting, which is needed to enable good decision making.[29]

The importance of pre-submission meetings was stressed by Pathology Technology Australia (PTA).[30]

7.22 Dr O'Malley was more concerned with the feedback process for unsuccessful submissions:

> The current approval process, especially that for novel medical technologies, is not constructive, that is, it does not encourage the development and implementation of novel medical technologies in Australia. A quick read of any of the Public Summaries of 'failed' MSAC Applications will inform you of what was lacking, usually clinical evidence, but will not tend to have any positive, practical suggestions of how to overcome this lack of clinical evidence. It is not really helpful to say that the application lacked level I or II clinical evidence.[31]

Interaction with the Therapeutic Goods Administration

7.23 There was considerable support for better coordination between the TGA and the MSAC. Abbott Diabetes Care noted that parallel processing is not available for TGA and MSAC submissions as it is for the PBAC. It recommended that this be introduced, which it said 'will increase efficiencies and reduce the time to reimburse critical technologies.'[32] This idea was put forward by Stryker South Pacific (Stryker), the ACvA, Edwards Lifesciences and Better Access.[33]

7.24 The MTAA commented that:

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29 MTAA, Submission 148, p. 47.

30 Mr Dean Whiting, Chief Executive Officer, Pathology Technology Australia (PTA), *Committee Hansard*, Canberra, 18 June 2021, pp. 11-12.

31 Dr O'Malley, Submission 150, p. [3].

32 Abbott Diabetes Care, Submission 191, p. 2.

33 Mr Maurice Ben-Mayor, President, Stryker South Pacific (Stryker), *Committee Hansard*, Sydney, 12 March 2021, p. 30; ACvA, Submission 76, p. 13; Edwards Lifesciences, Submission 83, pp. 32-33; Better Access, Submission 160, p. 6.

> One additional point of relevance is that MSAC and HTA committees generally need to avoid repeating evaluations already undertaken by the TGA. While the comparative safety of a device is relevant to HTA, whether the product is sufficiently safe has already been established by the TGA. Nonetheless, this question can sometimes be revisited at MSAC.[34]

7.25 STA likewise argued that 'the role of the TGA in determining safety and efficacy should be given higher weighting by the MSAC.'[35] It recommended that the TGA and MSAC work together to create a 'national list of novel health technologies recently approved…to allow for transparent reporting on their assessment and adoption.'[36]

7.26 Adjunct Professor John Skerritt, Deputy Secretary, Health Products Regulation, Department of Health, told the Committee that:

> Finally, a number of submissions said MSAC and TGA should work more closely together. I think that's worthy of exploration, although it's important to realise that MSAC looks at services. Sometimes the product is only part of that service, and often the product is not one particular company's product but a class of products. Of course, as a regulator, we look at individual commercial products; we don't register products by class. There's often an issue of differences in timing of submissions. A product may go onto the register and after a few years of use it seems that it will have a valuable and potentially cost-effective role as part of a service. So while I think alignment between TGA and MSAC is something that we need to do a bit more work on, it is not as straightforward as, for example, parallel processing in medicine applications.[37]

Novel technologies

7.27 Several submissions specifically addressed issues relating to the MSAC's assessment of novel technologies in general, or to specific types of technologies. Medtronic Australasia (Medtronic) that:

> Reimbursement arrangements can create perverse incentives not to adopt newer technology, despite the benefits to patient outcomes and total healthcare costs. Funding policies that dictate where technology has to be

34 MTAA, Submission 148, p. 49.

35 STA, Submission 7, p. 5.

36 MTAA, Submission 148, p. 42.

37 *Committee Hansard*, Canberra, 18 June 2021, p. 17.

> delivered can also create additional inefficiencies across the healthcare system.[38]

7.28 The MTAA recommended that the TGA and MSAC work together to create 'national list of novel health technologies recently approved…to allow for transparent reporting on their assessment and adoption.'[39]

7.29 AstraZeneca called for increased flexibility in the MSAC's processes 'to fast-track urgent…devices.'[40] This was echoed by the ACvA, which described it as a matter of 'urgency.'[41] Medtronic argued that 'establishing a process for breakthrough, high cost or highly specialised technologies would enable a more strategic national approach to patient access.'[42]

7.30 Roche Australia (Roche) raised concerns with how the MSAC's service-based funding model might work for some novel technologies. It noted that:

> …there is no subsidy or reimbursement scheme for patients for many medical devices including software or artificial intelligence technologies. While technically, the MBS can be used to fund a number of technologies, it is not set up to reimburse patients for the use of a technology, only medical service providers. The only specific schemes providing support to patients for the purchase of medical devices and aids are in disease-specific conditions such as the National Diabetes Supply Scheme, the Continence Aids Payment Scheme, the Stoma Appliance Scheme and the National Disability Insurance Scheme.[43]

7.31 PTA addressed the challenges and opportunities posed by digital pathology. It argued that:

> It is likely that some measure of value to patient outcomes and the healthcare economy will be delivered by data mining, correlating genomic data with clinical syndromes and with known therapies. Consideration needs to be given to how such data analysis is structured, regulated and funded.[44]

7.32 It submitted that 'Australia is falling well behind the developed world in our deployment of Point of Care Testing (POCT)' (that testing conducted where

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38 Medtronic Australasia (Medtronic), Submission 122, p. 3.

39 MTAA, Submission 148, p. 42.

40 AstraZeneca, Submission 42, p. 4

41 ACvA, Submission 76, p. 13.

42 Medtronic, Submission 122, p. 17.

43 Roche Australia (Roche) Submission 92, p. 15.

44 PTA, Submission 178, p. 5.

care is delivered, as opposed to the samples being sent to a laboratory). It suggested that 'mostly because there is no framework or funding for POCT.'[45]

Post-recommendation process

7.33 There was criticism of how positive recommendations from the MSAC are treated. The MTAA explained that:

> …after a positive recommendation the time for the Government to act on the decision is indefinite. Unlike Pharmaceutical Benefits Scheme (PBS) listings which, as of this [2020] October Budget, now have their own allocated funding in the Budget and can be announced at any time, MBS listings can disappear into the Budget process for a long period, are only announced at the Budget or MYEFO and still require a financial offset from within the Health portfolio.[46]

7.34 It asked for 'Government to commit to funding MSAC recommendations in a timely way, similar to PBAC.'[47]

7.35 AusBiotech similarly submitted that:

> …unlike PBAC, MSAC does not have specified timeframes and the Health Minister does not have authority to sign off recommendations under $20M without cabinet approval. MSAC recommendations are also tied into the budgetary cycle.[48]

Provisional access

7.36 The idea of allowing provisional or interim access to devices while further evidence is collected on their efficacy and potentially safety received strong support, just as it did for medicines. Unlike for medicines, there is no scope for this to occur under the current system, as was explained by Medtronic:

> Managed Entry Agreements (MEAs), which are routinely used for early access or interim funding of medicines whose clinical evidence or cost-effectiveness is uncertain at the time of application/market entry, could also have potential to be utilised for non-drug technologies.
>
> MEAs including Coverage with Evidence Development (CED) or risk-sharing agreements are suitable for devices and diagnostics where there is less

45 PTA, Submission 178, p. [6]

46 MTAA, Submission 148, p. 46.

47 MTAA, Submission 148, p. 7.

48 AusBiotech, Submission 114, p. 13.

> 'traditional' Clinical Trial evidence at launch but potentially an increased ability to collect real-time data that could be used to help answer these uncertainties through a remote patient monitoring platform.
>
> Coverage with Evidence Development (CED) has been utilised across a number of jurisdictions since its introduction by the US Centers for Medicare and Medicaid Services (CMS) in 2005.
>
> Australia has experimented with CED for devices in some forms in the past, but an established model has not been embedded in our HTA processes for non-drug technologies. Formal CED acceptance would enable earlier patient access to innovative technologies.[49]

7.37 Dr O'Malley provided a table of 17 past MSAC applications that were granted interim funding, with the first listed in 1997 and the last in 2007.[50] She argued that:

> Arising out of the uncertainty in decision making in any healthcare sector, decision makers are faced with the dilemma of determining which has the greater risk: making available medical procedures that are ineffective or even harmful (Type I error) or denying access to medical procedures that are beneficial and efficient (Type II error).
>
> In almost all cases, a Type I error is self-correcting since practitioners will cease to perform the medical procedure if it proves to be ineffective or unsafe. The Type II error is by far the more serious since it is not self-correcting. If a procedure is refused MBS funding it is extremely unlikely that the procedure will ever be performed in Australia.[51]

7.38 After providing the list of past instances of interim funding she stated:

> I believe that the reasons for the discontinuation of this interim funding pathway can be overcome and are far outweighed by the advantages. A quick check of this list shows that virtually all of these procedures are still MBS listed.[52]

She did not detail what the reasons for the discontinuation were.

7.39 The ACvA pointed to the US example, suggesting:

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49 Medtronic, Submission 122, pp. 16-17.

50 Dr O'Malley, Submission 150, p. [4].

51 Dr O'Malley, Submission 150, p. [3].

52 Dr O'Malley, Submission 150, p. [4].

> … a market entry scheme for devices, along the model of the US Centres for Medicare and Medicaid Service (CMS), which has introduced a Medicare Coverage of Innovative Technology (MCIT) and Early Feasibility Studies in the framework of the new EU Medical Devices Regulations.[53]

7.40 The US approach was noted by the Australian Clinical Trials Alliance (ACTA), along with the UK Cancer Drugs Fund. It suggested that neither of these approaches could simply be copied for use in Australia, but they both offer useful lessons. It stated its belief that:

> …the approval pathway for certain new services/technologies that are likely to be significant to patients and represent a high-cost to the MBS should be expanded to incorporate the conditional listing of the new item subject to the collection and analysis of robust clinical and patient-centred outcome data through either a randomised clinical trial or a clinical quality registry.[54]

7.41 It argued that this approach would provide support for investigator-led clinical trials and clinical quality registries.[55]

7.42 The Rare Disease Industry Working Group (RDIWG) noted that many 'novel technologies…will provide significant long-term health benefits for patients,' but 'may have limited data at the time of assessment.' It therefore argued that

> 'there should be a focus on the development of innovative access mechanisms to ensure patients have the advantage of being able to access treatment in parallel to the long-term collection of real-world evidence (RWE).[56]

7.43 The issue of the collection and use of RWE is discussed in more depth below.

7.44 The introduction of interim access for devices was supported by Stryker, Johnson & Johnson and Edwards Lifesciences.[57] The latter suggested that this could be 'along similar lines' to the Managed Access Programs used for medicines and 'could include the use of registries and dedicated multidisciplinary centres of excellence which have experience with these

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53 ACvA, Submission 76, p. 6.

54 Australian Clinical Trials Alliance (ACTA), Submission 149, pp. 6-7.

55 ACTA, Submission 149, p. 7.

56 Rare Disease Industry Working Group (RDIWG), Submission 51, p. 3.

57 Stryker, Submission 28, p. 15; Johnson & Johnson, Submission 134, p. 10.

high-value medical devices offering treatment to patients living with conditions where there is a recognised high unmet need.' [58]

7.45 Sleepfit Solutions focused on the specific issue of approval of digital therapeutics (DTx) and championed the German approach, which features a specific pathway for digital products introduced in 2020, known as the Fast-Track Process for Digital Health Applications (DiGA). It submitted that

> The key features of the German process are:
>
> - Clearly articulated with maximum transparency as a key focus…
> - Initial assessment of the DTx by the German government is completed within 3 months, after which time the DTx can be provisionally listed and prescribed by clinicians or requested by patients. At this point the DTx is reimbursable by insurers
> - Provisional listing allows the 'manufacturer' to gather enough evidence of positive healthcare effect to allow for full admission to the DiGA Directory. The evidence requirements are again, clearly outlined, and guided by recognised international standards.[59]

Approaches to evidence

Real World Evidence

7.46 As noted by the RDIWG, a closely related issue to the possibility of providing provisional access to devices is the collection of RWE.[60] In the medical devices context, Stryker explained the role that RWE should play in provisional access as follows:

> In relation to the introduction of innovative technology (without adequate clinical evidence or potentially without an adequate comparator) the ability to commit to an ongoing post-market clinical follow-up in lieu of excessive pre-market evidence generation is also important to enable access in both the public and private sectors. This should include maintaining reporting requirements and the ability to halt access should early issues be identified.[61]

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58 Edwards Lifesciences, Submission 83, p. 33.

59 Sleepfit Solutions, Submission 198, pp. [4]-[5].

60 RDIWG, Submission 51, p. 3.

61 Stryker, Submission 28, p. 15.

7.47 Edwards Lifesciences took a broader view, emphasising the need for RWE to be considered an important part of the evidence for device approval in general:

> The draft MSAC guidelines which are currently being reviewed need to adopt a more flexible approach to the levels of data and evidence it will accept. MSAC should look at the broader lifecycle approach to HTAs thus a greater need for incorporating observational and real-world-data into the assessment process. Further, unlike our pharmaceutical counterparts, device companies are dependent on issues outside our control including the surgeon's skills implanting the device.[62]

7.48 It recommended 'giving greater weight to other forms of evidence beyond clinical trials, including real-world evidence.'[63]

7.49 Ms Susan Martland, Member, PTA, told the Committee:

> Certainly at Pathology Technology Australia we believe that the MSAC guidelines really need to accommodate the realities of evidence generation and look carefully at some real-world evidence not only that can be generated in local jurisdictions but that has already been generated in international jurisdictions. Things like point-of-care testing, for example, are used widely in Europe. Looking at this real-world evidence can then be considered as an efficient, cost-effective way to assess new and existing healthcare technologies.[64]

7.50 Novartis Australia and New Zealand (Novartis) meanwhile called for 'a consistent approach, supported by Government, for the generation of [RWE] via registries to address evidence gaps in economic evaluations.'[65] Medtronic stated that:

> Real world data (RWD) sources can include routinely collected healthcare data from a variety of sources including electronic health records, government agencies, medical societies, product and disease registries and patients. The main issue for generating RWE studies from these sources in Australia is our inability to access this data. In Australia, RWD sources are characteristically fragmented and there is a complex data privacy and governance landscape

62 Edwards Lifesciences, Submission 83, p. 31.

63 Edwards Lifesciences, Submission 83, p. 35.

64 *Committee Hansard*, Canberra, 18 June 2021, p. 11

65 Novartis Australia and New Zealand (Novartis), Submission 138, p. [11].

> operating at different state and local levels that further impact the access and use of RWD.[66]

7.51 Takeda Pharmaceuticals Australia (Takeda) noted the potential for collection of thorough RWE for technologies which will require patients to undergo long-term monitoring for safety reasons, such as cell and gene therapies.[67] AstraZeneca addressed a more specific issue, asking for 'better clarity on how…MSAC …treat real world evidence (RWE) and secondary tiered data sources when addressing off-label and/or pan-cancer treatments.'[68]

7.52 The ACTA recommended:

> Conduct a review of potential reforms to the…MBS, aimed at facilitating the better generation of real-world evidence to improve outcomes and deliver value gains. The review should consider ways to use savings generated through investigator-initiated trials and [Clinical Quality Registries] as a means of funding these activities.[69]

Post-market surveillance

7.53 As discussed in previous chapters, an essential requirement for use of provisional access schemes and RWE is effective post-market surveillance. The University of Melbourne submitted that:

> Post market surveillance mechanisms such as prosthetic registries are often unreliable, requiring years of data collection which is often insufficient in its granularity to provide meaningful interpretation of causes for success or failure. A more pro-active, in-depth interrogation of efficacy, safety and health economic outcomes during the early implementation phase could be undertaken by relevant discipline experts at nationally accredited centres. This would provide a strong evidence base to support broader dissemination (or not) of a new technology. Devices/prostheses are examples of where this is most pertinent as controlled implantation under rigorous scrutiny and subsequent interrogation in specifically credentialed centres is required to truly evaluate cost and clinical effectiveness. Recommendations that support broad adoption of a new treatment should also include recommendations to restrict or prevent existing, less-effective treatment practices where there is strong evidence available. Further discussion is required on the source of such recommendations, which should be independent of funders, driven by the

66 Medtronic, Submission 122, p. 17.

67 Takeda Pharmaceutical Australia (Takeda), *Submission 66*, p. 4.

68 AstraZeneca, Submission 42, p. 3.

69 ACTA, Submission 149, p. 5.

> science and healthcare needs, and enabled by a body of experts in clinical care, health economists, and clinical triallists.[70]

7.54 Stryker meanwhile suggested 'utilising the early adoption of medical technology in Australia's private health sector to collect post-market surveillance and performance data to inform policy, regulatory and funding decisions.'[71]

Evidentiary requirements for different technologies

7.55 One particular challenge for the MSAC is the wide variety of different technologies it is responsible for assessing. Roche commented that there is insufficient guidance available for many of these technologies presently. It submitted:

> There are many technologies where guidance on the HTA requirements is absent, or has been insufficiently developed for sponsors to provide consistent evidence that meets assessment expectations. There is currently no existing guidance for digital health technologies and artificial intelligence, creating uncertainty for sponsors. Similarly for gene and cell therapies, a greater level of granularity in HTA requirements in the MSAC guidelines would assist sponsors prepare submissions and reduce assessment churn. Roche notes that the current review of MSAC guidelines did not provide further guidance on this.[72]

Digital technology and Artificial Intelligence

7.56 TALi Health outlined its work in digital therapeutics and called for 'alignment of the reimbursement processes so that real-life data collected on patients can be a more prominent factor in fast-tracking reimbursement.'[73] Sleepfit Solutions explained some of the difficulties with regulation of digital therapeutics. It noted that many of these difficulties apply to reimbursement, and summed up its argument by saying 'products are continually changing and improving, despite the ongoing need to prove clinical efficacy and health economic value (which typically requires a rigorous and lengthy clinical trial process).'[74]

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70 University of Melbourne, Submission 133, p. 3.

71 Stryker, Submission 28, p. 6.

72 Roche, Submission 92, p. 13.

73 TALi Health, Submission 187, p. [2].

74 Sleepfit Solutions, Submission 198, p. [4].

7.57 The MTAA commented on digital technology:

> …digital technology undergoes constant upgrading. It will be very difficult to generate new clinical data for every innovation cycle. Under the MSAC approach of 'beyond reasonable doubt' evidence levels, this technology will simply be blocked from patient access on the grounds that it is not 'cost-effective.'[75]

7.58 Varian Medical Systems Australasia provided a more specific example of the challenges technology involving artificial intelligence (AI) can face. It described its adaptive radiotherapy technology, which uses AI to assist a clinician to update a patient's radiotherapy treatment plan on a daily basis based on the patient's response to the treatment, as opposed to the traditional approach of developing the plan at the start of the treatment and sticking to it.[76] It explained that this benefits the patient, but the current approach to reimbursement does not account for the extra time involved for the clinician or the use of the AI technology itself.[77]

Diagnostic technology

7.59 Myriad Genetics explained that the MSAC has rejected 10 of its applications for a diagnostic test for a type of breast cancer. It argued that the MSAC is demanding a level of evidence that is unreasonable for such a test, including a randomised controlled trial which it said would be unethical to perform (because some participants would have to miss out on chemotherapy) and would take 15 years to complete. It argued that overseas authorities are do require the same level of evidence, and will accept 'well-designed retrospective trials (e.g. retrospective analyses of prospective data).'[78] STA made similar comments about its experience, apparently for the same test.[79]

Radiopharmaceuticals

7.60 BXTAccelyon made a submission regarding the MSAC's decision not to recommend a radiotherapy for prostate cancer. It submitted that 'MSAC is making decisions that are not reasonably assessing the clinical evidence

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75 MTAA, Submission 148, p. 49.

76 Varian Medical Systems Australasia, Submission 146, p. [5].

77 Varian, Submission 146, p. [6].

78 Myriad Genetics, Submission 47, pp. [1]-[2].

79 STA, Submission 7, pp. 11-16.

available,' that it 'is demanding a level of evidence that is not reasonable,' that 'the current approval process does not appear to measure all treatment options equally' and that it does not pay sufficient attention to clinicians' views.[80]

Alignment with the Pharmaceutical Benefits Advisory Committee

7.61 Some submitters questioned the division of HTA into separate PBAC and MSAC processes.[81] However many in the medical devices sector were adamant that the MSAC is in fact too closely aligned with the PBAC, particularly in its evidentiary requirements, which in the PBAC's case were designed for assessing pharmaceuticals rather than devices. Edwards Lifesciences submitted that:

> Part of the problem lies with MSAC aligning too closely with PBAC. That may work well for the process of pharmaceuticals assessment, which tends to rely on evidence from randomised controlled trials, but it does not suit the medical device sector that need to be measured using a wider range of evidence.[82]

7.62 Similarly, Dr O'Malley commented that:

> The approval process for novel medical technologies…applications to the…MSAC has been modelled on that for new drugs, applications to the…PBAC. This is despite the crucial differences between medical technologies and pharmaceuticals.[83]

7.63 Medtronic noted that 'there are challenges with evidence collection with devices. For instance, it is not possible to generate the same level of evidence through a randomised controlled trial (RCT) with implantable devices.'[84] Abbott Diabetes Care stated that:

> The guidelines for regulatory and reimbursement consideration are focussed and heavily weighted on randomised controlled trials (RCT) with double-blind groups (i.e. participants are blinded to the traditional drug treatment). However, it's impossible to run double-blind groups with [many devices]. Also, the data generated from devices is different given the product cycles and, therefore, may be more practical to run real-world evidence (RWE) to assess healthcare efficiencies. Ethics issues in device trials, sample sizes, all

80 BXTAccelyon, Submission 164, pp. [3]-[4].

81 For example Better Access, Submission 160, p. 22.

82 Edwards Lifesciences, Submission 83, p. 31.

83 Dr O'Malley, Submission 150, p. [3].

84 Medtronic, Submission 122, p. 16.

militate against direct comparison between pharmaceuticals and device therapies.

In practice Health Technology Assessment (HTA) Agencies such as MSAC can have a substantial focus on internal validity with RCTs from Australian HTA bodies that include both Government and academia bodies. It is quite likely that a perfect package of evidence cannot be generated to meet the needs of all decision makers, given the ethical, time and budget constraints for pivotal studies. The power of RWE with large sample sizes after market entry can be greater than the power of small RCTs but RWE is undervalued. Further, effectiveness of a technology in a more generalisable population should be considered equally as high as any available RCT's, particularly when there is substantial RWE demonstrating the effectiveness on a broad population.[85]

7.64 Johnson & Johnson similarly argued:

The current MSAC technical guidelines for therapeutic services and technologies – including the approach to evaluation, evidence quality appraisal and economic evaluation – are historically based on the evidentiary standard for pharmaceuticals included in the PBAC guidelines. The guidelines assume that…HTA methods, including the classical evidence hierarchy, suitable for drugs are suitable for therapeutic services, medical devices and other technology- enabled innovative technologies.

However, classical evidence hierarchy cannot always be applied for medical devices and some new medicines technologies, as…RCTs are difficult (and sometimes impossible) to conduct in a format acceptable to HTA bodies. RCT evidence is not always available and appropriate, especially for devices that have very short product life cycles before a new iteration is available.[86]

7.65 The MTAA submitted that:

The biggest challenge is the expected evidence levels that are applied to new technologies. HTA methodology was essentially developed for the pharmaceutical industry. However, pharmaceuticals typically lend themselves to the development of much more data than do medical devices.[87]

7.66 The MTAA went on to provide a list of some of the relevant differences between medicines and devices, some of which have already been discussed. It then list further challenges that many devices face:

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85 Abbott Diabetes Care, Submission 191, p. 2.

86 Johnson & Johnson, Submission 134, p. 11.

87 MTAA, Submission 148, p. 8.

- Device performance is dependent on operator skill
- Blinded trials often not practicable
- Short life cycles/incremental improvements narrow evidence window
- Low volume in some cases reduces quantity of evidence[88]

The valuation process

A broader concept of value

7.67 As was the case for the PBAC, many submitters felt that too narrow a range of factors is currently considered by the MSAC during its valuation process, and a broader concept of value should be used. The ACvA provided an example:

> …novel devices such as wearables are often fitted in the hospital on the day of discharge and worn by the patient in the community. This could be regarded as a service to the hospital (freeing up beds), the patient (allowing them to recover at home) and the physician (giving them flexibility in patient treatment).[89]

7.68 It claimed that 'widespread adoption of digital health technologies is inhibited by the lack of a coordinated framework for assessing the value of digital technologies and incorporating such value assessments into reimbursement mechanisms.'[90]

7.69 Edwards Lifesciences argued that:

> Assessment processes need to consider and reliably measure the breath of ways that medical technology can create value beyond the traditional clinical and safety outcomes of a product. This includes but is not limited to a broad array of patient-centric values.[91]

7.70 It encouraged the Government 'to adopt a holistic philosophy that incorporates both cost-effectiveness and the wider considerations at the heart of [value-based healthcare] and HTA.'[92]

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88 MTAA, Submission 148, p. 48.

89 ACvA, Submission 76, p. 10.

90 ACvA, Submission 76, p. 11.

91 Edwards Lifesciences, Submission 83, p. 25.

92 Edwards Lifesciences, Submission 83, p. 33.

7.71 Stryker likewise emphasised that 'evidence-based studies with a focus on clinical outcomes are vital, as is cost-benefit analysis, but this must be enhanced with data on patient outcomes and experiences in order to fully assess the value of the investment.'[93] Medtronic encouraged 'adoption of more value-based considerations in health technology reimbursement, where outcomes that matter to the patient contribute to the value being defined.'[94]

7.72 Roche noted that although consideration of 'the value of societal outcomes' is provided for in the MSAC Guidelines:

> …they do not do so in a quantitative manner - i.e societal outcomes are not included in the cost-effectiveness calculation. It would be valuable for the Government to provide transparency and clarity around how opportunities for more formal inclusion of societal benefits in cost effectiveness calculations can be undertaken. This would assist industry in understanding whether an application may be feasible.[95]

7.73 Johnson & Johnson commented on the MSAC Guidelines that:

> There is less focus or acceptance of societal value (e.g. carers, patient productivity etc.), improvements in patient experience in using product (e.g. compliance, ease of use), other savings to Government (e.g. savings to education, housing or justice), or economic productivity impacts.[96]

7.74 Novartis stated that 'based on the current evaluation framework, it is not possible for…MSAC to consistently consider benefits beyond patient outcomes and health system costs.' Its recommendation to remedy this problem was:

> Expand technical guidelines to incorporate additional attributes such as impact on the lives of carers, productivity, participation in workforce and education and an "innovation factor" potentially adjudicated by an independent agency that evaluates against attributes defining 'real innovation.'[97]

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93 Stryker, Submission 28, p. 5.

94 Medtronic, Submission 122, p. 4.

95 Roche, Submission 92, p. 19.

96 Johnson & Johnson, Submission 134, p. 10.

97 Novartis, Submission 138, p. [12].

7.75 Merck Sharp & Dohme Australia argued that 'the current HTA framework does not fully account for, or appropriately value, the full range of benefits offered by diagnostic technologies, potentially resulting in inequitable access and forgone benefits for the healthcare system.'[98]

Future benefits

7.76 The discussion of the MSAC's approach to valuing future benefits was less technical than that for the PBAC, with no exploration of the issue of discount rates, but the general sense was the same. The RDIWG did argue that:

> …novel technologies are likely to be associated with high upfront costs whereas the benefits may occur over a prolonged period of time. The uncertainty about long-term outcomes will require a sustainable framework for risk-sharing arrangements between manufacturers and the Government.[99]

7.77 Takeda similarly suggested that 'new funding models between manufacturers and reimbursement authorities will be critical to manage the uncertainty over future long-term outcomes.'[100]

7.78 Johnson & Johnson stated that:

> new innovative technologies…require a recognition of the potential curative benefits to patients and subsequently require consideration of the most appropriate model structure to reflect such benefits. This includes identification of appropriate extrapolation assumptions where long-term outcome data may not be available.[101]

7.79 Likewise Novartis encouraged the MSAC and the Department to:

> …work with healthcare professionals, academics and industry to outline an evaluation framework that addresses the limitations associated with one-time innovative therapies with life time benefits that can substantially improve life expectancy and quality of life for patients and carers.[102]

Choice of comparator

98 Merck Sharp & Dohme Australia, Submission 63, Appendix A, p. 2.

99 RDIWG, Submission 51, p. 3.

100 Takeda, Submission 66, p. 4.

101 Johnson & Johnson, Submission 134, p. 11.

102 Novartis, Submission 138, p. [12].

7.80 UCB Australia raised the issue of choice of comparator, commenting that the requirement for the MSAC to use the 'lowest cost comparator' means that it and the sponsor 'are not able to use the most clinically appropriate comparator consistent with the standard of care for patients.'[103]

The Prostheses List Advisory Committee

The scope of the Prostheses List

7.81 A number of submitters had comments to make on the PLAC. The PLAC sets the price of certain implantable devices on the Prostheses List (PL), which private health insurers must cover. Medistar criticised the fact that there is no equivalent to the PL for non-implantable devices as unfair to patients, and gave the example of one of its devices, a handheld nerve stimulator used to treat headaches and migraines.[104] It recommended that the Government 'establish a sustainable funding program for proven, cost effective, non-implanted medical devices.'[105] It noted that, in the case of devices that end up being listed on the PL, 'public patients may have access to a medical device for one or more years before private patients.'[106]

7.82 Medtronic suggested that the PL has successfully fulfilled its role for implantable technology, but likewise argued that 'there needs to be a designated reimbursement pathway for diabetes technology and non-implantable devices.'[107] It commented that:

> Any refinements to the current PL arrangements must encourage innovation that improves patient outcomes and must be pragmatic about evidence. Conversely, changes that slow innovation or create further hurdles, be they financial or time, for regulatory and/or reimbursement and that are not aligned with value for patients jeopardise government goals of effective healthcare delivery and sustainability.[108]

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103 UCB Australia, Submission 74, p. 4.

104 Medistar, Submission 188, pp. 2-3.

105 Medistar, Submission 188, p. 4.

106 Medistar, Submission 188, p. 1.

107 Medtronic, Submission 122, pp. 7, 25.

108 Medtronic, Submission 122, p. 8.

7.83 The MTAA also voiced its support for providing coverage for non-implantable devices.[109]

The functioning of the Prostheses List Advisory Committee

7.84 Edwards Lifesciences noted the delay the PLAC process can cause between public patients receiving access to a device and private patients.[110] It was highly critical of the PLAC, focusing particularly on what it suggested was 'inconsistency' in decision-making. It submitted that:

> We would question the purpose of the PLAC and its Clinical Advisory Groups (CAGs). From our perspective, the PLAC and CAGs operate as a second regulatory process. This duplicative process is already being performed by TGA and MSAC. In our experience, PLAC has been inconsistent, lacks transparency and accountability and constantly moves the goalposts. Reforms are needed, including clear metrics so sponsors know where they stand from the beginning. Further, members of CAGs should be limited to two four-year terms to ensure new input to the CAGs.[111]

7.85 Stryker argued that there is some duplication between the PLAC's role and the TGA's, and claimed that it has had difficulties with 'delays involved in including new technologies on the [PL], particularly when this involves creating a new product group.'[112] It suggested that the PLAC's evidence criteria are too strict, particularly for devices containing 3D-printed components, and that the listing criteria in general have not kept up with advances in medical technology and are in need of updating.[113]

7.86 The MTAA was critical of the PLAC. It submitted that 'PLAC and MSAC processes do not synchronise well, and this can lead to unnecessary delays.'[114] It criticised the PLAC's engagement with sponsors, which it suggested should be increased for 'applications for higher benefits,' where 'a more detailed HTA is almost certain.'[115] It also suggested the PLAC and its CAGs require more expertise in certain areas, such as bioengineering and digital technology, as well as more involvement from patients of the specific

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109 MTAA, Submission 148, p. 55.

110 Edwards Lifesciences, Submission 83, p. 28.

111 Edwards Lifesciences, Submission 83, pp. 33-34.

112 Stryker, Submission 28, p. 5.

113 Stryker, Submission 28, p. 16.

114 MTAA, Submission 148, p. 50.

115 MTAA, Submission 148, p. 52.

conditions under consideration.[116] Finally, it argued that the PLAC process is 'more onerous than is warranted', and in particular duplicates much of the safety work already performed by the TGA.[117]

Current debate on reform of the Prostheses List

7.87 The Committee recognised that tension existed between the medical technology industry and private health insurers.

7.88 The MTAA discussed the Agreement it has between the Government and the MTAA that concludes on 31 January 2022:

> Under the Agreement, medical device companies delivered $1.1 billion in savings to the Prostheses List. The Agreement included recognition of the need for further Prostheses List reform, something that MTAA has willingly engaged in.[118]

7.89 The MTAA commented on proposals being made by private health insurers that:

> …rather than facilitating access to the best technologies will likely dampen their uptake, or result in market failure in the form of out-of-pocket costs to consumers. The proposals include paying for devices through a DRG (activity-based funding) system rather than the Prostheses List. This would abolish the Prostheses List as a consumer protection for patients.[119]

7.90 Private Healthcare Australia (PHA), the insurers' industry body, reinforced its position to the Committee commenting that:

> The medical device funding system in Australia is broken. Australian consumers (through their health insurance premiums) pay too much. The system is so complex that it is prone to mistakes and to manipulation...
>
> Australians pay the highest prices for medical devices in the world. We pay 30-40 per cent more than New Zealand, France, South Africa and the United Kingdom. Some prices are just outrageous, twice and three times more than in other markets.[120]

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116 MTAA, Submission 148, pp. 52-53.

117 MTAA, Submission 148, p. 53.

118 MTAA, Submission 148, p. 54.

119 MTAA, Submission 148, p. 54.

120 Private Healthcare Australia (PHA), Submission 197, p. [1].

7.91 PHA concluded that the costs of doing nothing are huge, as private health insurance becomes less affordable for many Australian families.[121]

7.92 PHA provided information about the blueprint for PL reform. It informed the Committee that with these achievable reforms it would provide:

- Doctors and patients access to a full range of medical devices and there will be no co-payments, and
- Where patients have need for more expensive devices than the average, doctors will be able to access more funding through a simple declaration form.[122]

7.93 Biotronik Australia submitted that as a result of increased pressure for cost savings it:

> …has suffered over a 30 per cent decline in returns for our technologies through significant reductions to the Commonwealth Prostheses List that is impacting on our abilities to maintain our engagement with the Australia market.[123]

Reform announced in the 2021-22 Budget

7.94 As part of the 2021-22 Budget the Government announced that:

> The Australian Government is investing $22 million over 4 years to reduce the cost of medical devices used in the private health sector and streamline access to new medical devices, which will improve the affordability and value of private health insurance for Australians.
>
> This measure will modernise and improve the Prostheses List (PL). This will better align the price set for medical devices on the PL for private providers with those paid for in competitive markets such as those in the public hospital system.

121 PHA, Submission 197, p. [1].

122 PHA, Submission 197, p. [2]; PHA, 'Surgically replacing the list: a roadmap for Prostheses List reform', Sydney, December 2020, www.privatehealthcareaustralia.org.au/wp-content/uploads/Surgically-Replacing-the-List-PHA-Prostheses-Reform-Roadmap.pdf, viewed 15 October 2021.

123 Biotronik Australia, Submission 130, p. [4].

> This will be implemented by the Department of Health in conjunction with the Independent Hospital Pricing Authority, and in consultation with key stakeholders.[124]

7.95 The Department informed the Committee that:

> As part of the suite of reforms as announced in the 2021-22 Budget Measure, Modernising and Improving the Private Health Insurance Prostheses List, it is intended that the purpose and scope of the Prostheses List will be clarified. This may see some technologies become eligible for listing on the Prostheses List that are not currently eligible and, in particular, specific purpose, non-implanted devices. Currently only implanted devices are eligible for listing.[125]

Medical Services Advisory Committee Guidelines Review

7.96 When asked by the Committee about its view of the criticisms of MSAC discussed above, the Department referred to the new Guidelines. It stated that:

> New MSAC Guidelines recently published at: www.msac.gov.au/internet/msac/publishing.nsf/Content/MSAC-Guidelines, which better align MSAC assessment methods with best practice in HTA for therapeutic and investigative technologies, taking account of input from stakeholders.
>
> The new Guidelines are forward-thinking and applicable to the range of technologies and services MSAC will likely consider into the future. The updated MSAC Guidelines provide guidance for newer technologies, including genetic testing for heritable diseases and other screening tests.
>
> These aim to provide applicants with clarity and certainty about the assessment methods, which in turn will mean simpler and more successful applications. The Government is committed to continuing to improve MSAC processes, including in respect of stakeholder input, communication and transparency.

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124 Department of Health, 'Private health insurance – modernising and improving the private health insurance Prostheses List', Canberra, May 2021, www.health.gov.au/sites/default/files/documents/2021/05/private-health-insurance-modernising-and-improving-the-private-health-insurance-prostheses-list.pdf , viewed 15 October 2021.

125 Department of Health, Submission 15.6, p. [21].

…

The expansion of the Health Products Portal, currently used for applications for PBS listing, will provide a single, easy to use place where applicants can apply and track their applications to MSAC and is an opportunity for further process improvements.

In addition, the Department is developing options for improvements to MSAC processes and the potential introduction of cost recovery arrangements to address stakeholder feedback on the need for improved clarity, transparency, and certainty of timeframes.[126]

7.97 The Department has published an explanation of the differences between the old and new Guidelines on the MSAC website. Most relevantly for current purposes this includes:

- There are options to present additional relevant information such as the Inclusion of the 'Value of Knowing' and 'Other Relevant Considerations'
- The revised Guidelines provide guidance for newer technologies, including genetic testing for heritable diseases and other screening tests, incorporating information that used to be in the Clinical Utility Card (CUC) Proforma
- There is an exemplar/facilitated approach for investigative/diagnostic genetic tests[127]

Committee Comment

7.98 In the Committee's view the issues relating to the MSAC were some of the most difficult raised throughout the inquiry. The Committee believes that there are two main difficulties facing the MSAC: first and foremost, the wide variety of different technologies it is required to assess; and secondly, a less transparent and robust process in comparison to the PBAC.

7.99 It is clear to the Committee that the MSAC performs a flexible yet complex role, and it wishes to thank MSAC's members and the staff from the Department of Health (the Department) who support it for their work.

7.100 The Committee notes the Department's evidence that the Australian Government is considering the introduction of a cost recovery model for the

126 Department of Health, Submission 15.6, p. [13].

127 Department of Health, 'Guidelines for preparing assessments for the MSAC', Canberra, May 2021, www.msac.gov.au/internet/msac/publishing.nsf/Content/MSAC-Guidelines, viewed 15 October 2021.

MSAC. While this would clearly be a significant change to current arrangements, the Committee believes that it should be considered if the MSAC requires extra resources to fulfil its role, although only after consultation with the states and territories, industry, patients and clinicians to ensure that it will not interfere with patient access to devices. The Committee recommends the same submission fee waiver scheme for the MSAC, if implemented, as is recommended in this report for the PBAC. That being to include a scheme to include HECS-style fee waivers for Australian start-up companies, orphan drugs and companies with revenue of under $50 million per annum. Submission fees would only be payable for successful submissions once the drug has been listed and earned a specific amount of revenue in the Australian market to promote innovation. In addition, the Committee believes a sliding scale of fees should be considered for resubmissions, with fees being lower for resubmissions.

7.101 The Committee believes that, given the range of devices the MSAC must consider, expertise for assessments must depend in large part on effective consultation with clinicians, although the expertise of the MSAC's members should reflect the applications it is assessing as much as possible. The Committee notes the shortage of health economics expertise in devices, and a shortage of health economics expertise in general, and believes that the Australian Government should take steps to attempt to remedy this. In the Committee's view, the same considerations that were discussed in relation to the PBAC's international cooperation and harmonisation apply to the MSAC, and this should be increased where possible.

7.102 The Committee appreciates that the MSAC is in a difficult position in regard to the consistency of its processes, since it needs to maintain a greater level of flexibility than the PBAC. The Committee considers that there is room for more consistency while maintaining that flexibility, particularly in publishing its calendar and meeting agendas. The Committee notes that both the MSAC and sponsors need to work on improving their understanding of the system and their engagement. Consideration should be given to allowing sponsors more opportunities to present at the MSAC and to an expansion of pre-submission meetings.

7.103 The Committee notes the TGA's evidence that parallel processing of TGA and MSAC applications would be difficult however it suggests further consideration be given to this proposal. The Committee acknowledges that concerns were raised with the MSAC's approach to assessing many diagnostic technologies, however there appears to have been a serious attempt to address many of these concerns in the new MSAC Guidelines.

The Committee would like to see similar action taken to address the concerns that were raised about digital technologies.

7.104 The Committee notes that there were many similarities in the concerns raised with the MSAC's approach to evidence and valuation of devices and those raised in relation to the PBAC, such as the need for more use of Real World Evidence (RWE) and the need to consider non-health benefits in valuation. The Committee accepts that there are important differences between assessment of medicines and devices, and in particular that randomised controlled trials must play a smaller role in the approval of devices. The Committee supports the MSAC giving more weight to evidence beyond traditional clinical trials and considering a broader range of costs and benefits in its valuation process.

7.105 The Committee is unclear if the MSAC will form part of the independent HTA Review and believes it is important to include the MSAC in this review.

7.106 The Committee appreciates that there are a number of difficult issues concerning the PL and PLAC, and welcomes the Australian Government's recognition that reform is needed in its 2021-22 Budget. The details of those reforms were not publicly available[128] but the Committee hopes that they will at minimum expand coverage to include non-implantable devices, and improve the coordination between the MSAC and PLAC to reduce delays in access for patients.

[128] As of September 2021.

8. Rare Diseases

Overview

8.1 Rare diseases are those that are generally defined as affecting less than five in 10, 000 people. The number of rare diseases varies between countries and studies, however it is generally accepted that there are 7,000 different rare diseases in total. While individual diseases may be rare, globally, approximately eight per cent of the population live with a rare disease. This equates to around two million Australians.[1]

8.2 Rare diseases, like many other chronic diseases, are often serious and progressive. They typically display a high level of symptom complexity and are a significant cause of ongoing health and psycho-social challenges. There is no cure for many rare diseases, and so improving quality of life and extending life expectancy of people living with a rare disease relies on appropriate treatment and care.[2]

8.3 As there are limited treatment options, it is essential that people living with a rare disease can benefit from new and transformative health technologies such as genomics, gene and cell therapies and precision medicine. Timely access to these transformative technologies is critical as many rare diseases progress quickly. Financial support for rare disease is another challenge as research and treatments can be very costly.

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1 Department of Health, *National Strategic Action Plan for Rare Diseases,* Canberra, www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases, viewed 13 September 2021.

2 Department of Health, *National Strategic Action Plan for Rare Diseases,* Canberra, www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases, viewed 13 September 2021.

8.4 Rare disease can be difficult for health professionals to gain specialised knowledge of and experience with when seeing low patient numbers in comparison to more common diseases. Researchers face an uphill battle in securing funding and in coordinating statistically robust studies. In addition, pharmaceutical industry interest in rare disease research and development can be low due to the relatively low demand.[3]

8.5 This chapter discusses what the Australian Government is doing to support rare disease and how rare diseases are considered within the current Health Technology Assessments (HTA). The chapter examines the challenges that exist for orphan drugs and antimicrobials in Australia. Other areas discussed include gene and cell therapy, clinical trials, data collections and research for rare disease.

Government initiatives for rare disease

National Strategic Action Plan for Rare Diseases

8.6 The National Strategic Action Plan for Rare Diseases (Action Plan), launched in February 2020, is the first nationally coordinated effort to address rare diseases in Australia. The Action Plan outlines principles and actions to bring about the best possible health and wellbeing outcomes for Australians living with a rare disease. It outlines a comprehensive, collaborative and evidence-based approach built on 3 principles: person-centred, equity of access, and sustainable systems and workforce.[4]

8.7 The Action Plan has three pillars – Awareness and Education, Care and Support, and Research and Data, and aims to increase awareness of rare disease and improve engagement between sectors, enhance jurisdictional partnerships and collect high quality data of rare disease to facilitate research into the future.

8.8 The Action Plan called for the Government to recognise action and policy for rare disease to ensure equity of access to medicines and research/clinical trials for this priority population.

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[3] Department of Health, *National Strategic Action Plan for Rare Diseases*, Canberra, www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases, viewed 13 September 2021.

[4] Department of Health, *National Strategic Action Plan for Rare Diseases*, Canberra, www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases, viewed 13 September 2021.

8.9 Several other priority groups were recognised including 'Aboriginal and Torres Strait Islander people; people living in regional, rural and remote areas; people from culturally and linguistically diverse (CALD) backgrounds; and people experiencing socio-economic disadvantage.'[5]

8.10 The next step will be to implement the actions agreed to under each pillar. Many of the actions relate to the Committee's terms of reference and were suggested as recommendations by many submitters.

8.11 The Rare Disease Industry Working Group (RDIWG) informed the Committee that it welcomed the Australian Government's commitment to provide up to $3.3 million for activities to implement the first National Strategic Action Plan for Rare Diseases announced in February 2020. Importantly, the Action Plan's key priorities include equitable access to the best available health technology.[6]

Compact 2018

8.12 On 8 May 2018, the Department of Health (the Department) and Medicines Australia entered into a Compact to facilitate and promote cooperation between the parties in respect to ensuring the future sustainability of the Life Saving Drugs Program (LSDP). The agreement is underpinned by the shared principles of:

- Stewardship of the Australian health system and a responsibility for its ongoing sustainability
- Patient access to clinically effective medicines for chronic progressive rare diseases
- Improved value of medicines available on the LSDP that enable ongoing sustainability of the program
- Stability and certainty for the investment in medicines for rare diseases, including recognition of the role that transparent and streamlined processes play in encouraging investment
- Transparency and efficiency of processes for listing medicines on the LSDP and for subsequent reviews of medicines.[7]

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5 Department of Health, *National Strategic Action Plan for Rare Diseases*, Canberra, p. 8, www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases, viewed 2 August 2021.

6 Rare Disease Industry Working Group (RDIWG), Submission 51, p.1.

7 Department of Health, Submission 15, p. 6.

8.13 The Department in collaboration with the RDIWG and Rare Voices Australia (RVA) developed guidance to ensure transparency and associated timelines for consideration of medicines seeking funding through the LSDP. This guidance further delivers on the commitment to assist sponsors in preparing an application to make a rare disease medicine available on the LSDP; ensuring access to treatment for people with rare diseases is not unnecessarily delayed.[8]

National Health Reform Agreement

8.14 The National Health Reform Agreement (NHRA) is an agreement between the Australian Government and all state and territory governments and was signed in May 2020.

8.15 It commits to improving health outcomes for Australians, by providing better coordinated and joined up care in the community, and ensuring the future sustainability of Australia's health system. It is the key mechanism for the transparency, governance and financing of Australia's public hospital system.[9]

8.16 The 2020-25 NHRA Addendum provides specific arrangements to ensure Australians with some of the rarest conditions have access to new, life-saving highly-specialised therapies in public hospitals. These funding arrangements (50 per cent Australian Government, 50 per cent state and territory governments) apply to high cost therapies recommended by the Medical Services Advisory Committee (MSAC) to be used in Australia and delivered in a public hospital. State and territory governments, as system managers of public hospitals, will determine if, when and where these treatments are delivered. All governments have agreed to greater transparency and improved consultation processes so all jurisdictions can be engaged and informed in technology assessment processes.[10]

Funding

8 Department of Health, Submission 15, p. 6.

9 Department of Health, *National Health Reform Agreement*, Canberra, www.health.gov.au/initiatives-and-programs/2020-25-national-health-reform-agreement-nhra viewed 13 September.

10 Department of Health, *National Health Reform Agreement*, Canberra, www.health.gov.au/initiatives-and-programs/2020-25-national-health-reform-agreement-nhra viewed 13 September.

8.17 In 2020-21, the Department announced there will be two grant opportunities under the Clinical Trials Activity Initiative including $25 million for 'Rare Cancers, Rare Diseases and Unmet Need for COVID-19' and $25 million for 'Rare Cancers, Rare Diseases and Unmet Need'.[11]

Health Technology Assessment alternative pathways

8.18 Australian HTA processes utilise models that are designed primarily for more common diseases. This presents challenges for reimbursement decisions for rare disease medicines/technologies. Smaller patient numbers impact cost effectiveness, and there is often less clinical evidence available due to the challenges of conducting large-scale clinical trials.

8.19 Mrs Nicole Millis, CEO, RVA, highlighted innovation as being critical for advancement of rare disease treatment however the HTA processes need to be fit for purpose to allow the innovative health technologies to be provided to the patients who need them:

> For example, fundamental discovery research is of central importance to the development and testing of health technology innovation. Repurposing of medicines also provides an important opportunity to address unmet need in rare disease, but reimbursement of repurposed medicines is inequitable, uncertain and unsustainable. This is a systemic issue for rare disease, where the patient numbers are so small and where current approval processes are inappropriate and inflexible.[12]

8.20 Fabry Australia commented:

> Incentivising big global pharmaceutical companies to bring international research to Australia is imperative. There is uncertainty about how the Australian regulatory system works and the reimbursement model is unclear, and complex compared to other global models. The pathways need to be clearer and for all stakeholders, particularly those with financial investment in new novel medical technologies to ensure businesses are confident to come to the Australian market.[13]

8.21 Specialised Therapeutics Australia (STA) stated that whilst it was understood that the Australian health budget is a finite resource:

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11 Department of Health, Submission 15, p. 20.

12 *Committee Hansard*, Canberra, 11 March 2021, p. 2.

13 Fabry Australia, Submission 4, p. 2.

> … the processes for determining access to life-saving and life-changing technologies need to be faster and more transparent, with fewer administrative and financial barriers.[14]

8.22 RDIWG commented that the reimbursement process for rare diseases is unnecessarily lengthy meaning that Australian patients have to wait for treatments for very prolonged periods of time:

> Consideration should be given to ongoing negotiation rather than rejection, particularly with regard to price and population, after the first evaluation thus reducing submission churn.[15]

Health Technology Assessment Consumer Evidence and Engagement Unit

8.23 In 2019, a designated HTA Consumer Evidence and Engagement Unit was established within the Department's Technology Assessment and Access Division to allow the development of structured projects of engagement with consumer and patient groups.

8.24 The Department provided an example of where further engagement and assistance provided the support required to have a drug listed and reimbursed on the Pharmaceutical Benefits Scheme (PBS) successfully.

> The Department has actively supported rare disease organisations to engage in the submission processes, including those put forward by pharmaceutical companies, or in collaboration with clinical specialists. This has been demonstrated through the successful listing of vorinostat (Zolinza®) for relapsed or refractory cutaneous T-cell Lymphoma (CTCL) on the PBS as a result of submission by Rare Cancers Australia in 2016.[16]

8.25 Despite the Department establishing this new engagement unit within the Department, it was clear that the stakeholders were either unaware that the unit existed or they felt that the unit needed to be expanded to provide further assistance.

8.26 Patient Voice Initiative commented about the lack of awareness the patient voice has in knowing where they can contribute within the HTA process:

> Patients have an opportunity to provide input during reimbursement processes, but most are not aware of this or cannot access sufficient detail about what PBAC or MSAC are considering in order to address the

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14 Specialised Therapeutics Australia (STA), Submission 7, p. 5.

15 RDIWG, Submission 51, p. 7.

16 Department of Health, Submission 15, p. 39.

> knowledge gaps. Often it is too little, too late because patient knowledge was not part of the R&D [research and development] process informing everything from prioritisation to trial design and aiding recruitment (all identified benefits of patient involvement).[17]

8.27 Ms Jane Hill, Chief Executive, Ovarian Cancer Australia, emphasised that there needs to be further reform in patient engagement.

> There have been some advances made in the last couple of years, but I still feel that there is a lot to be done in that area. There are some innovative models in Canada and Scotland. There are some good things happening there; some principles, I think, that are worth considering—patient organisations being given prior access to sponsor submissions and having much more engagement throughout the process. I also think all stakeholders should be brought together to discuss reimbursement and approvals.[18]

8.28 Ms Jessica Bean, Chair, The Patient Voice, said 'I think we've seen small but positive steps in terms of consumer engagement with the formation of the consumer evidence and engagement unit. Recognition that there's need for better consumer engagement is really important. I think it has to be embedded across the life cycle.'[19]

8.29 Ms Vanessa Xavier, Head, Market Access, Sanofi, expressed the view that the current engagement that patients and clinicians receive during the Pharmaceutical Benefits Advisory Committee (PBAC) process is not meaningful engagement.

> Right now, I would say, for the PBS process and the LSDP processes, it's not meaningful engagement. It's not a dialogue. With the call for comments, you can write a very short statement about why you believe the product should be reimbursed, but nobody speaks to you about why it's important to you or what benefits you look for either as a physician or as a patient. So I think we need to do better with regards to engagement.[20]

8.30 The RDIWG commented that the patient voice should be incorporated as part of all pathways:

> Consumer hearings should be held for all rare disease treatments in order that the patient voice is heard in particular with regard to the effect of the

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17 Patient Voice Initiative, Submission 71, p. 2.

18 *Committee Hansard*, Melbourne, 23 April 2021, p. 40.

19 *Committee Hansard*, Brisbane, 17 May 2021, p. 9.

20 *Committee Hansard*, Melbourne, 23 April 2021, p. 18.

> condition on the life of patients and their families and the impact that a new treatment will have to these patients. In accordance with Action 2.1.5 of the National Strategic Action Plan for Rare Diseases, the voice of people living with a rare disease as well as families and carers should be embedded throughout structures and systems that impact rare diseases. Rare disease organisations should work with the HTA Consumer Evidence and Engagement Unit to take a more active role in HTA processes.[21]

Developing rare disease expertise in the Department of Health

8.31 Several submitters suggested that rare disease expertise should be developed within the Office of HTA (Action 2.4.1.4 of the Action Plan) and the evaluation template should include content and explanations that focus on the differences as a consequence of rarity.

8.32 Associate Professor Michelle Farrah, Clinician representative, Luminesce Alliance was supportive of establishing an Australian Office of Rare Disease.

> First of all, I am aware and extremely thankful that the Australian government has endorsed the National Strategic Action Plan for Rare Diseases, and priority 1 and pillar 1 is entirely focused on awareness and education for rare diseases. In terms of extending that vision, an Australian office for rare diseases would be very important. Overseas there's the EURORDIS office, and I think collaborating with them and adopting and translating that to the Australian context would be very important—running summer schools for all stakeholders, educating them on each other's perspective and the framework that's needed to access therapies in Australia. But also the office could have oversight and accountability and coordinate leadership and engagement to promote awareness and health literacy and really focus on access and equity within this office, and develop the infrastructure, the tools and the resources to promote awareness and education and therefore literacy so that we can promote equitable access for all people living with rare diseases, to optimise their therapies.[22]

8.33 RVA suggested:

> The establishment of a Rare Disease and Precision Health Office in Government, acknowledging the importance and future promise of precision health in driving person-centred healthcare. A number of similar exemplars already exist, both nationally and overseas. Locally, the Precision Health Council in Western Australia (a ministerial council) was established in 2019. Internationally, in the United States, the Rare Diseases Office within the

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21 RDIWG, Submission 51, p. 7.

22 *Committee Hansard*, Sydney, 7 May 2021, p. 36.

> National Institute of Health has statutory authority and was established through the Rare Diseases Act (2002).[23]

Cost

8.34 Many pharmaceutical companies raised the issue of the cost of submitting a successful submission to the HTA process. The Committee heard that it cost the pharmaceutical company approximately $240 000 to prepare a reimbursement submission to PBAC. This is often on top of about $120 000 to have the submission put together by a consultant/contractor, therefore making the approximate cost for one submission $360 000. However as many submissions do not get through on first application, the real costs of a successful submission sits between $500 000 to $1 million dollars.[24]

8.35 A concern around the high costs of submissions for rare disease was highlighted. Recordati Rare Diseases Australia (RRDA) commented that 'we struggle to afford any subsequent submissions with the PBAC if our first submission is rejected.'[25]

8.36 RRDA continued:

> Rejection of submissions is very common, very few get though in the first round. Our products treat only a very small number of patients, usually children. RRDA sales revenue is only a fraction of other pharmaceutical companies. Second round evaluation of submissions can cost over $300,000.00. This fee does not include the cost of market access consultants preparing submissions which adds another $150 000.00 to the overall cost.[26]

8.37 RDIWG echoed this view:

> Rare disease submissions are often complex and require additional data analysis and stakeholder engagement. They are rarely recommended following the first submission. The process can take years with multiple resubmissions to PBAC and can be cost-prohibitive even with the Orphan Drug Designation (ODD) fee waiver for the first submission, with subsequent resubmissions costing $166,220 from 1st January 2021. Therefore, the timeframes for claiming exemption of cost recovery fees should be extended

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23 RVA, Submission 86, p. 5.

24 Ms Leah Goodman, Managing Director, Merck Healthcare, Sydney, 12 March 2021, p. 1; p. 11.

25 Recordati Rare Diseases Australia (RRDA), Submission 3, p. 1.

26 RRDA, Submission 3, p. 2.

> for orphan drugs. These changes will provide additional incentives to bring orphan drugs to Australian patients.[27]

8.38 Several pharmaceutical companies that supply rare medicines suggested that a waiver of fees should be extended beyond the current 12 month timeframe.[28]

8.39 The Committee heard that a waiver of fees would encourage pharmaceuticals to submit more medicines to PBAC in the future.

8.40 RRDA commented 'I believe that we would be able to submit more medicines to the PBAC if the fees for the first 2 - 3 submissions were also waived.[29]

8.41 In addition, STA suggested that:

> Smaller companies with revenue less than $50 million annually be granted an exemption from paying new fees 'upfront' for at least the first two applications, and when, or if, a drug is listed on the PBS, the company then pays those fees in arrears, in instalments when Pharmaceutical Benefits Scheme (PBS) expense on that drug exceeds $3 million per year.[30]

8.42 Some submitters commented on the TGA's fee regime, particularly as it relates to therapies for rare diseases. Dr Falk Pharma commented that an orphan drug designation for a medicine:

> … should cover all submissions relating to that medicine with the same active ingredient for the treatment of the same condition.[31]

8.43 Similarly, Amicus Therapeutics submitted that the fee waiver that results from an orphan drug designation should be extended to five years (from one year currently) to 'encourage and support companies to continue investigating and following up with expanded populations such as paediatric indications for a therapy.'[32]

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27 RDIWG, Submission 51, p. 5.

28 STA, Submission 7, p. 6; RRDA, Submission 3, p. 2.

29 RRDA, Submission 3, p. 2.

30 STA, Submission 7, p. 6.

31 Dr Falk Pharma Australia, Submission 17, p. [3].

32 Amicus Therapeutics, Submission 31, p. 4.

8.44 Mr Rod Longmire recommended 'reducing registration and application fees for small enterprises or organisations with small budgets' to encourage registration of medicines for small populations.[33]

8.45 RESULTS International Australia similarly recommended that fees be waived for 'essential drugs' with small markets like drugs for tuberculosis.[34]

8.46 Commenting on the prospect of fees being raised to increase the TGA's budget, Dr Arnold and Dr Bonython said that 'there are no indications that overseas/domestic vendors will leave the market on the basis of moderate increases in fees.'[35]

8.47 Professor Skerritt responded to the discussions of expanding fee waivers by saying:

> …with our full cost recovery model, it is always a challenge if, let's say, there's a number of types of drugs that were able to receive a fee waiver. There's not a magic pudding, so that funding would have to come from somewhere, and that would be a decision by government on how to fund it.[36]

8.48 In response to the idea of charging different fees based on company size he explained:

> The fees are based on effort, and you can imagine, whether it's a new start-up company with a new drug or whether it's a multinational, the amount of effort is the same. There is no differential fee schedule for small companies versus big companies. That would be a question of policy for government.[37]

Managed Access Program

8.49 The Managed Access Program (MAP) (formerly the Managed Entry Scheme (MES)) Framework was developed in consultation with representatives of applicants for PBS listing. It came into effect in 2011 as part of the response to the trend for applications for new medicines in rare diseases, which were based on relatively preliminary evidence. The Department is undertaking further consultation with the pharmaceutical

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33 Mr Rod Longmire, Submission 113, p. 5.

34 RESULTS International Australia, Submission 106, p. 2.

35 Dr Bruce Baer and Dr Wendy Bonython, Submission 49, p. 8.

36 *Committee Hansard*, Canberra, 18 June 2021, p. 15.

37 *Committee Hansard*, Canberra, 18 June 2021, p. 22.

industry in the context of the Strategic Agreement with Medicines Australia on this program.

8.50 The MAP enables the PBS listing of products, under special circumstances of high unmet clinical need, on terms that allow for the resolution of otherwise unacceptable clinical or economic uncertainty for the PBAC. A submission that would not normally be recommended for listing by the PBAC because of unacceptable clinical and/or economic uncertainty could be recommended under a MAP.[38]

The MAP mechanism means:

- earlier access to the medicines by patients;
- earlier access to a subsidised market for the sponsor whilst acknowledging that some form of confidential discount may be required in recognition that the initial evidence is less convincing;
- clear articulation of the evidence required to resolve the identified area of uncertainty and the consequences of potential outcomes from the additional evidence;
- agreement by the PBAC to review a submission once the additional evidence becomes available and to reconsider the listing in light of the new evidence; and
- appropriate sharing of risk.[39]

8.51 The RDIWG was supportive of expanding the MAP. It commented:

> Managed access programs and other outcomes-based arrangements, particularly in the case of small populations and uncertainty of the data, would provide early access to patients while collecting data to reduce uncertainty over time.[40]

8.52 Novartis Pharmaceuticals was not supportive of the MAP as they commented that significant risk was borne by the sponsor and the uncertainty around the PBAC process acted as an disincentive for industry:

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38 Department of Health, Submission 15, p. 29.

39 Department of Health, Submission 15, p. 29.

40 RDIWG, Submission 51, p. 7

> Managed Access Programs, designed to accelerate access to PBS medicines, (previously known as Managed Entry Schemes) have led to significant risk born by the Sponsor and low uptake across the industry.[41]

8.53 Novartis Pharmaceuticals suggested:

> Remove disincentives for sponsors from considering applications that may qualify for Managed Access Program. The rigidity and/or uncertainty surrounding the PBAC process can act as disincentive to seeking faster TGA approval through expedited pathways.[42]

Orphan drugs

8.54 The orphan drug program aims to incentivise sponsors to bring medicines for serious and rare conditions to market that would otherwise not be financially viable. The program offers a 100 per cent waiver of TGA fees for application and registration to help offset orphan drug development costs. Similar arrangements operate with respect to reimbursement applications.[43]

8.55 In July 2017, changes to the orphan drug program were implemented to create a fairer program that aligns more closely with international criteria without impeding the availability of drugs for rare diseases. In particular the new program provides a more generous orphan disease prevalence threshold (fewer than five in 10,000 individuals in Australia), potentially allowing a larger number of medicines to classify as orphan. However, following approval, the decision to supply the product remains at the discretion of the sponsor.[44]

8.56 The eligibility criteria for orphan determination focus on the greatest unmet clinical need and include:

- the medicine is for the treatment, prevention or diagnosis of a life-threatening or seriously debilitating condition
- the condition affects fewer than five in 10,000 individuals in Australia
- it is not likely to be financially viable for the sponsor to market the medicine in Australia, and

41 Novartis Pharmaceuticals, Submission 138, p. [4].

42 Novartis Pharmaceuticals, Submission 138, p. [4].

43 Department of Health, Submission 15, p. 15.

44 Department of Health, Submission 15, p. 15.

- There are no other medicines to treat the condition marketed in Australia; or the medicine provides a significant benefit in relation to efficacy or safety of the treatment, prevention or diagnosis of the condition, or a major contribution to patient care, compared to existing marketed products.[45]

8.57 The Australasian Sleep Association noted that under the current orphan drug criteria a medicine is ineligible if the prevalence in Australia of the disease it treats is unknown, as is the case for narcolepsy. It recommended that in such cases the sponsor be allowed to use a 'reasonable extrapolation of prevalence from comparable countries.'[46]

8.58 Viiv Healthcare raised the issue of the TGA needing an alternative pathway for certain drugs that do not neatly fit the orphan drug pathway however there is no alternate pathway for some indications, for example, paediatric medicine for HIV. Viiv Healthcare raised with the Committee the scenario of paediatric medicine for HIV:

> ViiV Healthcare previously applied to the TGA seeking orphan designation for a paediatric indication of an HIV medicine. This application was rejected by the Therapeutic Goods Administration (TGA), despite the fact that there are estimated to be only 47 children currently living with HIV in Australia.[47]

8.59 Amicus Therapeutics suggested that an orphan drug designation should provide automatic entry to the Priority Review pathway, in order to reduce assessment times for orphan medicines.[48]

8.60 Merck Healthcare suggested that 'the disincentive of the 5 per cent statutory price cut at 5 years of PBS listing, for orphan-designated medicines or medicines which treat small patient populations'.[49]

8.61 The GUARD Collaborative informed the Committee that the United States (US) had introduced legislation for orphan drugs back in 1982.This meant that there were a number of orphan drug approvals nearly 40 years ago and had set the industry up well to research and commercialise orphan medicines:

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45 Department of Health, Submission 15, p. 15.

46 Australasian Sleep Association, Submission 16, pages 3-4.

47 Viiv Healthcare, Submission 80, p. 9.

48 Submission 31, p. 1.

49 Merck Healthcare, Submission 34, p. 1.

> Historically, it is the United States that has taken the first major political steps to accelerate the development of rare diseases therapies, as early as 1982, with the U.S. Orphan Drug Act that led to a sharp increase in the number of approved medicines. The U.S. Orphan Drug Act – unlike more recent EU Orphan Drug Regulation, did not include any provision related to the "significant benefit" of new orphan medicines.
>
> This makes the USA today more attractive to investors and companies than other markets, and as companies and funders are seeking to secure higher returns on their investments, with the concrete outcome of seeing many more generations of orphan medicines come to commercialisation first in the United States and then, only much later, elsewhere.[50]

Life Saving Drugs Program

8.62 Most medicines in Australia are subsidised through listing on the Pharmaceutical Benefits Scheme (PBS). Funding for medicines on the Life Saving Drugs Program (LSDP) is separate to the PBS. The LSDP covers medicines if:

- they are clinically effective, but not cost effective enough to list on the PBS
- they treat life threatening and rare conditions (defined as 1 case per 50,000 people or fewer in the Australian population)
- the pharmaceutical company (sponsor) applies for an LSDP listing.[51]

8.63 The Life Saving Drug Program (LSDP) provides approximately 430 patients fully subsidised access to expensive and life-saving medicines for rare and life-threatening medical conditions. Medicines on the LSDP are available to eligible patients at no cost and for as long as clinically necessary. This program cost $133.6 million in 2018-19. There are currently sixteen medicines on the LSDP for the treatment of 10 conditions.

8.64 The Committee was informed by the Department that 'Before a medicine is considered for inclusion on the Life Saving Drugs Program (LSDP), a medicine must first be considered by the Pharmaceutical Benefits Advisory Committee (PBAC) and accepted as clinically effective but rejected for

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50 GUARD, Submission 46, p. 7.

51 Department of Health, *Life Saving Drugs Program*, Canberra, www.health.gov.au/initiatives-and-programs/life-saving-drugs-program/about-the-lsdp, viewed 21 September 2021.

Pharmaceutical Benefits Scheme (PBS) listing because it fails to meet the required cost-effectiveness criteria.'[52]

8.65 RDIWG said the 'Australian Government should be commended for providing subsidised access to essential medicines to over 430 eligible patients with rare and life-threatening diseases through the Life-Saving Drugs Program (LSDP).'[53]

8.66 Numerous submitters raised concerns about the length of time it takes for medicines to be approved and listed on the LSDP. Some submitters suggested that the two step process should be abandoned. The RDIWG suggested the following:

> In a similar way that the Australian Technical Advisory Group on Immunisation (ATAGI) considers vaccines before the PBAC, consideration of products destined for the LSDP by the LSDP expert panel prior to the PBAC would simplify the process. The two-step LSDP process is unnecessarily lengthy particularly with regard to the need to be rejected by the PBAC prior to consideration by the LSDP Expert Panel.
>
> In the case of products to be listed on the LSDP, the inclusion of a LSDP representative as part of the multi- stakeholder review panel would ensure the specific challenges of the disease are well understood and provide clarity on the likely funding pathway prior to PBAC submission.[54]

8.67 Alexion Australasia commented that 'There has been good progress made over the last 10 years, however to achieve world-leading access for rare disease treatment, further streamlining of the assessment process is required as well as greater certainty that products which clearly fit the LSDP criteria will be made accessible to the public.'[55]

8.68 Alexion Australasia highlighted the issue of time delays and stated:

> Extensive delays to access can occur for these types of products even though they may address a high unmet clinical need.[56]

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52 Department of Health, Submission 15.5, p. [18].

53 RDIWG, Submission 51, p. 6.

54 RDIWG, Submission 51, p. 6.

55 Alexion Australasia, Submission 30, p. 1.

56 Alexion Australasia, Submission 30, p. 1.

8.69 Fabry Australia highlighted the fact that less than one third of all Fabry patients receive the medical treatment listed on the LSDP.

> There are three funded therapies approved on the Life Saving Drugs Program, two of which were listed in 2004. However, the criteria to obtain such medical treatment is very restrictive and not all of the 300+ Fabry patients are on therapy, actually less than a third receive formal Fabry disease medical treatment. The current guidelines do not allow children and young adults to access any treatment until the disease has progressed significantly. In the meantime, there is considerable individual suffering. Current guidelines do not consider the most recent clinical knowledge, such as our improved understanding of the use of cardiac MRI scanning in Fabry disease.[57]

8.70 At a public hearing in June 2021, Professor Andrew Wilson, Chair of the PBAC told the Committee the PBAC is aware that the concept of rare disease is changing, with increasing genomic sub-characterisation of more complex diseases. Professor Wilson commented:

> This has the potential to further challenge the whole concept of a separate program for life-saving drugs for rare diseases. The PBAC believes there is merit in examining whether the same purposes of the LSDP could be achieved through a PBS section 100 program, with specific criteria, as with other section 100 programs. This removes the need for a second-line assessment and would also provide greater consistency in approval, pricing and ongoing monitoring.[58]

8.71 The RDIWG encouraged the Government to consider alternatives for rare disease pathways. It suggested the following:

- Other rare disease treatments that do not meet the LSDP eligibility criteria may be able to be funded on the Pharmaceutical Benefits Scheme (PBS). However, special consideration needs to be given to rare diseases being evaluated by the PBAC. Firstly, greater input from expert clinicians and relevant patient organisations would help the PBAC to understand the disease impact, patient relevance and clinical meaningfulness of the (sometimes limited) data presented, and the seriousness of the unmet need.
- Secondly, cost-effectiveness needs to be considered in the context of rare disease treatments which often cannot meet the incremental cost effectiveness ratio (ICER) thresholds typically applied to treatments for

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57 Fabry Australia, Submission 4, p. 1.

58 *Committee Hansard*, Canberra, 24 June 2021, p. 3.

> more common conditions. Alternative measures of value should be considered, including societal perspectives, impact on patients, carers and the community, and indirect economic costs. In the case of orphan drugs, the overall budgetary impact should be given more weight than the ICER.[59]

8.72 The RDIWG suggested 'earlier engagement with the Department of Health would be welcomed by industry in order to be able to identify the appropriate reimbursement pathway, provide the patient voice and establish clinical need so that all parties facilitate the path to access without increasing submission churn.'[60]

Newborn screening

8.73 In December 2017, the Australian Health Ministers endorsed the Newborn Bloodspot Screening (NBS) National Policy Framework. Healthcare providers offer newborn bloodspot screening to all babies in Australia. This blood test detects certain rare genetic conditions and metabolic disorders. This screening test aims to improve the health of babies by identifying those at risk of developing a serious condition, allowing for early intervention.[61]

8.74 The Department supports the national newborn screening framework by conducting initial reviews on new nominations for NBS. Nominations with sufficient evidence proceed to a detailed review with the MSAC. Following receipt of the MSAC advice, state and territory governments deliver the newborn screening programs. Each state and territory decides which conditions to screen for. [62]

8.75 Several witnesses discussed the NBS and advocated for a more robust national NBS program that was consistent across Australia.

8.76 Ms Louise Healy, Member, Queensland Genomics Community Advisory Group, made the following observation in favour of an aligned national newborn screening program:

> Australia should definitely have a national newborn screening program. We do have a national newborn screening framework, and it's a strong

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59 RDIWG, Submission 51, pages 4-5.

60 RDIWG, Submission 51, p. 5.

61 Department of Health, *Health–topics*, Canberra, www.health.gov.au/health-topics/pregnancy-birth-and-baby/newborn-bloodspot-screening#program-delivery, viewed 20 September 2021.

62 Department of Health, *Health–topics*, Canberra, www.health.gov.au/health-topics/pregnancy-birth-and-baby/newborn-bloodspot-screening#program-delivery, viewed 20 September 2021.

> framework. But the way it's currently governed and administered does lead to inequities across states, with some states implementing recommendations arising from that policy quicker than other states and therefore babies missing out on that opportunity. I think there's some opportunity for leadership in newborn screening as well. We can look at the horizon and see what's coming and start to think about what a newborn screening program should look like … [63]

8.77 Better Access Australia fully supported adding more conditions to the newborn screening program and questioned why Australia was no longer a world leader in newborn screening:

> Why do we consider it acceptable for small patient groups to wait three years for access to a process for newborn screening for fatal but treatable diseases only to be told no. Australia once led the world in this testing but hasn't added a new disease to these tests since 1981.[64]

8.78 Many advocates for specific rare diseases were keen to see more conditions added to the newborn screening program. Mr Raymond Saich, President of Australian Pompe Association commented:

> Newborn screening is absolutely vital if we're going to stop these tragedies of premature death of Pompe babies. In Taiwan they've had newborn screening since 2005. They're able to diagnose a Pompe baby within five days of birth and get babies into treatment. They can see a difference if a Pompe baby has gone into treatment within five days of birth against 21 days of birth. So it's absolutely vital. Unfortunately here in Australia it takes about three months if you live in the metropolitan areas and six months if you live outside of those areas. First mum and dad have got to realise the baby's not well and get the baby to see a GP—often that's two or three visits. Then you get to see a paediatrician and then eventually a treating specialist. That's how the three months happen so quickly. As I said, if you are outside of metropolitan areas these things get so much harder and it doubles it.[65]

8.79 Dr Gaynor Heading, President of Alpha-1 Organisation Australia supports adding more diseases to the newborn screening:

> But there are other things that can be done to assist, like newborn screening for alpha-1. We have babies born with so much antitrypsin trapped in their

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63 *Committee Hansard,* Brisbane, 18 May 2021, pages 18-19.

64 Better Access Australia, Submission 160, p. 5.

65 *Committee Hansard*, Sydney, 11 March 2021, p. 34.

> livers that they go to immediate liver transplant. The genetic trials are brilliant not only for lung but for liver, so we can save all those sick babies as well.[66]

8.80 Dr Melanie Wong, Co-Chair, ASCIA, discussed the importance of a national newborn screening program for Australia. She illustrated her example using a rare disorder called severe combined immunodeficiency (SCID):

> … there are good, reliable newborn screening tests and there are readily available confirmatory tests. SCID newborn screening is currently being piloted in all infants born in New South Wales, funded by a research initiative, so we know that it can feasibly be incorporated into routine practice.
>
> SCID newborn screening is currently under evaluation by the newborn national framework, but this review has, unfortunately, been in progress for almost two years without a formal decision being made. Meanwhile, SCID newborn screening has been successfully introduced in many countries around the world, including the US and New Zealand, so Australia is actually falling behind best practice.
>
> Even if there is a supportive decision following this review process, every individual state will need to accept the decision and allocate funding to add SCID to their current testing regimes. So now we need to progress the newborn framework evaluation. We need to achieve support and funding for incorporation of SCID newborn screening into each state's program and thus provide a united national screening approach to preventing life-threatening infections and early death in the vulnerable group of children affected by this genetic condition.[67]

Cell and gene therapy

8.81 Cell and gene therapies are fundamentally different from more common medicinal products, as they generally have longer than average development times, more stringent manufacturing requirements, and a limited shelf life for products (sometimes as little as 24 hours).[68]

8.82 One third of cell and gene therapies are in development for rare diseases. Medicines Australia illustrated the way in which cell and gene therapy will expand in the future:

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66 *Committee Hansard*, Sydney, 11 March 2021, p. 35.

67 *Committee Hansard*, Sydney, 7 May 2021, p. 37.

68 Medicines Australia, Submission 141, p. 18.

> As of February 2020, there are nine cell or gene therapy products approved in the U.S. treating cancer, eye diseases and rare hereditary diseases. The FDA has indicated that it expects to approve 10 to 20 new cell and gene therapies between now and 2025.[69]

8.83 Takeda Pharmaceutical commented that the supply and administration of personalised cell and gene therapies is more complex and different to conventional drug products. It suggested that the Government may need to provide financial assistance to bring these new therapies to patients:

> Many institutions may not have the required capabilities to provide these therapies. Instead, it is expected that patients will be referred to a relatively small number of treatment centres where they will be carefully selected for treatment and managed by a multi-disciplinary team with a follow up period that could span over many years. These considerations may require the treatment centre to make additional investments and require financial support by the government.[70]

8.84 Roche discussed the future of genetic testing and treatment access in Australia. It suggested:

> … the establishment of a national genomics service which, through a range of public and private partnerships, can better link research and clinical care.
>
> The service would support patients and service providers by ensuring genomic testing and subsequent clinical services, including genetic counselling, are provided within a quality framework. It would also help build workforce and research capacity and capability.
>
> The data generated through the research component of the service would be used as evidence to support applications to repurpose existing medicines which can ultimately broaden the number of available treatments listed on the Pharmaceutical Benefits Scheme (PBS) for patients with rare diseases or cancer.[71]

8.85 RDIWG encouraged the Committee to consider recommending a fit-for-purpose system for evolving therapies such as gene and cell therapies which have attributes that require special consideration:

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69 Medicines Australia, Submission 141, p. 18.

70 Takeda Pharmaceuticals, Submission 66, p. 4.

71 Roche, Submission 92, p. 6.

> Development of such a system will mean that Australians can remain proud of the system which enables sustainable access to interventions irrespective of their health challenge.[72]

8.86 In addition RDIWG raised the issue of dealing with limited data at the time of assessment. It suggested:

> … there should be a focus on the development of innovative access mechanisms to ensure patients have the advantage of being able to access treatment in parallel to the long-term collection of real-world evidence (RWE).[73]

8.87 Novartis Pharmaceuticals commented that the current co-dependent pathways for access to genomic screening for cancer patients leads to delays. It suggested:

> A staged introduction of de-coupling of the cost effectiveness of the test and therapy where genomic panel testing is available is likely to improve patient access to targeted therapies for patients with rare diseases, build experience nationally with genomic testing.[74]

8.88 The Sydney Children's Hospital Network supported the streamlining of approval processes for cell and gene technology. It suggested decreasing the time it took to obtain a licence from the Office of the Gene Technology Regulator as this was seen as a barrier to bringing clinical trials to Australia.

> Streamlining gene technology licencing processes for investigators and industry. For many advanced therapeutics, clinical trial readiness relies on having adequate facilities (PC2 lab) and navigating complex regulatory processes. Some clinical trials of gene and cell therapies require a licence from the Office of the Gene Technology Regulator under the Gene Technology Act 2000, and most licences require sponsor accreditation. This process can take in excess of 4 months which is currently a deterrent for Sponsors to bring gene therapy clinical trials to Australia.[75]

8.89 In addition, the Sydney Children's Hospital Network proposed more international collaboration:

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72 RDIWG, Submission 51, p. 2.

73 RDIWG, Submission 51, p. 3.

74 Novartis Pharmaceuticals, Submission 138, p. 2.

75 Sydney Children's Hospital Network, Submission 185, p. 14.

> Enhanced national and international collaborative relationships to more efficiently and effectively identify areas of unmet need, guide the development of policy and regulation, and facilitate timely decision making in collaboration with industry.[76]

8.90 GUARD commented that more work is needed to be done by Governments and the integration along the development pipeline.

> Federal and State Governments have recognised the promise of genomic medicine and are progressing substantial programs of work to promote the integration of genomic technologies into healthcare. This is not enough. Innovation in approach to discovery, research, development and manufacture of new drugs, treatments and technologies must support this integration.[77]

Clinical trials for rare cancers

8.91 Victorian Comprehensive Cancer Centre stated that 'rare cancers disproportionately affect young patients, are associated with poor survival outcomes, and are under-represented in clinical research.'[78]

8.92 Clinical trials are essential to improving outcomes in all patients but rare cancers are frequently excluded from participation in clinical trials because:

- Clinical trials are often designed with an ultimate aim of registering a drug for a specific indication. Rare cancers may therefore be seen to be less desirable for inclusion as they represent a smaller number of patients.
- Randomised phase 3 trials for rare cancers are not feasible.
- For rare cancers, particularly ones that are treatment refractory, there may be no established standard of care.[79]

8.93 A number of new tactics to include rare cancers in clinical trials are being pursued. These include:

> Tumours can now be defined by specific molecular features (as opposed to the organ of origin), allowing enrolment onto Precision Oncology clinical trials based on the molecular features alone, aka. 'tumour agnostic' trial design.

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76 Sydney Children's Hospital Network, Submission 185, p. 14.

77 GUARD, Submission 46, p. 7.

78 Victorian Comprehensive Cancer Centre, Submission 61, p. 1.

79 Victorian Comprehensive Cancer Centre, Submission 61, p. 3.

> 'Umbrella' or 'basket' trials also facilitate recruitment of rare cancers into clinical trials by allowing a broad inclusion criteria that allows many rare cancer types.
>
> Examples of successful approaches utilising 'tumour agnostic' approaches include the identification of NTRK-gene fusions in a wide range of solid tumours types in both adult and paediatric patients, that allows treatment with targeted therapy.
>
> Because pharmaceutical company sponsored clinical trials are driven by market size and the ability to pay for expensive drugs, there is a health policy concern that a lack of clinical trials in some jurisdictions create significant disparities in access to new treatments and to better outcomes.[80]

8.94 The Sydney Children's Hospital Network commented that:

> Innovative trials should be encouraged and incentivised, including N of 1, adaptive, organoids and basket trials which are all currently in place in Australia. Regulatory approval pathways for rare diseases should be established to support the translation of these innovative trials into the health system.[81]

Limitations on data, research and clinical trials

8.95 In Australia, data for most rare diseases is not captured in either health information systems or registries and there is no coordinated strategy to collect, measure, build and translate data that does exist. For many rare diseases, there are many barriers to effective research and no active research programs. For many people living with a rare disease, participation in a clinical trial may be the only way to access treatment. Yet there are many challenges to running clinical trials. For example, there is no national infrastructure for rare disease clinical trials, nor a streamlined national ethics approval process.[82]

8.96 Market rewards for the development of new drugs for unmet medical needs such as new antimicrobials and some drugs for rare diseases can be insufficient to incentivise the needed R&D. This issue has been recognised as problematic and RVA was funded to develop an Action Plan to assist the

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80 Victorian Comprehensive Cancer Centre, Submission 61, p. 3.

81 Sydney Children's Hospital Network, Submission 185, p. 14.

82 RVA, Submission 86, p. 2.

Australian Government to provide a strategic way forward to support Australians living with rare disease now and into the future.

8.97 The Action Plan provides guidance around three priority pillars for Australians living with rare diseases. One of the three pillars is research and data. Action Plan Pillar 3 recommended the following for research and data:

- Priority 3.1: Enable coordinated and collaborative data collection to facilitate the monitoring and cumulative knowledge of rare diseases, informing care management, research and health system planning.
- Priority 3.2: Develop a national research strategy for rare diseases to foster, support and drive all types of research for rare diseases, contributing to agreed priorities and systematically addressing gaps.
- Priority 3.3: Ensure research into rare diseases is collaborative and person-centred.
- Priority 3.4: Translate research and innovation into clinical care; clinical care informs research and innovation.[83]

8.98 There is an understanding in the Australian rare disease community that, while research may not lead to better outcomes for people currently living with a rare disease, participating in research may drive change for future generations. This is supported by outcomes of the Rare Barometer survey undertaken in February 2018 by EURORDIS, Rare Diseases Europe.[84]

8.99 QIMR Berghofer suggested that 'research funding and grants with a focus on unmet needs and/or neglected health conditions should be developed.'[85]

8.100 Alpha-1 Organisation Australia commented that 'modifying or expanding existing structures could retain rare disease researchers and attract clinical trials to Australia. Other strategies could include:

- grant bodies such as the NHMRC could include dedicated rare disease grants and grants for repurposing drugs for rare disease
- post-doctoral scholarships in rare disease research could be mandatory in clinical trial design
- national awards for rare disease research could be promoted

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83 Department of Health, *National Strategic Action Plan for Rare Diseases,* Canberra, www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases, viewed 13 September 2021.

84 Department of Health, *National Strategic Action Plan for Rare Diseases,* Canberra, www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases, viewed 13 September 2021.

85 QIMR Berghofer, Submission 18, p. 1.

- a standardised rare disease data capture system could be developed / made available and offered to charities / other groups so that patient registries are available in all rare disease and a cohort of patients ready to be enrolled in research (reflecting priorities in the National Strategic Action Plan for Rare Diseases – Actions 3.2.5.1 and 3.4.2.1).[86]

8.101 The Pharmaceutical Society of Australia (PSA) represents the 34 000 pharmacists working across Australia. The PSA commented that the 'expertise of pharmacists can be better utilised to address the health care needs of all Australians.'[87]

8.102 Mr Chris Flood, National Manager, Pharmacy Guild of Australia, proposed an opportunity to be considered in the future for pharmacy trials and data collection.

> This is where there's a really good opportunity to capitalise on what pharmacists do, particularly if you're looking for patients who are living in regional or remote areas. You could actually have these patients participating and being supported by the local pharmacist who is able to assist with monitoring any of the outcomes. We actually see this already with a lot of the pharmacy trials that happen as part of the agreement. I see lots of opportunities that way.[88]

Rare disease register

8.103 RDIWG was supportive of the need to develop a Rare Disease Register and Clinical Trials Network. It commented that 'this would improve the national co-ordination of data collection and patient identification for trial participation in rare diseases with very small numbers. Additionally, the development of registries and better access to registry data by all stakeholders would facilitate the review of treatments.'[89]

8.104 Mrs Sheridan Campbell, Chair, Fabry Australia commented that many rare disease stakeholders and advocates called for a 'national rare disease registry and coordinated infrastructure to support these clinical trials'.[90]

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86 Alpha-1 Organisation Australia, Submission 29, p. 3.

87 PSA, Submission 203, p. 1.

88 *Committee Hansard,* 26 March 2021, p. 11.

89 RDIWG, Submission 51, p. 5.

90 Fabry Australia, Sydney, 11 March 2021, p. 20.

8.105 Professor Farrah from Luminesce called for the establishment of a rare diseases registry:

> Another thing that could help with health literacy would be a registry for people who are living with rare diseases. Registries serve multiple purposes in terms of knowing who can potentially access new therapies. I know from my patients that they live with the anxiety that something will be out there with them and I won't know about it and they won't know about it; it's a missed opportunity. While I know that there are privacy concerns with registries, my own experience with my patients is that they're very willing and wanting to be on registries to really make sure that they're not missing out on opportunities. Also they serve important functions in terms of dealing with appropriate clinical trial design, measuring real-world outcomes and informing phase IV studies to promote access, which is very important for the patients we treat.[91]

8.106 The Action Plan for rare diseases calls for the Australian Institute of Health and Welfare (AIHW) to re-establish the Australian National Congenital Anomalies Register (NCAR), including rare disease coding.

> This will accelerate, extend and nationalise rare disease coding already underway in the Western Australian Register of Developmental Anomalies (WARDA), and contribute to International Classification of Diseases 11th Revision (ICD-11) preparedness.[92]

Equity and funding for rare diseases

8.107 Professor Adam Jaffe, Member of Scientific and Medical Advisory Committee, RVA, discussed the future as having better equity for Australians living with rare disease. He stated:

> The process needs to involve innovation and new designs to really streamline that. It really comes down to better-value health care. With respect to the previous question on cost, we're really lucky in Australia. It's not the gold standard. We look at France as the gold standard for rare disease. It's written into the law of equity that if you've got a rare disease you have to have equitable treatment and funding.[93]

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91 *Committee Hansard*, Sydney 7 May 2021, p. 36.

92 Department of Health, *National Strategic Action Plan for Rare Diseases,* Canberra, p. 36, www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases, viewed 13 September 2021.

93 *Committee Hansard*, Sydney, 11 March 2021, p. 9.

8.108 MOGA was concerned that research priorities, commercial imperatives and advocacy favours access to oncology drugs and treatments for more common cancers in Australia.

> For instance, clinical trials for rare cancers are often conducted through collaborative trials groups with less industry support and the data collected may be less suited to registration and reimbursement requirements. The Australian regulatory process and our Government seem reluctant to fund effective treatments for rare cancers even though the overall impact on the health budget would be minimal.
>
> The negative impact of this situation on the quality care of Australian rare cancer patients and the lack of available treatment options is unacceptable. Our national systems for research and development, oncology drugs regulation and reimbursement need to be reviewed and revised to be supportive of drug development and access for rare cancers.[94]

8.109 MOGA stated:

> It is expected that novel technologies, such as cell and gene therapies, will provide significant long-term health benefits for patients. The types of interventions in development may have limited data at the time of assessment. Therefore, there should be a focus on the development of innovative access mechanisms to ensure patients have the advantage of being able to access treatment in parallel to the long-term collection of real-world evidence (RWE).[95]

8.110 RVA discussed the inequities of Australians living with rare disease:

> In the rare disease context, companies often state that it is unfeasible to submit for new indications, due to extremely small patient numbers, lack of conventional clinical trials etc. This means that many people living with a rare disease have to rely on the uncertainty of off-label use or self-fund (often equating to thousands of dollars in costs) to access a medicine that is recommended by their clinician. This is both unsustainable and inequitable.[96]

8.111 RVA has sourced de-identified data from one of Australia's largest public hospitals (that also includes a children's hospital) around their funding of off-label medicines. This is a sample from 2017 to August 2020.

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94 MOGA, Submission 50, p. 3.

95 RDIWG, Submission 50, p. 3.

96 RVA, Submission 86, pages 12-13.

> The data shows 570 instances of off-label hospital use that relate to a total of 30 medications for a total of 144 different indications/ reasons.[97]

8.112 GUARD is an alliance of genetic and rare disease networks. It made a comment that acknowledged the challenges the Government has in relation to research funding and incentives and described the reality that often those who get funded is not always the strategic and equitable approach.

> … we recognise that incentivising research as a key driver ultimately inevitably leads to inequity. The loudest voices get heard when there is not a strategic and agreed approach.[98]

8.113 Myasthenia Alliance Australia suggested government will need to invest in research for rare diseases.

> Governments will need to financially support research for rare diseases avoiding dollar driven limitations. Drug companies required by their shareholders to generate a profit are very unlikely to spend money on research when the return on the investment will be low. With a motivation of unrestricted considerations, best treatment outcomes can genuinely be sought.[99]

8.114 RDIWG suggested as the Government consider risk-sharing arrangements with manufacturers as it is likely that novel technologies will be associated with high upfront costs, whereas the benefits may occur over a prolonged period of time:

> The uncertainty about long-term outcomes will require a sustainable framework for risk-sharing arrangements between manufacturers and the Government.[100]

Paediatrics

8.115 The Committee heard from stakeholders that were calling for incentives for R&D for drugs and novel medical technologies where the needs exist in paediatrics.

8.116 Associate Professor Hansford, Australian and New Zealand Children's Haematology/Oncology Group, called for changes to our reimbursement

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97 RVA, Submission 86, p. 13.

98 GUARD, Submission 46, p. 7.

99 Myasthenia Alliance Australia, Submission 21, p. 3.

100 RDIWG, Submission 51, p. 3.

system pointing out that often adult cancer drugs were listed on the PBS however the paediatric cancer drugs were not.

> Currently, legislation exists around the world—and I would refer back to our submission around some of the specifics—on how you can increase paediatric inclusion. Whether for drug development, for the conduct of clinical trials or the approvals for these new and novel therapies, there are ways about how we can improve our access. Thirdly, paediatric cancer is not adult cancer. On the PBS, most drugs are not accessible for children.[101]

8.117 The Association of Australian Medical Research Institutes (AAMRI) commented that in Australia there are few incentives to develop or speed up the availability of drugs to combat rare diseases, or diseases in smaller populations such as in disease sub types or paediatrics. For these populations, the major barrier to approval of new drugs is that clinical trials are incredibly challenging and overly prolonged due to difficulties in recruiting participants as well as being expensive.

8.118 The current regulatory pathway seeks trial-based evidence for indicated populations. AAMRI suggested:

> There are approaches taken by the FDA in the US that could be considered in Australia by the TGA. One example is incentivising sponsors seeking approval of new drugs to have development plans in place for paediatric populations. Ensuring that there is a streamlined regulatory pathway could safely allow the use of cutting-edge, life-saving therapies in children sooner.
>
> In the US process, sponsors that have responded to this and are seeking drug approval are provided a patent extension of six months and are also provided accelerated approval vouchers that can be on-sold. The provision of such accelerated approval vouchers does not have a direct cost to government but does provide an incentivising mechanism.[102]

Committee Comment

8.119 Throughout the inquiry, the Committee received evidence from many advocates of rare diseases in Australia. The Committee is grateful to all the impassioned individuals, supportive family members and carers and organisations that put in submissions and took the time to speak with the Committee.

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101 *Committee Hansard*, Melbourne, 23 April 2021, p. 52.

102 AAMRI, Submission 88, p. 9.

8.120 The Committee noted the comprehensive priorities of the National Strategic Action Plan for Rare Diseases (the Action Plan) that were endorsed by many submitters to the inquiry. Several of the priorities related to the inquiry's terms of reference and it is hoped that the Australian Government will use both the Committee's recommendations and the Action Plan's priorities to move forward on rare disease policy reform.

8.121 It was evident that the Australian Government needs to consider how best to develop fit-for-purpose Health Technology Assessment (HTA) pathways for rare diseases now and into the future. The biggest challenges are to manage the issue of equity and timely access to new medicines and devices for people living with rare disease. The Committee recommends a new HTA pathway be developed for cell and gene therapy in Australia. The evidence was clear that Australia's HTA systems will receive an influx of requests for registration and reimbursement in cell and gene therapies and treatments over the next few years. Australia will need to have robust HTA processes in place to enable Australians to access these new treatments in an equitable and timely manner.

8.122 The Committee heard that cell and gene therapy and treatment is at the forefront of a new wave of precision medicine that is currently on Australia's doorstep and these therapies and treatments require new pathways to allow for its seamless regulation.

8.123 The Committee believes it is pertinent for the Department of Health to expand its understanding and expertise of rare diseases with a focus on precision medicine and cell and gene therapies. The Committee recommends the establishment of a 'Centre for Precision Medicine and Rare Diseases' within the Department of Health. The objective of this Centre is to ensure the capacity of the Department of Health is enhanced to ensure Australians have timely access to new drugs and novel medical technologies, including for rare diseases. This Centre would provide education and training information on precision medicine and rare diseases and would develop a comprehensive horizon scanning unit within the Centre.

8.124 A significant advantage of novel health technology development, such as cell and gene therapies compared to that of conventional pharmaceuticals, is their utilisation of modular and platform technologies that can be rapidly reconfigured to treat multiple disease targets such as genetic diseases, cancers and infectious diseases. This may lead to more cost effective treatments in the future.

8.125 The Committee agrees with submitters that affordable access to genomic testing is needed not only for patients but for the future of Australia's health system. Therefore the Australian Government should establish a National Genomics Testing Program to provide equitable access to genomics testing nationwide, including provision for genomics counselling for all patients.

8.126 It is the Committee's view that there is the need for a greater role for patient evidence in HTA decision-making where traditional clinical evidence is inadequate, such as for many rare diseases. It encourages the Department of Health to give the highest possible priority to strengthening the role patient evidence plays in its HTA decisions, particularly in relation to rare diseases.

8.127 The Committee believes the Australian Institute of Health and Welfare (AIHW) should be funded to re-establish the Australian National Congenital Anomalies Register (NCAR) and should consider how this register could be adapted to capture appropriate data for rare disease.

8.128 Australia's HTA system needs to take into consideration rare disease and develop robust pathways that provide equity and access to treatments and therapies that don't fit neatly into the current system such as rare cancers, antimicrobials, orphan drugs, and upcoming precision medicines.

8.129 Included in the independent HTA Review should be a pathway that facilitates consideration of new medicines and therapies for paediatric populations for a broad range of conditions. In particular, the Committee believes the Australian Government should streamline a regulatory pathway to safely allow the use of cutting-edge, life-saving therapies in children sooner.

8.130 The Committee acknowledges that there are specific problems with how the orphan drug criteria apply to paediatric medicines, as well as to medicines with an unknown prevalence in Australia, which it believes the TGA should remedy. The Committee believes that there is merit in the suggestion that the orphan drug designation should be automatically linked to the priority review pathway, to support faster access to medicines for rare disease patients and to reduce the administrative burden on the TGA.

8.131 **The Committee is of the view that applicants shouldn't have to wait for a rejection from the PBAC before they lodge with the LSDP Expert Panel. This current system adds considerable time before patients have access to life saving drugs. A new approach is required that allows earlier and faster consideration. The Committee believes there is merit in either providing sponsors with immediate pathways to the LSPD Expert Panel or considering whether** the same purposes of the LSDP could be achieved by

providing a new pathway through a PBS section 100 program, with specific criteria, as with other section 100 programs.

8.132 One of the main challenges in supporting better rare disease treatment is that research and development of new medicines and devices is time consuming and expensive. The Committee believes that a more sustainable framework is required for rare disease funding and risk-sharing arrangements between manufacturers and the Australian Government.

8.133 Lastly, the Committee believes it is imperative that the Australian Government complete the standardisation of the national newborn screening framework so that every baby born in any state or territory of Australia receives the same newborn screening test. Further, the Committee believes that this program should be urgently expanded, based on new understandings of genomic testing for conditions and international best practise, with a review undertaken every two years.

9. Clinical Trials

Overview

9.1 Clinical trials are essential for evaluating the effectiveness and safety of medicines, devices, services and interventions to help prevent, detect or treat illness and disease. It is through the research done in clinical trials that people gain access to better treatments. Clinical trials also boost the economy and support a highly skilled workforce.

9.2 Australia has an excellent reputation in relation to the safety and quality of its clinical trials. Over the last decade there has been work done at the national, state and territory levels together with industry and stakeholders to improve the clinical trial environment and to attract more clinical trials to Australia. It is in Australia's best interest to hold onto its reputation as a tier one clinical trials country to reap the benefits from a public health and economic perspective.

9.3 Many countries compete internationally for clinical trials as they provide early access to novel medicines and therapies for patients. They advance medical knowledge including increasing clinician experience with new innovations and enhance the translation of evidence into local practice. In addition, clinical trials forge links between local and international researchers and drive investment in Australia's economy.

9.4 In Australia, a number of national policy initiatives and investments have underpinned recent improvements in the clinical trials sector. The Australian Government's Clinical Trials Project Reference Group oversaw the implementation of the $7 million Federal Budget measure in 2016 to encourage more clinical trials in Australia, with a further $6 million

committed in the recent 2021-22 Federal budget to enhance Australia's status as a leading option to conduct clinical trials.[1]

9.5 Industry investment in active clinical trials was estimated to be worth over $1 billion to the Australian economy in 2015[2]. This investment helped support 6,900 jobs with a potential for up to 6,000 new highly skilled jobs to be created by 2025.[3]

9.6 The environment in which clinical trials are conducted is complex, often occurring across multiple jurisdictions and with every trial needing ethics and governance approvals before it can commence.

9.7 In Australia, clinical trials are delivered by teams of clinical trial investigators and clinical and non-clinical staff working in partnership with trial sponsors, regulators, trial participants, consumers, patients their families and carers. Clinical trials are delivered in public and private health service organisations and in trial sites ranging from sole proprietorships to large statutory corporations and public companies.

9.8 This chapter outlines the Government's current clinical trial regulations and outlines Australia's current competitive advantage for attracting clinical trials. It discusses what changes are needed and why, in areas of regulation, infrastructure, funding, clinical trial registries, data collection and reporting requirements. These changes will ensure Australia maintains its excellent reputation as a tier one clinical trial country now and into the future.

Regulations for clinical trials in Australia

Clinical Practice Guidelines in Australia

9.9 Australia has adopted the European Union version of Good Clinical Practice (GCP) guidelines. These guidelines detail the requirements for trial documentation, protocol amendments, requirements such as indemnity, reporting lines for adverse events and provision of medical care for trial participants.[4]

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1 Australian Commission on Safety and Quality in Health Care (ACSQHC), Submission 207, p. 2.

2 Roche, Submission 92, p. 23.

3 Pfizer Australia, Submission 137, p. [7].

4 Department of Health, *Australian Clinical Trials,* Canberra, www.australianclinicaltrials.gov.au/researchers/good-clinical-practice-gcp-australia, viewed 23 August 2021.

9.10 Although the methods for implementing and enforcing the principles of Good Clinical Practice vary, the main objective is a global environment in which trials collect high quality, credible data that contribute to the answering of specific scientific and clinical questions, while most importantly protecting the rights, safety and well-being of clinical trial participants.[5]

9.11 Complementing these guidance documents is Australia's National Statement on Ethical Conduct in Human Research (National Statement), published by the National Health and Medical Research Centre (NHMRC). The National Statement provides guidance on a wide range of ethical issues in human research. It describes the overarching principles of ethical conduct in research, but provides guidance for specific types of research, specific instructions for the formation and operation of human research ethics committees, advice regarding consideration of multi-centre research and specific issues for Human Research Ethics Committees (HREC) to consider when reviewing a clinical trial proposal.[6]

9.12 The National Statement requires that, before granting approval to a clinical trial, a HREC must be satisfied that the protocol conforms to:

- the National Statement
- the World Medical Association Declaration of Helsinki
- where relevant, the CPMP/ICH Note for Guidance on Good Clinical Practice (CPMP/ICH-135/95), the ISO 14155 Clinical Investigation of Medical Devices and the requirements of the TGA, and
- any requirements of relevant Commonwealth or state/territory laws.[7]

Therapeutic Goods Administration regulates access only

9.13 In Australia, the Therapeutic Goods Administration (TGA) must be notified of clinical trials involving unregistered therapeutic goods and the intention to start a new trial under the Clinical Trial Notification (CTN) Scheme or the

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5 Department of Health, *Australian Clinical Trials,* Canberra, www.australianclinicaltrials.gov.au/researchers/good-clinical-practice-gcp-australia, viewed 23 August 2021.

6 Department of Health, *Australian Clinical Trials,* Canberra, www.australianclinicaltrials.gov.au/researchers/good-clinical-practice-gcp-australia, viewed 23 August 2021.

7 Department of Health, *Australian Clinical Trials,* Canberra, www.australianclinicaltrials.gov.au/researchers/good-clinical-practice-gcp-australia, viewed 23 August 2021.

Clinical Trial Authorisation (CTA) Scheme. The TGA also ensures compliance with International Organization for Standardization Guidelines for Therapeutics and Medical Devices.[8]

Regulatory pathway for clinical trials

9.14 The TGA operates the CTN and a Clinical Trial Exemption (CTX) schemes.

9.15 Clinical trials of unapproved medicines in Australia are conducted by a trial sponsor with oversight by a HREC. For the vast majority of trials that are notified through a CTN, the TGA does not (re-) evaluate the trial.

9.16 Clinical trials that do not involve the use of 'unapproved' therapeutic goods (including placebos) are not subject to CTN or CTX requirements. However, all clinical trials require HREC approval before the clinical trial can commence.

9.17 The Department of Health (the Department) provided the following table of information on the number of notifications for new clinical trials involving unapproved therapeutic goods received for the past few years:

Table 9.1 Notifications for new clinical trials for unapproved therapeutics

Year	2017–18	2018–19	2019–20
Number of new clinical trial notifications (CTN)	920	1059	984

Source: Department of Health, Submission 15, p. 21.

9.18 The CTX route is generally designed for high-risk or novel treatments where there is no or limited knowledge of safety. For medical device trials, the CTX scheme may be more appropriate where the experimental device introduces new technology, new material or a new treatment concept, which has not been evaluated previously in clinical trials in any country. The CTX scheme should be considered for medical devices that pose a risk of serious patient harm.[9]

8 Australian Commission on Safety and Quality in Health Care (ACSQHC), Submission 207, pages 2-3.

9 Department of Health, Submission 15, p. 21.

9.19 In many cases, a HREC recommends that the CTX scheme is used and this will depend on whether the committee has access to appropriate scientific and technical expertise in order the safety of the product. However, certain Class 4 biologicals must be submitted under the CTX scheme.[10]

Clinical trials involving Genetically Modified Organisms

9.20 The Department informed the Committee of the mandatory processes when dealing with Genetically Modified Organisms (GMOs).

> Before a clinical trial involving GMOs can proceed, it must be appropriately authorised under both the *Gene Technology Act and the Therapeutic Goods Act 1989*. Each approval process is independent and typically occurs in parallel.
>
> As risks to trial participants are addressed through oversight by TGA and HRECs, the Gene Technology Regulator's focus is on assessing risks posed to people other than those participating in the clinical trial, and to the environment. This includes risks to people preparing and administering the GMO therapeutic, and risks associated with import, transport and disposal of the GMO. Clinical trials with CAR-T cells are Exempt Dealings (no licensing required under the *Gene Technology Act*).[11]

Australian Government initiatives and funding

9.21 In late 2020, the Department outlined the Australian Government's multi-pronged approach to encourage clinical trials in Australia, taking into consideration the current environment and the impacts of the COVID-19 pandemic. This included: international promotion; funding to attract international clinical trials and research, investigators and investment; and streamlining the operating environment and improving processes to make it easier to undertake trials in Australia.

9.22 The Department informed the Committee that virtual roadshows promoting Australia as an ideal destination for clinical trials to potential sponsors in Greater China, Korea and the United States were delivered in partnership with industry stakeholders, with 300 industry delegates attending the sessions from these regions. Austrade led a strong Team Australia delegation (hybrid) to Bio Korea 2021 with all major states and MTPConnect,

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10 Department of Health, Submission 15, p. 21.

11 Department of Health, Submission 15, p. 21.

and involving both physical pavilion and virtual Australia capability promotion activities.[12]

9.23 The Australian Government has been focused on providing direct investment in the clinical trials sector to encourage companies to undertake clinical trials in Australia. Two examples include:

> … the Biomedical Translation Fund (BTF) which invests in promising biomedical discoveries with the aim to address various costs constraints, which may include support for clinical trials in Australia. The Modern Manufacturing Initiative (MMI) Translation grant stream for medical products in part aims to help overcome barriers to commercialisation costs including costs associated with clinical trials.[13]

Government funding for clinical trials

9.24 The Australian Government announced a $5 billion, 10 year investment plan for the Medical Research Future Fund (MRFF) in the 2019-20 Federal Budget. This plan continues to support lifesaving research and gives researchers and industry some longer term certainty and direction. Under the MRFF 10 Year Plan, $614 million has been committed to the Clinical Trials Activity Initiative.

9.25 Programs funded under MRFF Clinical Trials Activity Initiative include Rare Cancers, Rare Diseases and Unmet Need (RCRDUN) and International Clinical Trial Collaborations (ICTC).[14]

9.26 RCRDUN supports clinical trials research that investigate new drugs, devices or treatments for rare cancers/diseases or for areas of unmet medical need. Examples of funded grants include studies on larotrectinib (a new drug) for children with newly diagnosed high-grade glioma; treating mitochondrial dysfunction with a novel form of anaplerosis; and clinical trial combining azactidine and defactinib for high-risk myelodysplastic syndrome patients who fail to respond to azacitidine alone.[15]

9.27 The Department commented that in 2020-21 'there will be two grant opportunities under the Clinical Trials Activity Initiative including

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12 Department of Health, Submission 15.6, p. 27.

13 Department of Health, Submission 15.6, p. 27.

14 Department of Health, Submission 15, p. 20.

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$25 million for Rare Cancers, Rare Diseases and Unmet Need for COVID-19 and $25 million for Rare Cancers, Rare Diseases and Unmet Need.[16]

9.28 The Australian Government has invested a total of $614.2 million over 10 years in the Clinical Trials Activity Initiative to increase clinical trial activity in Australia.[17]

9.29 The ICTC, which has been consolidated into the Clinical Trial Activity Initiative under the MRFF 10-year plan, supports Australian research teams to lead or participate in international investigator-initiated clinical trials through the establishment and co-ordination of clinical trial sites in Australia. Researchers, not pharmaceutical companies, run this type of trial.[18]

Australia's competitive advantage

9.30 Throughout the inquiry many stakeholders reiterated that Australia has a competitive advantage of running high quality clinical trials on an international scale. More specifically, witnesses commented that Australia is well respected and has a well-qualified workforce that included nurses, research centres and hospitals. However these stakeholders went on to suggest that there were critical areas of clinical trials that needed immediate reform to future proof Australia's standing as a tier one nation for clinical trials.

9.31 Medicines Australia set the scene and informed the Committee that:

> In 2019, there were 1,820 ongoing trials in Australia: a 22% increase on 2015. This contributes an estimated $1.1 billion a year to the economy.[19]

9.32 Medicines Australia suggested that:

> …sustaining this reputation is increasingly challenging, as international competition for the placement of clinical trials has already begun to erode Australia's advantage. Rather than relying on historical recognition as a reliable destination for quality clinical research, Australia needs to actively

16 Department of Health, Submission 15, p. 20.

17 Ms Jessica Pace, Submission 40, p. 2.

18 Department of Health, *Clinical Trials*, Canberra, www.health.gov.au/initiatives-and-programs/international-clinical-trial-collaboration-ictc-initiative, viewed 30 August 2021.

19 Medicines Australia, Submission 141, p. 25.

> demonstrate superiority against other international benchmarks in clinical trials, to secure status as a preferred destination of choice.[20]

9.33 Dr Anna Lavelle, Chair, Medicines Australia, commented that Australia is seen as being attractive for clinical trials for two reasons:

> One is that Australia has ethnic diversity, which is very attractive to companies. Also, we have quality data, so our data is considered highly reliable, which is extremely important for them.[21]

9.34 Dr Kaustuv Bhattacharya, Scientific Adviser, Rare Voices Australia (RVA) stated that Australia does deliver high quality clinical trials however it needs to improve its efficiency of delivering trials:

> We definitely have high-quality clinical trials delivered by this country. That's what we have to be able to market as a country: we deliver and we deliver a good product. But we have to do it more efficiently and effectively than we have done so far.[22]

9.35 Roche highlighted that there are enormous growth opportunities for clinical trials in Australia.

> Compared to most other nations Australia has managed the COVID-19 pandemic relatively well, and this provides a stable environment to undertake clinical trials. Combined with other key population advantages and a strong medical research sector there is room for expansion.
>
> However, expansion within some hospital and healthcare settings will need encouragement. Most large hospitals are already inundated with clinical trials and are not able to take up all opportunities because of space and capacity constraints. Work will need to be undertaken to grow the clinician researcher workforce, find funding solutions for sponsor monitoring, data management and research nurse costs, and crucially, provide space within hospitals.[23]

9.36 AusBioTech highlighted the importance of maintaining Australia's competitive advantage to attract local clinical trials. It stated:

> Global competition for clinical trial investment is intense and hinges on factors such as start-up times, researcher capabilities, tax incentives and quality

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20 Medicines Australia, Submission 141, p. 5.

21 *Committee Hansard,* 26 March 2021, p. 4.

22 *Committee Hansard*, 11 March 2021, p. 8.

23 Roche, Submission 92, p. 23.

> assurance. Investment capital is mobile and will move to the country best able to meet the research needs in the time available.[24]

Early access to new medicines

9.37 Clinical trials not only assists in increasing access to research and potential new medicines in the health care system, they provide one avenue for patients to receive early access to new medicines.

9.38 AusBioTech suggested that early access to new medicines through clinical trials is an important part of the health ecosystem. It discussed the benefits of providing access to patients for new treatments, and other benefits for researchers and the health sector education and training experience and economic gains.

> This access has been estimated to save Australian taxpayers around $100 million annually in healthcare costs, as well as providing patients with significant benefits from timely treatments. This healthcare saving includes reduced Government expenditure on the PBS due to patients' access to innovative treatments. Other benefits include: enhanced translation of evidence into local practice; enhanced local clinical trial expertise; enhanced global profile and linkages for Australian researchers; and retention of researchers in the Australian public health system. [25]

9.39 Whitecoats Foundation illustrated:

> Clinical Trials can also provide patients with access to potentially life-saving options in the management of their health particularly in circumstances where choices are limited or there are none.[26]

Rare Disease

9.40 For many people living with a rare disease, participation in a clinical trial may be the only way to access treatment.

9.41 A 2016 Australian study found that almost 90 per cent of respondents living with a rare disease were interested in joining a patient registry, in recognition of the key role that registries play in linking people living with a rare disease with clinical trials for new health technologies (drug treatments and therapies). The translation of rare diseases research into clinical settings,

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24 AusBioTech, Submission 118, p. 11.

25 AusBioTech, Submission 118, p. 11.

26 Whitecoats Foundation, Submission 136, p. 2.

while currently hampered, is vital. This two-way relationship benefits from active participation by patients, their families and carers, and patient advocacy groups to ensure the best outcomes for people living with a rare disease.[27]

The challenges for clinical trials in Australia

9.42 The Committee received numerous suggestions from stakeholders in relation to improvements that could be made to make Australia a more attractive location for clinical trials.

9.43 The harmonisation of regulations and reducing red tape was clearly at the top of the list requiring immediate attention. Other barriers discussed included the non-existence of a national clinical trial register, lack of data and reporting, infrastructure deficits, rising costs, and cell and gene technology limitations.

Regulation – ethics and governance

9.44 Many submitters stressed that the harmonisation of regulations between jurisdictions had received a lot of attention recently however they commented there was still more to do if sponsors were going to view Australia as a favourable country to undertake clinical trials here in the future.

9.45 Research Australia commented that the road to reform had been long and slow and that a better 'single system' technological platform was required:

> The reform of the Australian clinical trials environment has been ongoing for over a decade. While progress has been made in many areas there is still more work to be done. When it comes to ethics approval, the current National Mutual Acceptance scheme has been an improvement, but more work needs to be done to achieve a truly national and all-inclusive scheme.
>
> More can also be done to create a 'single system' post the ethics approval, with the adoption of common technology platforms, processes and reporting requirements by all parties, including state regulators.[28]

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27 Department of Health, *National Strategic Action Plan for Rare Diseases,* p. 23, Canberra, www.health.gov.au/resources/publications/national-strategic-action-plan-for-rare-diseases, viewed 13 September 2021.

28 Research Australia, Submission 78, p. 10.

9.46 Myositis highlighted disincentives for sponsors bringing clinical trials to Australia and described the delays incurred from individual ethics and governance regulations:

> The diverse ethics approval processes between Australian research sites is a disincentive for clinical trial set-up and results in recruitment delays and cost burdens.
>
> Australia's decentralised Ethics Committee process is a discouraging aspect of the conduct of clinical trials in this country.
>
> Under the Australian system, the ethics review process for clinical trials requires human research protocols to be reviewed by institutional level ethics committees. That is, the Ethics Committee at each hospital/institution site for the trial, both public and private, undertakes its own review of the ethics protocols. In addition to delays, this can lead to inconsistencies, lack of transparency and lack of public accountability.[29]

9.47 Medicines Australia also emphasised the time delays as problematic and called for one ethics approval that is acceptable to all institutions.[30]

> …the start-up of a clinical trial involves a range of activities, the most significant of which is the ethical review and approval of the trial by a Human Research Ethics Committee (HREC) and the Research Governance Review and approval via a Site-Specific Assessment (SSA). These processes are almost always managed consecutively at present, despite local evidence that parallel review significantly increases start-up times.[31]

9.48 Ms de Somer, CEO, Medicines Australia, continued:

> The second barrier is that each individual institution then implements their own governance processes, which obviously are relevant to that institution but differ from institution to institution. Therefore, trying to get through all of the governance at each institution wastes time. We believe there could also be agreement across public and private institutions on the level of governance that's required so that one standard of governance would be suitable for all institutions.[32]

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29 Myositis, Submission 79, p. 1.

30 *Committee Hansard,* 26 March 2021, p. 4.

31 Medicines Australia, Submission 141, p. 25.

32 *Committee Hansard,* 26 March 2021, p. 4.

9.49 Medicines Australia outlined difficulties in streamlining the Ethics Review for clinical trials:

> Success has been limited as public health policies do not allow the use of all ethics committees that have been nationally certified by the [National Health and Medical Research Council] NHMRC for multi-centre research (e.g. private ethics committees).
>
> In addition, public health policies do not routinely allow private research centres to be covered by public hospital ethics committees without a range of varying written agreements in place. As it is very common for a mix of public and private trial centres to be included in trials, at least two ethics committees are required and possibly three if university centres are also involved. This leads to a duplication of effort, increased costs and inefficiency for the initial submission and delays in approval of a clinical trial, resulting in unnecessary delays in patient access to medical treatment.[33]

9.50 Medicines Australia suggested several ways to improve the current system:

> To improve the efficiency of regulatory processes the review and approval times for Human Research Ethics Committees (HRECs) and Research Governance Offices (RGOs) should be prescribed to an acceptable timeframe. For multi-centre trials conducted across sites residing in different jurisdictions, it is usual to require the services of more than one HREC and each trial site conducts its own Research Governance Review. The timelines for review and approval of the trial by both HRECs and Research Governance offices (RGOs) are variable and unpredictable.[34]

9.51 Numerous stakeholders including AAMRI echoed the following sentiments and all agreed that despite the efforts that have already taken place to streamline the ethics and governance processes, more was need to be done in a timely manner:

> The ethics and governance approval processes can take too long, delaying clinical trials and making Australia a less appealing destination for investment. The COVID-19 pandemic has shown that when needed the approval process can be sped up safely.
>
> For Australia to be more competitive in a global market a streamlined approval process is needed. Significant work has been undertaken to find ways to streamline processes, such as the accreditation framework for clinical trial sites, but more effort is needed. The processes currently differ by state, by

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33 Medicines Australia, Submission 141, p. 26.

34 Medicines Australia, Submission 141, p. 25.

institution, by setting, and whether the recruiting site is public or private. Action is needed to both speed up processes, and for one single approval process to cover clinical trials across the whole of Australia.[35]

9.52 Roche Australia stated:

> If Australia is to remain competitive on this global stage, we need to continue to advance the environmental conditions for clinical trials. An important first step in this regard would be to reduce red-tape around how clinical trials are structured and administered.[36]

9.53 QIMR Berghofer submitted the following:

- Mutual acceptance of ethical review should be implemented nationally (where research has been reviewed and approved by an NHMRC-certified Human Research Ethics Committee)
- Standardised governance processes (site-specific assessment) should be implemented across public health hospitals and health services.[37]

9.54 AusBioTech highlighted many of the red tape difficulties that industry must navigate to bring clinical trials to Australia:

> There are over 200 Human Research Ethics Committees (HRECs) in Australia, each with similar concerns, but different requirements. The National Mutual Acceptance (NMA) Scheme only supports the acceptance of a single scientific and ethical review for multi-centre research conducted in publicly funded clinical sites. Given that clinical trials are commonly conducted across public and private hospitals, ethical approvals for trial start-up must be separately granted by different HRECs, leading to multiple submissions and unnecessary duplication of effort.[38]

9.55 Mrs Nettie Bourke from Cystic Fibrosis Australia called for a national office of ethics:

> We really believe that there should be an office of ethics which would oversee ethics across Australia. That would be about ethics for clinical trials and also for registries. After COVID, the big companies overseas want to come to

35 AAMRI, Submission 88, p. 7.

36 Roche, Submission 92, p. 24.

37 QIMR Berghofer, Submission 18, p. 1.

38 AusBioTech, Submission 118, p. 12.

> Australia to do clinical trials because we have a clean environment, but the ethics get in our way every time. That's where the expertise comes in.[39]

9.56 Mr Lance Dale, Policy Officer, Save or Sons Duchenne Foundation, highlighted a serious barrier that Australia has in terms of ethics approval processes. He stated:

> We have a problem with protracted ethics and research government approval processes, which lag way behind the rest of the world. We take 100 to 160 days to get approval, compared to the UK's 90 days. Something can be done in that space.[40]

9.57 Mr Ali, Chief Executive Officer (CEO), MND Australia concurred with the calls to streamline the ethics approval process for clinical trials. He stated:

> One of the other recommendations is the need to implement a streamlined single-point ethics approval process rather than the very convoluted, time-intensive ethics approval process. We need to be concerned about how quickly people die with MND and why we need the processes to change.[41]

National clinical trials governance framework

9.58 The Australian Commission on Safety and Quality in Health Care was engaged by the Department to develop a National Clinical Trials Governance Framework (the Governance Framework) on behalf of all Australian jurisdictions. The draft Governance Framework was endorsed for implementation by Health Ministers in 2019. Its implementation will streamline clinical trial approval processes and improve time to trial start-up, workforce capacity, and engagement with sponsors.

9.59 The Governance Framework requires health service organisations to do the following:

- Monitor compliance with national regulation, legislation and policies and requires health services conducting clinical trials to monitor compliance with legislation, regulation and state or territory requirements
- Keep information about instances of noncompliance with the organisation's policies, procedures and protocols. Where appropriate,

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39 *Committee Hansard*, Sydney, 11 March 2021, p. 33.

40 *Committee Hansard*, Sydney, 11 March, p. 35.

41 *Committee Hansard,* Canberra, 26 March 2021, p. 4.

incorporate the information into the organisation's risk register and quality improvement planning processes.

- Maintain well-designed legislative compliance processes. Incorporate a compliance register to ensure that the organisation's policies are regularly updated, enabling the organisation to respond to regulatory changes, compliance issues and case law.[42]

9.60 The Department commented that:

> The pilot and finalisation of the National Clinical Trials Governance Framework is an important element of the clinical trials reform agenda to ensure nationally consistent accreditation of health services undertaking trials. In November 2019, all Health Ministers endorsed the Governance Framework and the national pilot commenced on 1 September 2020 following a COVID-19 suspension. Pilot outcomes will be evaluated in early-mid 2021.[43]

9.61 In August 2021, the Department provided the Committee with the following update on the Governance Framework:

> A priority is to continue to build on recent work to develop and pilot the National Clinical Trials Governance Framework, currently being finalised and widely recognised as a significant and positive reform for the sector. Implementation, anticipated from 2022, will streamline trial approval processes, improve time to trial start-up, improve workforce capacity, reduce administered efficiencies and better engage sponsors. The outcome will be the integration of clinical trials into health service corporate and clinical governance systems and nationally consistent accreditation of clinical trial services under the National Safety and Quality Health Service Standards.[44]

National mutual acceptance scheme

9.62 The Committee received evidence from the Australian Commission on Safety and Quality in Health Care (ACSQHC) discussing its current work scoping the expansion of the National Mutual Acceptance Scheme across public health services in all states and territories. The objective of this scheme will:

> … enable mutual recognition of non-public, accredited Human Research Ethics Committees (HRECs) approvals by the public, private and not-for-

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42 ACSQHC, Submission 207, pages 3-4.

43 Department of Health, Submission 15, pages 21–22.

44 Department of Health, Submission 15.6, p. 28.

> profit sectors, will streamline the acceptance of the HREC approvals across jurisdictions and the public and private health care sectors.[45]

9.63 The ACSQHC continued:

> Consultation is also underway on the requirements for a national platform for all health and medical human research, with jurisdictional and industry support. It represents an opportunity for transformative change for the sector, providing a national ethics authorisation and research management system, which incorporates of the Clinical Trial Notification (CTN) and Clinical Trial Authorisation (CTA) schemes. This platform will reduce duplication, expedite approvals and trial commencement and enable the first real-time national picture of health and medical human research activity.[46]

Training as an investment

9.64 In addition to providing the incentives to encourage industry to bring more clinical trials to Australia, Australia should ensure it has the best infrastructure and training in place to facilitate clinical trials.

9.65 Continuous educating and training of staff involved with clinical trials was an important issue raised by Medicines Australia:

> … we are already falling behind the eight ball on creating the infrastructure for clinical trials because we don't have training of clinical trial nurses, assistants and people who monitor clinical trials, and that's for the number clinical trials we've got right now. We are also in this unique position of being relatively COVID free with a First World health infrastructure. Clinical trials are as much of an export as education. We should be exporting our knowledge, our capacity and our ability to do clinical trials to encourage that direct foreign investment into more clinical research, so there is absolutely an opportunity.[47]

9.66 Johnson and Johnson discussed the importance of training in areas including the potential benefits of standard training processes for 'staff involved in governance activities and standard processes for centres involved in clinical research.'[48]

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45 ACSHHC, Submission 207, p. 2.

46 ACSHC, Submission 207, p. 2.

47 *Committee Hansard*, 26 March 2021, p. 6.

48 Johnson and Johnson, Submission 134, p. 17.

National One-Stop-Shop

9.67 The ACSHC is currently conducting national consultations on behalf of all jurisdictions to scope the requirements for a National One-Stop-Shop. This proposed national online portal will make it easier for researchers, industry representatives and sponsors to find, conduct, participate and invest in research in Australia.

9.68 The concept for the National One-Stop-Shop was developed by the Clinical Trials Project Reference group and presents a significant opportunity to achieve a national, interconnected, rapid and streamlined approvals platform that will:

- include a cross-jurisdictional ethics and governance approvals platform that incorporates key application, notification and approval systems
- incorporate the Clinical Trials Notification and Clinical Trials Approval schemes administered by the Therapeutic Goods Administration (TGA)
- include an embedded and automated next-generation national clinical trials registry
- provide sophisticated monitoring and reporting functionality for different users.[49]

9.69 Options for improving patient recruitment through a related National Clinical Trials Front Door will be considered. Consultations start in July 2021 to gather the requirements for the National One-Stop-Shop and the National Clinical Trials Front Door. A project advisory group, chaired by Professor Ian Chubb, former Chief Scientist and clinical trial participant, will be established to guide the consultation process.[50]

9.70 In August 2021, the Department provided the Committee with an update of the One Stop Shop for Clinical Trials and Human Research Approvals and commented:

> The announcement to establish the one stop shop has been applauded by the sector and presents a significant opportunity to achieve a national,

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49 Department of Health, *Clinical Trials*, Canberra, www.australianclinicaltrials.gov.au/australian-government-clinical-trials-initiative, viewed 23 July 2021.

50 Department of Health, *Clinical Trials*, Canberra, www.australianclinicaltrials.gov.au/australian-government-clinical-trials-initiative, viewed 23 July 2021.

> interconnected, rapid and streamlined approvals platform and will make it considerably easier to undertake and participate in research in Australia. [51]

9.71 The Department continued:

> It builds on international evidence that nationalised platforms are critical to building a stronger and more competitive research sector, and that jurisdictional collaboration is critical to success in federated systems.
>
> The National One Stop Shop will facilitate rapid and streamlined approvals and address long-standing challenges with duplication, delays and fragmentation that are unlikely to be otherwise overcome. It will underpin the new nationally consistent approach to accreditation for trials sites in public and private hospitals, and provide reporting functionality that will serve to maintain Australia's reputation for safety and quality in research, and drive quality improvement and strategic positioning.
>
> Through ongoing and effective collaboration with jurisdictions, the Commonwealth Government considers that a harmonised national approach is achievable through the Governance Framework, to incorporate clinical trials into routine health service provision, and the single national platform for approvals – the One Stop Shop.[52]

National clinical trials registry

9.72 Many witnesses called for a national clinical trials registry. There were discussions around the challenges that exist between Commonwealth and States that precluded a streamlined system from being in place in Australia. This was seen as a difficulty to navigate for not only industry but for patients and clinicians as well.

9.73 Whitecoats Foundation described the benefits that would accrue if Australia developed a national clinical trials registry.

> Improving awareness and participation rates to clinical trials in Australia not only delivers potential health benefits to patients but it can also improve Australia's profile as a more desirable destination to conduct more clinical trials.
>
> Protracted recruitment timelines delay time to market for sponsors and can lead to increased research and development costs. The lost revenue

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51 Department of Health, Submission 15.6, p. 28.

52 Department of Health, Submission 15.6, p. 28.

> opportunity is estimated at $600k/day for niche market drugs and up to $8million/day for blockbuster drugs.
>
> Timely recruitment and sites/countries with demonstrated success are favoured by sponsors.[53] There are many factors that can affect recruitment, however, one of the key reasons associated with poor participation and engagement is low awareness. The awareness issues extend to both health care professionals and the general public.[54]

9.74 Many of the peak bodies for disease informed the Committee that they already have patient registries. Dr Gethin Thomas, Executive Director, Motor Neurone Disease (MND) Australia, made the following salient comment about clinical registries:

> We have a national patient registry for MND that is funded through an NHMRC partnership grant plus contributions by ourselves and Fight MND. That has been very effective. ... The biggest problem for these is funding… It's very easy to establish these; it's very difficult to maintain them.[55]

9.75 Medicines Australia emphasised that this area of patient recruitment needs a digital innovation solution that will secure patient recruitment and consent and deliver cost effective data from the trials.[56]

> Australia will need to improve patient identification, patient recruitment, retention and completion rates for existing trials.
>
> As many as 86% of clinical trials do not reach participant recruitment targets and as such, the ability of sites within a country to recruit to their contracted participant target is a key factor in study placement in the country.[57]

9.76 HealthMatch suggested that the Australian Government needed to support truly patient-centric tools and technologies that promote equal and direct access to discovery, education and participation in clinical trials.[58]

9.77 AstraZeneca recommended the following measures could make Australia a more attractive location for clinical trials:

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53 Whitecoats Foundation, Submission 136, p. 2.

54 Whitecoats Foundation, Submission 136, p. 2.

55 *Committee Hansard,* 26 March 2021, p. 18.

56 Medicines Australia, Submission 141, p. 26

57 Medicines Australia, Submission 141, p. 26.

58 HealthMatch, Submission 201, p. 3.

> Recommend the implementation of a National Health Registry (NHR). This disease-agnostic registry will utilise a "general" OPT-IN consent for future participation in a wide variety of health conditions. Similar model to the Scottish Health Research Registry (SHARE).[59]

9.78 AstraZeneca continued and said it agreed with the recommendations made by Medicines Australia in its submission to the Senate Select Committee on COVID-19 in May 2020. Those recommendations suggested that Federal and State Governments work together with industry, through Medicines Australia and the Research and Development Taskforce (RDTF), to:

- Promote domestically and internationally that Australia is open for business to conduct clinical trials
- Embed clinical trials as part of the standard treatment of care in the national health infrastructure, including regionally through clinical tele-trials
- Harmonise ethics, governance and regulatory processes nationally for consistently faster and more efficient establishment of clinical trials across Australia, building on the proposed Front Door initiative and work underway through the Australian Commission on Safety and Quality in Health Care
- Strengthen the capacity to conduct clinical tele-trials in rural, remote and regional areas
- Develop nationally agreed clinical trials standards and guidance on:
  - tele-health
  - tele-trials
  - remote monitoring (including delivery and management of Investigational Medicinal Product)
  - the utilisation of digital technology, such as access to electronic Medical Records (eMR),
- e-signatures and e-consent
- Retain for the future, the more efficient changes to ethics, governance and regulatory measures implemented under COVID-19
- Linkage of existing Health care data, possibly via eHealth, forming an up-to-date National Health Registry. This would help to cement research as part of routine healthcare
- Introduction of a National Framework for Australian Trials: Inclusive of contractual negotiations and governance, reporting etc

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[59] AstraZeneca, Submission 42, p. 3.

- Introduction of a National Trials Centre with the purpose of supporting researchers with trial design and execution. A large part of the centre should be dedicated to participant recruitment.[60]

9.79 Roche suggested that it would be helpful if the Australian Government would assist with identifying where clinical trials are located, provide a valet style service to connect sponsors of trials with research organisations, and connect potential partners that can access harmonised patient registries. Further, Roche suggested the Australian Government should move quickly to:

- adopt and invest in technologies and associated practices to ensure all clinical trial centres (public hospitals) have Electronic Medical Records
- allow for remote monitoring of clinical trial participant records by sponsors
- establish national standards for the use of e-Consent in clinical trials
- adopt technologies for e-signatures on clinical trial documents.

This harmonisation should be coordinated through a new Clinical Trials Front Door agency that provides these services, collects metrics on clinical trials and assists with navigating with startups and patient transitions at the conclusion of the trial.[61]

9.80 The Committee heard evidence from The George Institute for Global Health, an independent global medical research institute, based in Sydney with major centres in China, India and the United Kingdom (UK). The George Institute along with George Health have developed a national research register called 'Join Us'.

9.81 Professor Bruce Neal, Executive Director, The George Institute for Global Health explained that the two key purposes of the Join Us register were to get people efficiently into clinical trials and to enable the efficient use of the collected health data. Professor Neal commented:

> The Join Us proposal is seeking a million Australians—any adult Australian over the age of 18—to go to the website and click the sign-up button and read an explanation of the project. They then provide basic contact details and agree to be contacted about studies that might be relevant to them. To make sure we send appropriate invitations, we also collect some basic checkbox data about medical conditions they might or might not have.

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60 AstraZeneca, Submission 42, pages 3-4.

61 Roche, Submission 92, pages 24 -25.

> In the second part of the sign-up, participants agree to allow Join Us to gather their routinely collected health data and store it for research purposes. At that point, any researcher anywhere in the country and at any institution seeking to answer any question can come to the Join Us register and ask for our assistance. We can identify participants who may be eligible for their study and, on behalf of the researchers, extend an invitation. This hugely simplifies the recruitment process because you've basically got a one-stop shop that is directly connected to hopefully a million people who might be interested in joining the research.[62]

9.82 In August 2021, the Department updated the Committee with the following information regarding the National Clinical Trials Front Door initiative.

> The Australian Commission on Safety and Quality in Health Care (ACSQHC) has been engaged to undertake national consultations on these initiatives. A project advisory group, chaired by Professor Ian Chubb, former Chief Scientist and clinical trial participant, will guide the consultation process.[63]

Australia New Zealand Clinical Trials Registry

9.83 The Australia New Zealand Clinical trials Registry (ANZCTR) was established in 2005 with $1.5 million in funding from the Australian Government, through a National Health & Medical Research Council Enabling Grant. The ANZCTR is overseen by an Advisory Committee with wide representation from a variety of stakeholders including government, clinicians, the research community, journal editors, the pharmaceutical industry and regulator, and consumers.

9.84 The ANZCTR receives funding from the Department, Therapeutic Innovation Australia, National Collaborative Research Infrastructure Strategy, and the Health Research Council of New Zealand.[64]

9.85 The ANZCTR is an online public registry of clinical trials, held at the NHMRC Clinical Trials Centre, University of Sydney. It is a Primary Registry in the World Health Organization (WHO) Registry Network, which means that it fulfils certain criteria for content, quality and validity, accessibility, unique identification, technical capacity and administration.

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62 *Committee Hansard*, Sydney, 7 May 2021, p. 50.

63 Department of Health, Submission 15.7, p. 2.

64 Department of Health, *Clinical Trial Register,* Canberra, www.anzctr.org.au/Faq.aspx#g1, viewed 28 July 2021.

Trials from all ICTRP Primary Registries can be searched at: www.who.int/trialsearch [65]

9.86 The ANZCTR accepts both interventional and observational studies for registration from all countries and from the full spectrum of therapeutic areas including trials of pharmaceuticals, surgical procedures, preventive measures, lifestyle, devices, treatment and rehabilitation strategies and complementary therapies.

9.87 Key points about the ANZCTR included that:

- It is publicly owned and managed by a not-for-profit organisation
- All details of trials registered on the ANZCTR are made publicly available
- Registration is voluntary, but if a registrant chooses to register a trial, certain fields are mandatory
- Registration is free of charge
- Responsibility for registration lies with the sponsor.[66]

9.88 The ANZCTR is part of the worldwide initiative to make public all clinical trials being conducted for the following reasons:

- To improve research transparency: Making details of all trials publicly available improves research transparency and helps to overcome publication bias and selective reporting, thereby enabling clinicians and consumers to make more informed decisions.
- To facilitate trial participation: People interested in participating in a clinical trial and doctors investigating relevant trials for their patients have access to a reputable and comprehensive on-line register showing what trials are occurring across all areas of health, which may facilitate recruitment.
- To avoid duplication: Improving awareness of similar or identical trials will make it possible for researchers and funding agencies to avoid unnecessary duplication.
- To identify potential research areas: Describing clinical trials in progress can make it easier to identify gaps in clinical trials research.

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65 Department of Health, *Clinical Trial Register*, Canberra, www.anzctr.org.au/Faq.aspx#g8, viewed 28 July 2021.

66 Department of Health, *Clinical Trial Register*, Canberra, www.anzctr.org.au/Faq.aspx#g8, viewed 28 July 2021.

- To promote research collaboration: Enabling researchers and health care practitioners to identify trials in which they may have an interest could result in more effective collaboration among researchers.
- To improve trial quality: Registries checking data as part of the registration process may lead to improvements in the quality of clinical trials by making it possible to identify potential problems (such as problematic randomisation methods) early in the research process.[67]

The clinicaltrialsNSW project

9.89 The Committee received evidence from the NSW Department of Health's Office for Health and Medical Research, that discussed the clinicaltrialsNSW project. The project acts as the front door for sponsors and researchers seeking to undertake clinical trials in NSW:

> clinicaltrialsNSW works across the sector to enable clinical trial capacity, capability and collaboration, and embed clinical trials in core hospital service delivery and clinical care. It has designed a continuous improvement agenda for clinical trials that develops the ecosystem across trial quality, operations, workforce and equity of access.[68]

Multi-centre trials

9.90 Multi-centre trials – those that are held over multiple sites and/or in multiple jurisdictions – are becoming more common as technologies improve. This is bringing more flexibility to conducting clinical trials and huge benefits to both patients and sponsors.

9.91 Medicines Australia discussed the importance of establishing clinical trials in regional areas in Australia. The benefits were discussed as being good for patients, good for research data and good for the regions in terms of upskilling the workforce in regional and remote areas:

> Decentralisation of clinical trials can increase patient diversity in clinical trials, allow faster recruitment to target and ultimately accelerate the development of new treatments. Importantly, it also strengthens the healthcare service in regional areas of the country by exposing doctors and other healthcare professionals to innovations in clinical practice and treatments. Through clinical tele-trials, smaller regional hospitals and clinics can be involved in

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67 Department of Health, *Clinical Trial Register*, Canberra, https://www.anzctr.org.au/Faq.aspx#g1, viewed 28 July 2021.

68 NSW Government, Submission 93, p. 14.

clinical trials by partnering with larger health service organisations via a hub and spoke model.[69]

9.92 Dr Kaustuv Bhattacharya, Scientific Adviser, RVA, suggested that rare disease would really benefit from multi-centre clinical trials set up all over Australia.

> Ideally, you would set up that kind of trial as a multicentre. You would recruit from all of Australia. In that sense, I agree with you that we should set up across the whole country in order to attract the numbers from across the whole country. We're automatically discriminated against by our population relative to, say, India or Brazil or other more populous countries.[70]

9.93 Noxopharm supported the need for more funding to enable the collaboration of multi-centre trials. This would involve allowing Australian hospitals and research networks to capitalise on overseas networks using a multi- centre trial for clinical trials conducted in Australia:

> One of the key challenges in conducting clinical trials in Australia is simply the low population. Recruiting sufficient trial participants, in a timely manner, to create a statistically meaningful study is challenging, especially for less common health conditions. There are a number of Australian hospitals that have clinical research networks outside of Australia, however smaller organisations do not have the resources and networks to readily tap into these groups.
>
> Government relationships and incentives to encourage these hospitals to leverage their networks to initiate multi-centre trials for Australian health technology innovations will enable the establishment of meaningful clinical trials that include an Australian population.[71]

9.94 Roche alerted the Committee to the following problem for multi-centre trials:

> Currently multiple ethics approvals are required for a clinical trial that is undertaken in more than one state or territory. A number of initiatives designed to implement the recommendations from the McKeon Review (2013) to harmonise and streamline the start-up of clinical trials are still continuing. Despite these initiatives, a range of public health policies do not allow national

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69 Medicines Australia, Submission 141, p. 27.

70 *Committee Hansard*, 11 March 2021, p. 6.

71 Noxopharm, Submission 70, p. 2.

certification by the National Health and Medical Research Council for multi-site trials.[72]

9.95 Novartis commented that Australia needs to better incentivise locally hosted clinical trials:

> There should be better incentives for companies to invest in conducting R&D in Australia and/or enrol Australian patients in multinational trials. Conducting multinational trials in Australia is an important way for Australian clinicians and patients to gain early access and experience with new therapies.[73]

Teletrials

9.96 The Department outlined progress has been made for teletrials across different jurisdictions.

> A National Teletrials Compendium was developed through effective cross-jurisdictional collaboration and funding from the Encouraging More Clinical Trials in Australia measure. The Compendium aligns with the minimum standards of the International Council for Harmonisation (ICH) Guideline for Good Clinical Practice (GCP) and the National Clinical Trials Governance Framework, and will support a consistent national approach. It is expected to contribute to growth in the number of teletrials in Australia, and pave the way more international teletrials and clinical trials in future.[74]

9.97 Research Australia noted the following issues could improve efficiencies in the recruitment and administration of clinical trials in Australia.

> The early lockdown in Australia disrupted research but also led to new innovations, including more 'remote' recruitment of participants for clinical trials. This innovation is complemented by a current initiative in Australia to normalise the use of electronic consent for participation in clinical trials, currently being led by CT:IQ.
>
> The COVID-19 pandemic has also accelerated the introduction of telehealth, 'normalising' remote engagement for patients and clinicians. This also provides an opportunity for clinical trials to expand the use of remote

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72 Roche, Submission 92, p. 24.

73 Novartis Pharmaceuticals, Submission 138, p. 8.

74 Department of Health, Submission 15.6, p. 29.

engagement with patients, potentially enabling more people, particularly in rural and remote Australia, to participate in clinical trials. [75]

Technology

9.98 Many submitters recommended that industry invests in technologies to utilise Electronic Medical records wherever possible and establish standards for the use of e-Consent and e-signatures.[76]

Data collection and reporting

9.99 Stakeholders sent a clear message to the Committee that modern health technology platforms to be used for data collection and reporting were critical to the success of attracting clinical trials to Australia.

9.100 Johnson and Johnson commented that health data is a critical element in delivering better health outcomes. It is vital in:

- determining the real value of new treatments and the terms and conditions upon which access to those treatments is secured
- driving the research and development of new treatments
- providing the most useful tools to help secure clinical trial investment in Australia
- to drive significant system efficiencies.[77]

9.101 The Monash Institute for Medical Engineering (MIME) made the following suggestion to make Australia a more attractive location for clinical trials:

> A critical gap for our clinical trials industry is the software platforms required for clinical trial management or recruitment cohort identification and efficient data study extraction from electronic health records.[78]

9.102 MIME continued and stated:

> The main gap in our national strategy to increase clinical trial activity is the application of digital innovations to:
>
> - maximise identification of patients and their recruitment, with suitable consent;

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75 Research Australia, Submission 78, p. 11.

76 AusBioTech, Submission 114, p. 10.

77 Johnson and Johnson, Submission 134, p. 18.

78 MIME, Submission 158, p. 4.

- ensure cost efficient recruitment to trials;
- efficiently capture date[data]; and
- project manage and maximise retention in trials.

Monash Partners Academic Health Science Centre, of whom MIME partners, is developing a digital, whole-of-country model to achieve these goals. It is being undertaken in partnership with the university, public health and patient advocacy communities and at a federal and state government level.[79]

9.103 Sydney Children's Hospital highlighted the need for improved health technology platforms:

> Enhanced support for information and health technology platform-development to increase diagnostic capacity and improve recruitment and triaging of patients to enable rapid access to emerging therapies via clinical trials or special access schemes. Emerging capabilities for data linkage, 'omics and organoids present an opportunity to transform the way we identify, diagnose and select patients that are likely to benefit from novel or repurposed therapeutic approaches.[80]

9.104 In a 2016 report released by the Department it said that limited research and data is available on clinical trial participation nationally, with the exception of oncology trials, and there is currently no central coordination point for the collection of clinical trials data, including recruitment and retention rates:

> In 2011, the Clinical Trials Action Group (CTAG) reported that 18,600 people were enrolled in 1,265 trials conducted in 2009. A 2014 survey conducted by Clinical Trials Connect (CTC) assessed the recruitment success for a range of clinical trials in Australia. The survey identified that 20% of trials met their recruitment deadline and the majority of respondents met their targets 50-79% of the time. Research and consultation also revealed limitations on data in relation to retention, with exact rates generally unpredictable.'[81]

9.105 Medicines Australia commented that whilst a national office for clinical trials had been considered frequently by government and industry, it was 'not sure that introducing another layer of regulatory process is necessarily the answer.'[82]

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79 MIME, Submission 158, p. 2.

80 Sydney Children's Hospital, Submission 185, p. 4.

81 Department of Health, *Scoping and analysis of issues in recruitment and retention in Australian clinical trials*, Final Report, June 2016, p. 3.

82 Ms de Somer, CEO, Medicines Australia, *Committee Hansard,* 26 March 2021, p. 4.

9.106 The Whitecoats Foundation was supportive of mandatory reporting requirements.

> Mandatory reporting related to clinical trial outcomes on a publically accessible register is essential to building and securing public trust and engagement with research. This will also help reduce research duplication and waste.[83]

9.107 Research Australia commented:

> At a Commonwealth Government level, Research Australia notes with approval the recent funding provided to the Therapeutic Goods Administration (TGA) to modernise its IT systems. This provides an opportunity to improve the provision of clinical trials data to the TGA and also the reporting of adverse events.
>
> There are currently multiple systems, specifications and standards used across Australia and internationally for the collection and reporting of health information, including for clinical trials reporting. While a single system might be unattainable, better harmonisation of systems and improvements in interoperability within Australia could provide significant efficiency benefits for Australian clinical trials as well as the health system more broadly. This could help make Australia a more attractive location for international clinical trials. [84]

9.108 Professor Bruce Neal from The George Institute for Global Health at UNSW commented that recruitment, particularly to clinical trials, is a major problem in Australia.

> … the processes for accessing routinely collected health data introduce major inefficiencies and waste in the research process, and also recognise that widespread implementation of Join Us has the potential to immediately provide an actionable solution to these problems.[85]

9.109 HealthMatch suggested that the Australian Government should leverage innovation in patient recruitment technologies in the private sector to support public good and explore partnerships to lessen the burden on taxpayer funded projects.[86]

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83 Whitecoats Foundation, Submission 136, p. 4.

84 Research Australia, Submission 78, p. 10.

85 *Committee Hansard*, Sydney, 7 May 2021, p. 50.

86 HealthMatch, Submission 201, p. 3.

9.110 In line with the McKell report released in 2016, the Committee was reminded of Recommendation 7 that stated:

> The Federal Government must design legislation, administrative processes and policies that will simplify the access to health data collections for medical research. The policies must maintain privacy and security.[87]

Marketing – education and awareness

9.111 The Western Australia (WA) Department of Health suggested Australia needs to invest in marketing itself better as a trial destination of choice.

> In WA, Linear Clinical Research has grown significantly over the last several years and currently attracts global studies to WA. Lessons learnt from companies such as Linear can contribute to the design of marketing approaches for other companies involved in the research and development of new drugs and novel technologies.[88]

9.112 PSA suggests greater investment in raising awareness about clinical trials with patients, families and the public is essential. This is not only to encourage patient participation in clinical trials but to generate conversations and improve understanding about the potential value of clinical trials more generally.

9.113 It is acknowledged that pharmacists are:

> 'a very accessible and trusted source of health information for the community' and therefore uniquely placed to support patients with clinical trials information.[89]

Infrastructure

9.114 Medical research and the development of new medicines, therapies and devices have expanded rapidly in recent years and countries are becoming competitive in terms of offering incentives to run clinical trials. The Australian Government has been investing in this area however infrastructure is an area of clinical trials that requires further consideration.

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87 The McKell Institute, *Big Data Big Possibilities How Australia can use big data for better health care,* p. 13. www.allens.com.au/globalassets/pdfs/sectors-services/healthcare/healthcare-mckellreport.pdf, viewed 27 August 2021.

88 Western Australia (WA) Department of Health, Submission 129, p. 5.

89 PSA, Submission 203, p. 3.

9.115 AAMRI commented that there are enormous growth opportunities for clinical trials in Australia:

> Combined with other key population advantages and a strong medical research sector there is room for expansion. However, expansion within some hospital and healthcare settings will need encouragement. Most large hospitals are already inundated with clinical trials and are not able to take up all opportunities because of space and capacity constraints.
>
> Work will need to be undertaken to grow the clinician researcher workforce, find funding solutions for sponsor monitoring, data management and research nurse costs, and crucially, provide space within hospitals.[90]

9.116 AusBioTech suggested examining opportunities to better leverage public hospital facilities and public health networks to better support clinical trials in areas of unmet need or priority need.[91]

9.117 The WA Department of Health proposed the following:

> To enable Australian companies to expand and attract clinical trials and global investment there needs to be suitable access to infrastructure such as manufacturing facilities and wet laboratories. [92]

9.118 Ms Nicole Millis, Chief Executive Officer, RVA suggested a lot more can be done to make Australia a more attractive and sustainable location for clinical trials:

> Australia needs … investment in clinical trial infrastructure, including registries, and also building up workforce and staffing capacity through the development of centres of excellence. We need the adoption of unique and more appropriate trial designs and to have these prioritised and recognised in HTA [Health Technology Assessment] processes. We also need to reduce the regulatory and bureaucratic burden of running clinical trials.[93]

Clinical trial networks

9.119 The Committee received evidence from the Australian Clinical Trials Alliance (ACTA), a national peak body representing Clinical Trial Networks (CTNs), Clinical Quality Registries (CQR) and Coordinating Centres (CCs).

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90 AAMRI, Submission 88, p. 7.

91 AusBioTech, Submission 114, p. 10.

92 WA Department of Health, Submission 129, p. 5; Roche, Submission 92, p. 24.

93 *Committee Hansard*, 11 March 2021, p. 2.

The CTNs represent groups of clinicians and researchers spanning a wide range of disease areas comprising over 10 000 clinical researchers. All of these trials are referred to as investigator-initiated trials (IITs).

9.120 ACTA highlighted the important role CTNs have in Australia's clinical trial sector:

> CTNs play a significant role in increasing clinical trial recruitment to address unanswered clinical questions. CTNs help to ensure that national, consumer and community priorities are met. Clinical trials require specialised skill sets. CTNs, and the coordinating centres that support them, strengthen the collaborative development of research proposals through extensive consultation processes involving internal peer review of study proposals to ensure scientific merit and rigour.[94]

9.121 ACTA discussed the important shared intellectual and virtual infrastructure that CTNs bring to clinical trials.

> Considerable efficiencies are created through the recruitment of specialists in trial design that build and share intellectual and virtual infrastructure across a longitudinal series of trials conducted within a network, making trial initiation easier, quicker and more cost-effective. Peer input and experienced coordinators generate a higher-quality trial design with a greater impact on patient outcomes and gross value generation.[95]

9.122 ACTA continued:

> Established CTNs possess critical trial infrastructure including access to a greater sample size through the collaboration of more sites, CTNs are ideally placed to conduct trials for orphan, personalised and off-patent drugs that could be repurposed and used to treat new conditions.[96]

9.123 ACTA works with the Department to increase the effectiveness of CTNs in Australia. It pointed out that more seed funding was required to get more CTNs started, such as an Indigenous Health CTN and it was emphasised that funding needs to be sustainable:

> Greater funding is needed for CTNs to support large-scale, investigator-led clinical research in Australia to generate evidence of comparative effectiveness and test innovative approaches. Networks need core infrastructure support.

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94 ACTA, Submission 149, pages 2-3.

95 ACTA, Submission 149, p. 3.

96 ACTA, Submission 149, p. 3.

> Infrastructure support will enable networks to be more efficient and sustainable in order to liaise with industry and facilitate further trials.[97]

Costs of clinical trials for sponsors

9.124 Several industry representatives commented that the different set up costs in Australia had increased over recent years. This has created a disincentive to bring clinical trials to this country.

9.125 Novartis Australia indicated that Australia had missed out on at least two studies, in part, due to high institutional and per-patient costs compared to similar top-tiered countries (such as the USA and Western Europe).

> Growing variation in Institutional costs of conducting clinical trials in Australia (including the addition of significant 'overheads' to already high study visit costs) may be contributing to Australia becoming less attractive as a destination for global clinical trials. Although Australia (understandably) sits in the top tiers of countries in terms of trial costs, Australian Institutions have been gradually increasing costs over recent years. This is pushing our trial costs well above comparable countries. Due to the resulting high per-patient costs, Novartis is seeing global decision makers capping enrolment from Australian sites.
>
> There have been two national initiatives in recent years to seek to standardise Institutional trial costs utilising the Independent Hospital Pricing Authority. Unfortunately these initiatives have not proved effective in reaching agreement on trial costs that are mutually accepted by Institution or sponsors. Sponsors should rightfully pay the cost of conducting global clinical studies in the Australian health system, however there should also be recognition of the significant benefits that global clinical trials accrue to patients, medical staff and the health system when institutions formulate clinical trials costings.[98]

9.126 AusBioTech added:

> Costs to conduct trials have similarities across institutions, although they must currently be separately negotiated with each institution. The IHPA developed a standardised framework for costing services in 20158F9, although this has not been widely adopted. The requirement to agree costs delays contracting and, ultimately, trial start up.[99]

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97 ACTA, Submission 149, p. 3.

98 Novartis Australia, Submission 138, p. 9.

99 AusBioTech, Submission 118, p. 12.

9.127 A study completed by the Department found that 'one of the key barriers to both Australian and international pharmaceutical companies looking to conduct trials in Australia was cost, noting that this country was more expensive than South-East Asia and Latin American sites.'[100]

Free drugs for lifetime

9.128 BioMarin discussed two barriers they believe exist when consideration is given to holding clinical trials in Australia. The first concern was about Australia not having a timely reimbursement process. The second concern was to do with sponsors being required to provide a continuation of free drugs to participants after the trial has finished:

> The challenge in obtaining timely reimbursement for new drugs and novel technologies is one of the greatest barriers to bringing clinical trials to Australia, because sponsors may be ethically required to provide free drug to participants for many years up to a lifetime if a viable pathway to funding does not exist.
>
> The consequences of challenging reimbursement conditions can be seen in the PHARMAC system, where significant delays and/or indefinite deferrals of medicine reimbursement have resulted in sponsors becoming extremely wary of investing in clinical trials in New Zealand.[101]

Cell and gene technology

9.129 The Committee heard evidence throughout the inquiry that cell and gene therapies were becoming an important and increasingly more common treatment for precision medicine. As a result, this has led to complex regulations to try to fit cell and gene therapies into Australia's current regulatory systems. This has created an untidy system of regulation that is time consuming to navigate and creates a barrier to investments in the clinical trials sector.

9.130 The Sydney Children's Hospital supported the need to streamline the processes for cell and gene technology:

> Streamlining gene technology licencing processes for investigators and industry. For many advanced therapeutics, clinical trial readiness relies on having adequate facilities (PC2 lab) and navigating complex regulatory processes. Some clinical trials of gene and cell therapies require a licence from

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100 MitoFoundation, Submission 125, p. 4.

101 BioMarin, Submission 152, p. 4.

> the Office of the Gene Technology Regulator under the Gene Technology Act 2000, and most licences require sponsor accreditation. This process can take in excess of 4 months which is currently a deterrent for Sponsors to bring gene therapy clinical trials to Australia.[102]

9.131 Pfizer highlighted the barriers that currently exist with clinical trials and cell and gene therapies, noting there are significant challenges in navigating the regulatory processes to set up a clinical trial for a gene therapy.

9.132 The complex regulatory environment acts as a hindrance to bringing clinical trials to Australia and providing patients early access to novel therapies. Pfizer's recent experience establishing clinical trials for fidanacogene elaparvovec is a prime example of this.

9.133 The Office of the Gene Technology Regulator (OGTR) is responsible for administering the *Gene Technology Act 2000* (Cth). Specifically, it regulates dealings with genetically modified organisms (GMOs) including dealings conducted in human clinical trials involving therapies that contain a GMO.[103]

9.134 Before commencing a clinical trial in Australia involving a GMO, an organisation must complete/obtain the following permissions:

- Accreditation (90 business days): The process of accreditation assists the Regulator in assessing if the organisation has the resources and the internal processes in place to enable it to effectively oversee work with GMOs. Once an organisation is accredited, in order to maintain the accreditation status, it must comply with a range of conditions on an ongoing basis. Note, the organisation is not required to apply/renew accreditation for each new clinical trial.
- Access to and partnership with an Institutional Biosafety Committee (IBC): Before an organisation can be accredited, it must have established, or have access to, an IBC. IBCs provide a quality assurance mechanism, providing advice to assist organisations with the identification and management of the risks associated with GMO dealings, including containment of GMOs. They also review an organisation's GMO licence application prior to submission to the Regulator to confirm that the information included is complete.
- Licence application for each individual GMO therapy: The DNIR licence application preparation is extensive involving 16 parts with more than 70 questions and a significant amount of work to complete. There is

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102 Sydney Children's Hospital, Submission 185, p. 4.

103 Pfizer Australia, Submission 137, pages [8-9].

limited opportunity to leverage existing work completed for trials run conducted in equivalent overseas markets so additional work is required to make information relevant for the Australian experience.

- Endorsement of the application by the IBC: Prior to submitting to the OGTR, the draft application must be reviewed and supported by an Institutional Biosafety Committee (IBC) for the purpose of ensuring that all of the necessary information is complete. In Pfizer's experience, the involvement of the IBC involves a significant amount of collaboration, with the process taking approximately 6 weeks to complete before the application form is finalised.
- OGTR evaluation timeline: Once the licence application has been submitted, the OGTR has a minimum legislated timeline of 90 business days to evaluate the application.
- Following approval of the DNIR licence the organisation must complete a Clinical Trial Notification to inform the Therapeutic Goods Administration (TGA). This process takes approximately 5 to 10 days to approve.
- An application must also be made to Human Research Ethics Committee (HREC), part of the National Health and Medical Research Council. This process takes approximately 30 business days.[104]

9.135 Pfizer Australia currently holds a DNIR licence for the purpose of running a trial for fidanacogene elaparvovec.[105]

9.136 This entire process involves close to nine months' work across at least three different national agencies. While we acknowledge that regulatory governance is critical to ensuring the maintenance of appropriate standards of quality, safety and efficacy, Pfizer commented that Australia can do more to expedite regulatory efficiency and support timely access to innovative and transformational therapies in the gene therapy space.[106]

9.137 Pfizer Australia suggested there were opportunities for streamlining the regulatory pathway for a clinical trial approval in this space. These include:

> clarifying and streamlining the partnership and governance responsibilities of the IBC and OGTR, particularly in the licence application processes and seeking approval for commercial supply, as well as creating opportunities to expedite regulatory review in Australia by leveraging overseas clinical trial

104 Pfizer Australia, Submission 137, pages [8-9].

105 Pfizer Australia, Submission 137, pages [8-9].

106 Pfizer Australia, Submission 137, pages [8-9].

applications and equivalent biosafety approvals delivered by equivalent regulatory markets overseas.[107]

Injury from clinical trials

9.138 Dr Diane Sheehan provided a submission that highlighted issues with the clinical trials system in Australia when Adverse Events (AE's) affect patients involved in clinical trials.

9.139 Dr Sheehan commented that there needs to be investigation into how AE's are dealt with including:

> more support for trial participants, improved clarity around where trial participants can get information and advocacy support, to be provided before, during the trial and increased to an appropriate level following any (AE).[108]

9.140 Further, Dr Sheehan proposed the following changes to clinical trials:

> It would be useful to have a systematic approach to sharing information with trial participants and giving them a voice and input when the trial is being set up. Plus a full discussion about the information shared with trial participants and a framework of protection and management set up around those involved in trials, this needs to be a practical extension of the "National Statement on Ethical Conduct in Human Research".[109]

9.141 Dr Sheehan pointed out that Australia's National Clinical Trials Governance Framework does not address any issues that patients may encounter such as Adverse Events and possible compensation. Dr Sheehan commented that it would be beneficial to wrap protection around clinical trial participants by including a framework that:

- Protects trial participants by setting up a regulatory environment
- Expands an Information and Communication strategy
- The use of advocates to manage participants and liaise with trial co-ordinators
- Support participants throughout the trial.[110]

The future

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107 Pfizer Australia, Submission 137, pages [8-9].

108 Dr Diane Sheehan, Submission 194, p. 2.

109 Dr Diane Sheehan, Submission 194, p. 2.

110 Dr Diane Sheehan, Submission 194, p. 3.

9.142 Australia's clinical trial sector has the potential for significant growth in the near future. The Committee heard evidence that Australia needed to make some changes to be ready for a surge in demand for novel medicines and devices in the clinical trial sector.

9.143 Medicines Australia alerted the Committee to this and encouraged the Australian Government to make some significant reforms to capitalise on the future demand for tier one countries carrying out clinical trials.

> Australia currently holds a strong international reputation as a location for high quality clinical trials. However, sustaining this reputation is increasingly challenging, as international competition for the placement of clinical trials has already begun to erode Australia's advantage. Rather than relying on historical recognition as a reliable destination for quality clinical research, Australia needs to actively demonstrate superiority against other international benchmarks in clinical trials, to secure status as a preferred destination of choice.[111]

9.144 Medicines Australia suggested clinical trials in Australia could 'expand clinical trial access to regional areas, [as this] would generate economic activity and support economic and health recovery.[112]

9.145 The Department acknowledged that they needed to improve the clinical trials system in relation to gene technology. Adjunct Professor John Skerritt, Deputy Secretary, Health Products Regulation, Department of Health commented:

> The final area where people felt that we needed to have clearer guidance, especially around clinical trial requirements and manufacturing standards, was medicines made through gene technology. We've commenced a targeted stakeholder consultation to identify those issues and to see if there's a need for regulatory changes or explaining the current system better.[113]

9.146 Professor Adam Jaffe, Member of Scientific and Medical Advisory Committee, RVA, discussed a gap in Australia's current clinical trials. Professor Jaffe is a specialist in rare diseases who deals with precision medicine and sees the landscape changing rapidly in this field of medicine. He described the 'need to understand novel clinical trial design, such as basket clinical trials or adaptive clinical trials, which are a relatively new

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111 Medicines Australia, Submission 141, p. 5.

112 Medicines Australia, Submission 141, p. 5.

113 *Committee Hansard*, Canberra, 18 June 2021, p. 17.

concept, and understanding the use of technology and embedding them in precision medicine.'[114] Professor Jaffe went on to state:

> For example, in cystic fibrosis, there is the use of tissue organoids as precision medicine to project clinical responses. It's about ensuring that there's equity of access for patients with rare and ultra-rare mutations. …
>
> These are bits of tissues that we take from children. We can have them in a test tube and we can test them against new treatments. For example, we have a child with a rare mutation who would respond to this drug, but we'll never get access through the current process.[115]

9.147 Professor Jaffe argued against using this new approach for some clinical trials as he stated it is unethical to take a child off a medication:

> In fact, some trial designs have shown that, if you take them off a medication for a washout period to start on a new drug, their lung function starts declining. We need to look at new paradigms of how we do clinical trials. Hence my point earlier that we should be looking at adaptive trials. We should be using precision medicine. In our case, we use organoids.
>
> If we can develop a companion diagnostic that gives regulators, physicians and patients confidence—'We have tested your child in a test tube and we can predict that your child should not take this medication,' because they won't respond to, say, Orkambi, but they will respond to Trikafta. That saves the taxpayer $300,000, it saves the child from any potential side effects, but it also gives you the opportunity to make sure that the child gets on a better drug, because 'We have evidence in our test tube that a drug not available in Australia is better than a currently available drug in Australia. It's probably available in Europe.' We need to embrace new technologies.
>
> You need a body that could actually understand the change and could advise the TGA, the PBAC and MSAC. That's the gap that's missing, and a precision medicine advisory committee or group would fill that gap.[116]

9.148 Medicines Australia advised that to remain a world leader in the delivery of clinical trials, and to attract more clinical trials to Australia, we must be able to:

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114 *Committee Hansard*, 11 March 2021, p. 3.

115 *Committee Hansard*, 11 March 2021, p. 3.

116 *Committee Hansard*, 11 March 2021, p. 3.

- Commence trials quickly and in a consistent, harmonised, and efficient manner across multiple centres around Australia
- Increase the ability for patients to participate in clinical trials. In particular, ensure there is wide recognition and equitable access to clinical trials for patients located in regional areas, through building tele-trials capabilities. This will ensure that clinical trials recruitment is similar to, or greater than that seen in other countries
- Adopt modern and future-ready technologies to enable clinical trial processes to be conducted efficiently, cost-effectively, and where possible, remotely.[117]

Committee Comment

9.149 An important challenge for the Australian Government is the continuation and enhancement of Australia's excellent reputation as a tier one country for clinical trials. The way forward needs to be considered carefully to maximise the advantages that clinical trials bring to the Australian healthcare sector and the economy.

9.150 Australia's regional neighbours are becoming more competitive on an international scale and could potentially be chosen over Australia to conduct clinical trials now and in the future. The Committee believes Australia needs to strengthen its position by streamlining regulations in ethics and governance as a matter of urgency to ensure Australia remains a tier one nation for clinical trials.

9.151 The Committee understands that a lot of good work has been done over the past few years to make changes to ethics committees harmonisation between different jurisdictions. However it is imperative that the Australian Government act now to implement a seamless ethics and governance system. This important change will facilitate a regulatory process for sponsors that is easy to navigate and guarantee timeframes that are competitive with overseas countries.

9.152 The current work being undertaken by the Australian Commission on Safety and Quality in Health Care (ACSQHC) must finalise an agreement between all jurisdictions for the National Mutual Acceptance Scheme to harmonise ethics approvals within all jurisdictions. Governance requirements must be streamlined into a simplified national system.

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117 Medicines Australia, Submission 141, p. 25.

9.153 Equally, the Committee believes the infrastructure needs to keep pace with advances in medicines and technologies to ensure the clinical trials can be run successfully in Australia. This includes ensuring there is sufficient space within hospitals to conduct clinical trials, along with highly trained staff and optimal technology.

9.154 The Committee believes an Australian national clinical trial register is critical for Australia to attract sponsors to conduct clinical trials here. Sponsors currently find Australia attractive for clinical trials due to our diverse population and a highly skilled workforce. Australia will become even more attractive to sponsors if it has a well-maintained database of patients registered for different diseases, which will have a beneficial effect on medical research. A national registry should use state-of-the-art technology so that relevant data can be sourced from the My Health e-records of individual patients.

9.155 The Australian Government should consider doing more to encourage the participation of Aboriginal and Torres Strait Islander populations in clinical trials. This should be taken into consideration when developing the national clinical trial register. The Australian Government should provide seed funding for Indigenous Health Clinical Trial Networks. In addition, the Committee sees benefit in providing Clinical Trial Networks with medium to long term funding to strengthen research findings, improve data management systems and connect with industry to facilitate further trials.

9.156 Multi-centre trials are becoming more common in Australia. To further support them, it is imperative that there is more investment by the Australian Government in technological platforms, infrastructure, and education and training. Encouraging and supporting multi-centre trials is critical given Australia's widely dispersed population and sometimes large distances between hospitals. Australia must ensure it has the infrastructure to support multi-centre trials. Further investment will not only attract foreign sponsors but will benefit rural, regional and remote patients wanting to participate in clinical trials.

9.157 The Committee is of the view that the collection of data and the reporting of clinical trials should be improved. The Australia Government should investigate the costs that are currently being charged for clinical trials and benchmark them against other countries. The Committee believes that Australia needs to make itself as internationally competitive as possible and this includes keeping costs of running clinical trials at a reasonable level. The

Committee sees merit in standardising the costs for clinical trials throughout Australia.

9.158 Cell and gene therapy is a new field of medicine that is expected to increase significantly with new innovation and discoveries for medical therapies and devices. The clinical trials sector in Australia needs to be examined closely to streamline processes that will facilitate cell and gene therapy trials. The Committee believes this is a high priority and that it needs further examination to better integrate the clinical trials system with the Health Technology Assessment (HTA) process.

9.159 The Committee believes Australia's HTA systems need to become more flexible in terms of relying on evidence by way of head-to-head clinical trials for new medicines, therapies and medical devices. With the benefit of horizon scanning, Australia can adapt our approval systems early on to maximise the benefits of new medical technologies.

10. Research and Development

Overview

10.1 Ongoing advances in medical research and development (R&D) are generating important new therapies and novel health technologies worldwide. These advances can target disease and lead to improved patient and survival outcomes. For patients, innovative medicines and advanced technologies can improve quality of life, relieve symptoms, delay disease, prevent health related complications, increase life expectancy and, in some cases, cure the disease.

10.2 The journey from initial research discovery to a new drug, therapy or technology is a long one. In the classic model, the pharmaceutical company is responsible for development of a drug, and its testing and commercialisation. The significant investment costs are recouped from the profits from the product's subsequent sale.

10.3 International and domestic partnerships for research and development are becoming increasingly important. The COVID-19 pandemic and the global response to it has reinforced this notion that a global problem needs a globally supported solution.

10.4 Australia is already playing a valuable role in a growing global network of research and development in medical science and technology. Australia will need to continue to build upon its strengths in research and development to remain internationally competitive and attractive.

10.5 Global companies make decisions on investing in research and development in countries that have: a stable economy; streamlined laws and regulations; high returns; a highly skilled workforce and investments that are potentially scalable in other countries.

10.6 This chapter examines the research and development initiatives that the Australian Government has in place, including Commonwealth funding grants, tax incentives, intellectual property laws and protections. The chapter also considers the merits of horizon scanning, what it is and what is needed to strengthen the future of research and development in Australia.

Australian Government funding initiatives

10.7 The investment in supporting innovation, research and commercialisation is a state and national responsibility. Ongoing federal and state government support of growth and development across medical technologies, digital health, biotechnologies and the pharmaceutical sector remains critical for the future growth of this sector.

10.8 The Australian Government funds two main programs, the Medical Research Future Fund (MRFF) and the National Health and Medical Research Council (NHMRC).There are other research and development programs including programs funded by the Department of Education Australian Research Council (ARC) funded programs and Research Support Program; and programs provided by the Department of Industry, Science, Energy and Resources.[1]

Medical Research Future Fund

10.9 The Medical Research Future Fund (MRFF) was established under the *Medical Research Future Fund Act 2015 (Cth),* providing long-term sustainable funding for Australian health and medical research with an aim to improve health outcomes, quality of life and health system sustainability. The MRFF reached maturity at $20 billion.

10.10 The net interest from the fund is available to health and medical researchers across a range of 20 initiatives under four themes: patients, researchers, research missions and research translation. The Australian Government's $5 billion, 10-year investment plan for the MRFF, announced in the 2019- 20 Federal Budget, outlined how health and medical research would be funded over the next decade. Under the MRFF the Australian Government has funded the following different initiatives totalling over $1 billion.

- The Frontier Health and Medical Research initiative is a program that will provide $570 million over 10 years to enable researcher collaborations to explore bold, innovative ideas and/or make discoveries

[1] Research Australia, Submission 78, p. 8.

of great potential and global impact, through research relevant to any area of healthcare.

- The Genomics Health Futures Mission has been allocated $500 million from the MRFF over ten years and aims to improve the lives of Australians by accelerating research that delivers more effective testing, diagnosis and treatment; facilitates the adoption of new interventions; and consolidates Australia's international leadership in genomics.
- The Million Minds Mental Health Mission aims to support a million Australians with mental health issues access new approaches to prevention, diagnosis, treatment and recovery. The MRFF has allocated $125 million over 10 years for this mission.
- The Global Health initiative will invest $28.4 million over 10 years to fund projects on understanding global health threats, including tackling antimicrobial resistance and drug-resistant tuberculosis.
- The Indigenous Health Research Fund is investing in Indigenous-led research to tackle health issues facing Aboriginal and Torres Strait Islander people. It will provide $160 million over 10 years.
- The Stem Cell Therapies Mission will invest $150 million as part of the 10-year plan, to develop innovative, safe and effective treatments and to translate stem cell innovations into commercial products.
- $5.9 million has been allocated to eight projects to find new innovative treatments for diseases. The Cardiovascular Health Mission will invest $220 million over 10 years to make transformative improvements in heart and vascular health, and stroke for all Australians.
- The Medical Research Commercialisation initiative aims to support early-stage health and medical research and innovation in Australia through to proof-of-concept and beyond, providing opportunities for commercialisation. It will provide $311 million over 10 years.
  - Two programs have been established under the Medical Research Commercialisation initiative to date – BioMedTech Horizons and Biomedical Translation Bridge.
- The Biomedical Translation Fund (BTF) is a $500 million equity co-investment venture capital program to support the development of biomedical ventures in Australia. The BTF aims to help translate biomedical discoveries into high growth potential companies that are improving long term health benefits and national economic outcomes.[2]

[2] Department of Health, Submission 15, pages 18-19.

10.11 Recently, as part of the Government's Coronavirus Research Response, $95 million from the MRFF has been invested in COVID-19 research including for diagnostics, vaccine development, antiviral development, clinical trials, digital health research infrastructure, studies on the human immune response to COVID-19 infection, community information needs and behavioural responses during outbreaks.[3]

National Health and Medical Research Council funding

10.12 The National Health and Medical Research Council (NHMRC) is the Australian Government's main health and medical research funding body. NHMRC supports excellence in research that meets the health needs of Australians, from basic science through to clinical, public health and health services research and research that reflects national, state and territory and community priorities. One of NHMRC's key objectives is to support the translation of health and medical research into better health outcomes. [4]

10.13 NHMRC's Ideas Grant scheme funds innovative and creative research in any area of health and medical science from discovery to implementation. This scheme focuses on funding research that aims to challenge and shift current paradigms and/or have a major impact on a health research area through one or more studies that creatively develop novel concepts, improve applications or integrate technologies for a new purpose. In 2019, NHMRC funded 293 Ideas Grants to a total value of approximately $240 million.[5]

10.14 NHMRC's Development Grants scheme supports the commercial development of a product, process, procedure or service that, if applied, would result in improved health care, disease prevention or provide health cost savings. In 2019, NHMRC funded 31 of these grants to a total value of approximately $75 million.[6]

Future funding challenges

10.15 The Association of Australian Medical Research Institutes (AAMRI) commented that 'continued strong investment by the Australian Government in all stages of health and medical research will be needed to

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3 Department of Health, Submission 15, pages 20-21.

4 Department of Health, Submission 15, p. 17.

5 Department of Health, Submission 15, p. 17.

6 Department of Health, Submission 15, p. 17.

respond to the future health challenges facing the nation and to ensure Australia remains a world-class medical research nation. '[7]

10.16 AAMRI added that Australia has the potential to be a world leader in medical research in the decades to come and emphasised that:

> … a more strategic approach will be needed to ensure the right level of investment is being made at the different stages of the research pipeline.[8]

10.17 Save of Sons Duchenne Foundation (SOSDF) commented that university research was a critical component in the fight to find a cure for rare diseases.

10.18 The SOSDF were pleased the Australian Government provided $1billion for the university research community in the 2020-21 Federal Budget. In addition funding included the following:

- $2 billion and other revisions to the Research and Development Tax Incentive, and
- $1.3 billion in the modern manufacturing strategy, of which medicines manufacturing has been identified as a key industry.[9]

10.19 SOSDF was disappointed to note that NHMRC funding is declining in real terms:

> … the largely unchanged budgets for the NHMRC and MRFF are disappointing with Research Australia highlighting the fact that researchers are dealing with extra costs due to the delays and disruptions caused by COVID-19. Further, that there is no research program support for researchers in Medical Research Institutes, and NHMRC funding continues to decline in real terms.[10]

10.20 Research Australia suggested that a more coordinated view had to be taken in order to commercialise more drugs and novel medical technologies where there is an unmet need. Research Australia commented :

> … the programs need to be reviewed to ensure that the guidelines better support the research and commercialisation process.[11]

10.21 Research Australia added that:

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7 AAMRI, Submission 88, p. 5.

8 AAMRI, Submission 88, p. 5.

9 SOSDF, Submission 33, p. 19.

10 SOSDF, Submission 33, p. 20.

11 Research Australia, Submission 78, p. 9.

> … this requires an 'end to end' review, identifying what funding and incentives currently exist and where the gaps are. Consideration then needs to be given to what action can be taken to closing these gaps, including modifying existing programs to better support commercialisation, and providing a more streamlined progression through the pipeline for research that has commercialisation potential.[12]

10.22 The Australian Cardiovascular Alliance (ACvA) proposed a review:

> Undertaking an "outcomes review" of recent NHMRC grant activity to test linkages between research awards and translational pathways into clinical guidance and practice.[13]

10.23 The Medical Technology Association of Australia (MTAA) stated it 'strongly supports the focus of the MRFF on long term outcomes and this should receive ongoing support and not be subject to continuous change.[14]

10.24 Stryker echoed this sentiment stating that some of the challenges for the health sector include:

- A lack of continuity in funding for research and innovation due to the short political cycle leading to frequent changes in policy direction.
- Fragmented and inconsistent support for industry and researchers via government tax incentives, funding schemes and research grants programs. [15]

10.25 Similarly, the Centre for Law and Genetics highlighted the funding challenge for research and emphasised that the lack of continuous funding was a barrier to translational development:

> Many referenced the fact that their research existed 'grant to grant', and that many innovative products that have clinical potential fall at the translational hurdle due to lack of industry funding.[16]

10.26 MTAA described the challenge of Australia not being large enough to compete with itself to develop the next generation of innovation:

> We particularly welcome the end-to-end model of national collaboration championed by the Australian Cardiovascular Alliance, which has the breadth

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12 Research Australia, Submission 78, p. 9.

13 ACvA, Submission 76, p. 10.

14 Medical Technology Association Australia (MTAA), Submission 148, p. 28.

15 Stryker, Submission 28, p. 9.

16 Centre for Law and Genetics, Submission 179, p. 6.

> of expertise and commitment to consider all phases of technology development and to determine which gaps need to be filled. This is a model that should be encouraged in other disease states, potentially through linkage grants.[17]

10.27 MTAA added that the Australian Government may wish to consider funding 'no fault' investigations of past grants and programs as a critical review of these unsuccessful past programs may reveal some hidden gems.[18]

10.28 Stryker suggested that 'A MedTech "green paper" should be developed by the government in collaboration with industry and other stakeholders to canvas options for strengthening the MedTech sector through supporting the development of a sovereign MedTech research, development and manufacturing sector focussed on the commercialisation of new products and technologies which improve health outcomes, create high-skill jobs, attract international investment and deliver significant health and economic benefits.'[19]

10.29 The ACvA suggested the Government should consider integrating funding between different industry schemes:

> Innovative research through strong funding support across the whole research pipeline, that can bridge health and economic imperatives should be supported and mechanisms to integrate across the NHMRC, MRFF and TTRA should be developed and implemented. Small to medium biotech companies should also be better supported in Australia.[20]

10.30 The National Aboriginal Community Controlled Health Organisation (NACCHO) suggested that the Government establish 'a research fund specifically related to Aboriginal and Torres Strait Islander people's access to medicines.'[21]

Research incentives

10.31 Medicines Australia described how Australia needs to 'reassert a place at the forefront of major innovation for pharmaceutical discoveries. Reinforcing

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17 MTAA, Submission 148, p. 28.

18 MTAA, Submission 148, p. 28.

19 Stryker, Submission 28, pages 9-10.

20 ACvA, Submission 76, p. 4.

21 NACCHO, Submission 190, p. 5.

our existing strengths, that underpin Australia's capacity and capability in research and development (R&D), and improving policy vulnerabilities around intellectual property (IP), will be key to securing the early discovery and pipeline development for new, innovative, and advanced therapies in Australia.'[22]

10.32 AusBiotech told the Committee that the biotechnology industry can play a significant role in our country's economic future and healthcare, particularly when areas of innovation and opportunity are supported through the right policy levers and incentives.[23]

10.33 Johnson & Johnson suggested that the successful research, development and commercialisation of new drugs and novel medical technology requires several key elements to create an eco-system for innovation. These include:

> … quality scientific infrastructure, great research, a willingness to collaborate, a robust, respected and enforced intellectual property framework, an entrepreneurial mindset and a competitive funding base that is stable, consistent and knowledgeable. If one or more of those elements is missing, the chances of turning a great idea into useful medical innovation is significantly diminished.[24]

10.34 Many stakeholders shared views on the importance of Government funding of the research and development 'pipeline' at critical points from end to end as well as ensuring the tax incentives remain stable.

Research and development tax initiatives

10.35 The Research and Development Tax Incentive (RDTI) helps companies innovate and grow by offsetting some of the costs of eligible R&D. The Committee noted that pharmaceutical companies invested around a quarter of their revenue per year in R&D.[25]

10.36 The Committee did not go into the detail of RDTIs however it was aware that the 2020-21 Federal Budget introduced new RDTIs, effective 1 July 2021.

10.37 AusBiotech emphasised the importance of stability and predictability of the innovation environment. It commented that 'the US system of R&D

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22 Medicines Australia, Submission 141, p. 5.

23 AusBiotech, Submission 114, p. 7.

24 Johnson and Johnson, Submission 134, p. 15.

25 Mr Benjamin Basil, General Manager Australia, Eli Lilly, *Committee Hansard*, Sydney, 7 May 2021, p. 27.

incentives has remained essentially unchanged for the past 30 years. In contrast, Australia has made substantive changes, on average, every five years.'[26]

10.38 Mr Ian Burgess, Chief Executive Officer, MTAA admitted there had been concerns from industry about potential changes to the RDTIs.

> Together with other organisations such as AusBiotech and Medicines Australia, we were concerned about the cutbacks that had been proposed. We consider it to be a very important program to provide assistance for local R&D. [27]

10.39 Throughout the inquiry, the Committee realised there was broad agreement from industry that the retention of the RDTIs were very welcome.[28]

10.40 Research Australia welcomed the Government's decision announced in the 2020-21 Federal Budget that will reverse many of the changes it had proposed to the RDTI Scheme. The RDTI Scheme provides an incentive for further commercial investment, and the removal of the caps in particular could support the significant investment required for Phase 2 and Phase 3 clinical trials to be undertaken on a commercial basis in Australia.[29]

10.41 AstraZeneca echoed its support for the 'continuation and strengthening of the existing R&D tax incentive for small Biotech companies.' [30]

10.42 Professor Ian Alexander, Head, Gene Therapy Unit, Children's Medical Research Institute provided the Committee with an example of the success of tax incentives.

> … at least one of the companies that has engaged with the CMRI for development of vector technology, set up Australian subsidiaries that were incentivised by tax incentives. So certainly making Australia attractive as a place to come and spend research dollars is very important.[31]

10.43 At the public hearing, Professor Mackay, QIMR Berhofer Medical Research Institute (QIMR Berhofer), at a public hearing encouraged the Committee to

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26 AusBiotech, Submission 114, p. 5.

27 *Committee Hansard,* Sydney, 11 March 2021, p. 16.

28 Mr Al-ouf Ashraf, Bayer Australia, *Committee Hansard,* Sydney, 7 May 2021, p. 22; Professor Mackay, QIMR, p.23; AusBiotech, Submission 114, p. 8; ACvA, Submission 76, p. 8.

29 Research Australia, Submission 78, p. 9.

30 AstraZeneca, Submission 42, p. 3.

31 *Committee Hansard*, Sydney, 12 March 2021, p. 25.

consider the importance of really promoting Australia's research and development tax incentives.[32]

10.44 Medicines Australia added that the recently adapted RDTIs should be evaluated 'to ensure that barriers have not been inadvertently introduced and that local and global pharmaceutical companies continue to invest in clinical trials within Australia, as this investment can be a key driver of economic growth.'[33]

Taxation on Intellectual Property

10.45 A patent is a legally enforceable right for a device, substance, method or process. [34] A standard patent provides long-term protection and control over an invention. It lasts for up to 20 years from the filing date of the application and up to 25 years for pharmaceutical substances.[35]

10.46 The Centre for Law and Genetics commented that Intellectual Property (IP) is emerging as a major factor in whether research leads to successful outcomes. It described some of the challenges that are associated with IP in Australia:

- difficulty in funding IP protection (particularly patents) and the fact that frequently, specific funding schemes need to be targeted to provide funding to allow a patent application; a lack of institutional knowledge about what is required to seek patent protection, and the fact that a focus on commercialisation necessarily impedes research outcomes, because it restricts researchers from publishing. This was viewed as a real catch-22, and a number of researchers had been 'caught out' by disclosing their research prematurely.
- On the other side of the coin, gaining an understanding of the patent landscape in which researchers are operating (and must invent around), is often difficult, time-consuming, technical and costly. Researchers often lacked knowledge of the market in which they anticipated their research will have an impact.[36]

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32 Professor McKay, QIMR, 17 May 2021, p. 23.

33 Medicines Australia, Submission 141, p. 6.

34 Department of Industry, Science, Energy and Resources, Canberra, www.business.gov.au/planning/protect-your-brand-idea-or-creation/patents, viewed 8 September 2021.

35 IP Australia, Canberra, www.ipaustralia.gov.au/patents/understanding-patents/types-patents, viewed 8 September 2021.

36 Centre for Law and Genetics, Submission 179, p. 7.

10.47 MTAA outlined one of the problems it has had with IP in Australia including the translation of research into manufacturing. In its submission MTAA commented:

> Currently, the Commonwealth, via the R&DTI, the MRRF, and NHMRC, spends more than $3B p.a. to support medical breakthroughs. However, the process halts as there are currently no incentives for onshore commercialisation of the resulting intellectual property. In effect, this is leading to the exportation of this IP just as it is beginning to become profitable and deliver value to the Australian economy. The exact cost to the Government could only be calculated once the specific parameters of this policy are set.[37]

10.48 Dr Bruce Arnold, during a public hearing, was emphatic about not needing to extend IP protection. He informed the Committee he does not support extending the IP protection for pharmaceutical companies. He commented that 'what you're getting by extending protection beyond the existing period is simply rent-seeking.'[38]

10.49 Dr Arnold added 'there is no justification for extending the period of patent protection for pharmaceuticals and medical devices to incentivise research and development.'[39]

10.50 Dr Arnold highlighted the following issue with patents:

> There are fuzzy indications of development through analyses of patenting (indicating that the bulk of patents are by/for overseas life sciences corporations rather than Australian-owned entities) and grants made by the NHMRC.[40]

10.51 The majority of stakeholders did not have an issue with the IP laws in Australia other than stating the need to review them with a view to aim for a consistent approach between states and territories. The Committee heard from the Western Australian Department of Health who are in the process of finalising a new IP Policy for the WA health system:

> Consistent practice (legal documentation, governance and incentives for inventors) across the Health Service Providers, and in collaboration with other

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37 MTAA, Submission 148, p. 27.

38 *Committee Hansard*, Canberra, 26 March 2021, p. 23.

39 Dr Arnold and Dr Bonython, Submission 49, p. 4.

40 Dr Arnold and Dr Bonython, Submission 49, p. 9.

> institutions, are required for more effective translation of Department of Health research and innovation.[41]

The 'Patent Box' tax scheme

10.52 The 'Patent Box' scheme also known around the world as the 'Knowledge Development Box', was foreshadowed by the Government in the 2021-22 Federal Budget to encourage investment in Australia's biotechnology and medical technology sectors. From 1 July 2022, income derived from Australian medical and biotech patents will be taxed at a concessional corporate tax rate of 17%.[42] This is lower than the standard corporate income tax rate of 30%, or 25% for small and medium companies.[43]

10.53 In submissions received by the Committee prior to the above announcement, several submitters including the Australian Cardiovascular Alliance suggested that a 'A Knowledge Development Box (KDB, also known as a patent box) should be implemented and funded.'[44]

10.54 The Australian Cardiovascular Alliance at a public hearing, described the Patent Box tax scheme:

> A patent box tax scheme allows the rate of company tax levied on income generated from qualifying patents to be effectively reduced. This would mean that an owner or licensee of a granted patent could obtain tax relief at a significantly reduced rate when compared to the normal company tax rates in the country within which the patentable invention is to be worked.
>
> The Irish government introduced the world's first OECD compliant patent box regime- the Knowledge Development Box (KDB). The KDB regime offers a 6.25% effective tax rate for profits arising from patents and copyrighted software. In the UK, their patent box offers a 10% effective tax rate. [45]

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41 WA Department of Health, Submission 129, p. 3.

42 The concessional tax rate will only be available for income years from 1 July 2022. To be eligible for the concessional tax rate, the patent in question must be granted, and must have been filed after the Budget announcement on 11 May 2021.

43 Australian Taxation Office, Canberra, www.ato.gov.au/General/New-legislation/In-detail/Direct-taxes/Income-tax-for-businesses/Patent-Box---tax-concession-for-Australian-medical-and-biotechnology-innovations/ viewed 8 September 2021.

44 ACVA, Submission 76, p. 4; ACvA, Submission 76, p. 4; MTAA, Submission 148, p. 5; BioScience Managers, Submission 206, p. 1.

45 ACvA, Submission 76, p. 8.

10.55 The Australian Government hopes that this new incentive will encourage additional investment in Australia's biotechnology and medical technology sectors, and encourage companies to develop and apply their innovations in Australia.[46]

10.56 Industry was overwhelmingly supportive of the introduction of the Patent Box tax scheme. During a public hearing held after the Budget, Ms Lorraine Chiroiu, CEO, AusBiotech, commented:

> The federal government last week pledged a patent box, and AusBiotech warmly welcomes this. A lower tax rate for revenues derived from IP developed here creates an incentive to stay in Australia, as the benefits from manufacturing pay back into the economy. It impacts companies at a different stage but dovetails with the R&D tax incentive as its support diminishes. The key is that it helps create end-to-end motivation, bridging over extensive investments. Keeping the benefits of home grown, often publicly funded IP in Australia will enable Australia to economically and societally benefit and incentivise the associated manufacturing to stay here and boost our sovereign capability.[47]

10.57 Pfizer Australia emphasised the importance of a strong patent system:

> An intellectual property (IP) policy environment that includes, for example, a strong patent system and regulatory data protection, is critical to incentivize and drive the extensive investments and risks involved in the development of innovative medicines. A country's record on intellectual property is an influential factor when determining long-term investment decisions that drive local employment and patient access to breakthrough medicines.
>
> A strong and effective IP system is of significant importance to the biopharmaceutical industry, where on average it takes at least 10-15 years to bring a medicine from drug discovery through approval to market. The term of patent protection is 20 years from the filing date, subject to extension in limited circumstances, leaving approximately 5-10 years for innovative companies to recover the extensive research and development investments and fuel the next generation of breakthrough therapies. [48]

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46 Australian Taxation Office, Canberra, www.ato.gov.au/General/New-legislation/In-detail/Direct-taxes/Income-tax-for-businesses/Patent-Box---tax-concession-for-Australian-medical-and-biotechnology-innovations/ viewed 8 September 2021.

47 *Committee Hansard*, Brisbane, 18 May 2021, p. 22.

48 Pfizer Australia, Submission 137, p. 6.

10.58 Noxopharm described an issue they have encountered in relation to the high costs of IP protection:

> There are several grants in Australia that leverage both public and private funding. Part of this process is negotiating the proportional ownership of shared IP generated by the project. Access to government assistance to fund legal support for smaller MedTech companies to go into negotiations with large academic institutions would reduce the risk and expense of submitting applications for grants.[49]

10.59 Dr Merrilyn Clancy, MTAA, described the benefits of having a Patent Box in Australia.

> We currently have a 30 per cent tax rate for corporate tax. Obviously, five per cent sounds much better, so 'I don't want to go and make my things in Singapore, but it's so attractive and there are so many things. It just sounds easier to do that there.' One response from the government has been: 'That could be rorted. We're not going to give you carte blanche to have such a big tax reduction.' But the result is that you wouldn't get any tax income. Thirty per cent of zero is zero. Young manufacturers, young developers and large corporations who want to bring small enterprises here are looking at using Australia as a place to grow their business. Why? Because we have excellent safety, we have a quality workforce, and we have a system that really is very well set up to do that. But there are some key gaps in which we can do better.[50]

Data exclusivity

10.60 In Australia, the *Therapeutic Goods Amendment Act 1998 (Cth) (the Act)* established a five year data exclusivity period for new products containing pharmaceutical actives approved after 17 April 1998. The data exclusivity period begins on the date of marketing approval.[51]

10.61 Data exclusivity is only provided to new active components that have never been included in the Australian Register of Therapeutic Goods. Therefore, data exclusivity is not provided for new uses or new formulations of existing compounds.[52]

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49 Noxopharm, Submission 70, p. 2.

50 *Committee Hansard*, 11 March 2021, p. 14.

51 www.findlaw.com.au/articles/1576/data-exclusivity-further-protection-for-pharmaceut.aspx viewed 9 September 2021.

52 www.findlaw.com.au/articles/1576/data-exclusivity-further-protection-for-pharmaceut.aspx viewed 9 September 2021.

Table 10.1 Data exclusivity laws around the world

Country	Data exclusivity in years
Australia	5
US	Up to 5
European Union	6-10
New Zealand	5
Japan	4-10
China	6

Source: findlaw.com.au (https://www.findlaw.com.au/articles/1576/data-exclusivity-further-protection-for-pharmaceut.aspx)

10.62 The United States only provides three years of data exclusivity for new indications of an existing drug. The European Union (EU) varies due to differing national registrations, however it is considering harmonising protection to 10 years for all EU countries. Interestingly, Japan's exclusivity period varies from four years for new indications or formulations of a drug to six years for drugs containing new chemical entities, and up to 10 years for orphan drugs.[53]

10.63 Medicines Australia suggested that Australia should compete in the global race for investments in this area of data exclusivity:

> There is an opportunity, including through the current free trade agreement negotiations with the United Kingdom and European Union, to strengthen the intellectual property system to compete with those jurisdictions. In particular, the current system of five years' data exclusivity is less attractive than comparable innovation and investment driven OECD countries.[54]

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53 www.findlaw.com.au/articles/1576/data-exclusivity-further-protection-for-pharmaceut.aspx viewed 9 September 2021.

54 Medicines Australia, Submission 141, p. 22.

10.64 Alexion Pharmaceuticals drew the Committee's attention to the fact that in Australia 'there are no orphan specific IP provisions.[55] Alexion proposed that orphan drug specific measures receive: seven - 10 years data protection and an addition two years for paediatric indications.[56]

10.65 Pfizer Australia pointed out how data protection (or data exclusivity) works in parallel with IP and called for an increase in data protection to drive local investment and affordable access to new medicines:

> Regulatory data protection (RDP) is a separate mechanism that operates independently and in parallel to the patent system, protecting the disclosure and unfair commercial use of the clinical trial data submitted to regulators for the registration of a new medicine. A strong RDP regime can incentivise the development and local study of new medicines and drive timely patient access, and is particularly important in situations where patents may not be available due to the nature of a new medicine, or in situations where the time needed to develop, test and secure approval for a medicine is so long that little or no patent term remains.
>
> Australia's RDP term for innovative biologics and small molecules is just five years from regulatory approval - low by global standards. In comparison the US offers 12 years for biologics and the EU offers 10 years, covering both biologics and small molecules, with an extra year available for new clinical indications. Australia offers no added protection for new clinical indications, which limits incentives to research and repurpose medicines to treat new conditions.
>
> A strong RDP term is essential to drive investment incentives for new medicines, without which innovative companies are deterred from pioneering high-risk and high-cost breakthrough research and development.[57]

10.66 Data exclusivity is discussed further in this chapter under the section titled 'Repurposing of drugs'.

Generic drugs and the problem with expired Intellectual Property stifling innovation

10.67 The Medical Oncology Group of Australia (MOGA) illustrated a problem that exists in Australia when generic competition following patent expiry

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55 Alexion Pharmaceuticals, Submission 30.1, p. 1.

56 Alexion Pharmaceuticals, Submission 30.1, p. 1.

57 Pfizer Australia, Submission 137, p. [6].

and Pharmaceutical Benefits Scheme (PBS) reform has combined to reduce the price of generic medicines:

> In some cases, reference pricing methods have resulted in a price that fails to demonstrate cost-effectiveness of a new drug to the PBAC, or that is viable for the sponsor to list the on the PBS. The system should be structured to guarantee the supply of generic cancer medicines, which are more costly in Australia than in other countries, including identifying appropriate remuneration to ensure consistency of supply.[58]

10.68 ViiV Healthcare discussed some problems that the Australian Health Technology Assessment (HTA) system has with price referencing and comparator price erosion. Viiv stated:

> In the case of the generic pricing policy changes, this can influence access to new medicines, when innovator medicines are compared to older, inexpensive medicines. The benchmarking method for determining new medicine pricing should be made more equitable.[59]

10.69 Viiv highlighted a problem with the current system for niche areas such as antibiotics.

> These medicines have been in use for many years, are no longer on patent and are distributed at low prices via a range of generic manufacturers. This is how our intellectual property system is intended to operate. Once a medicine is off patent, generic medicines are permitted to enter the market and deliver savings to government and the consumer through increased competition. However, these low prices and the application of the reference pricing policy acts as a barrier to continued innovation with respect to antibiotics. Antimicrobial resistance is an increasing public health concern. However, it will be difficult for a new antibiotic to come to market in Australia due to the reference pricing policy as it will not be possible for the manufacturer to generate a return on investment (as discussed above, the development costs are approximately US$2.6 billion), particularly when combined with the careful, low-volume, use of the antibiotic to conserve its effectiveness and slow resistance.[60]

10.70 Viiv Healthcare continued by stating that:

> … the policy doesn't recognise that some individual consumers may have better or worse outcomes from medicines that are considered clinically

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58 The Medical Oncology Group of Australia, Submission 50, p. 2.

59 ViiV Healthcare, Submission 80, p. 6.

60 ViiV Healthcare, Submission 80, p. 7.

> equivalent on average across the whole target population. For example, a patient may have side effects from the old medicine but not from the new one. So, patient choice is also important at a personalised level.
>
> One option to improve the current system is to adopt the process that is currently utilised by the National Institute for Health and Care Excellence (NICE) in the United Kingdom. Under this process, a scoping document is developed with the input of clinicians and patient groups to determine patient population, place in clinical practice and most appropriate comparator for the therapy. This mechanism would allow for independent recommendation and ensure the proposed therapy is assessed within a framework that is appropriate for the patient need. This ensures transparency in the process and allows companies to understand their likely prospect of success before submitting to the PBAC and incurring the costs that such an application entails. Additionally, by involving independent expert opinion specialised in the disease area, the process may help to prevent re-submissions, which are often the result of a difference of opinion on the comparator between the sponsor company and the PBAC.[61]

Antimicrobials

10.71 Whilst generic drugs are effective in reducing the cost of medicines for consumers after their IP has expired, it has created a problem for the innovation of novel antimicrobials in response to the growth of antimicrobial resistance (AMR). The MTAA raised this issue as an area requiring immediate attention.

> Despite the huge societal costs of antimicrobial resistance (AMR) and urgent need for antimicrobials, there is no viable market for new antibiotics in Australia and therefore few incentives and little funding available to support clinical research in this important area. The lack of commercial return for antibiotics has resulted in a decline in the number of companies undertaking antimicrobial R&D and a weak pipeline of new therapies to address AMR. This pipeline is unlikely to be sufficient to successfully keep up with the pace of AMR development globally.[62]

10.72 The World Health Organization (WHO) has described AMR as one of the key global health issues facing our generation. If no action is taken, it has been estimated that by 2050, 10 million lives a year could be lost as a result of AMR by 2050, exceeding the number of deaths caused by cancer (8.2

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61 ViiV Healthcare, Submission 80, p. 7.

62 MTAA, Submission 148, p. 7.

million). In Australia, the estimate is 10 000 lives per year. DMTC Ltd submitted that:

> According to the OECD, Australia is particularly vulnerable as our antibiotic usage is significantly higher than global averages. The OECD has estimated that nearly 10 per cent of infections in Australia are antimicrobial resistant and that an average of 290 people die each year due to multidrug-resistant bacterial infections. It forecasts that this number is likely to grow significantly in coming years.[63]

DMTC noted that it is currently working with its research and industrial partners on two projects 'specifically targeted at combating antimicrobial resistance in Q fever and the bacterium behind a disease called melioidosis.'[64]

10.73 Despite Australia's high-quality research capabilities, there remains a gap between research and commercialisation. In addition, there is a lack of coordination of AMR research into novel antimicrobials, diagnostics and surveillance.

10.74 Early stage research and development at research and academic institutions is supported by current government research funds, including specific funding for AMR from the National Health and Medical Research Council (NHMRC). [65] However, while connections between the research sector and industry are improving, closer collaboration would facilitate product development, capture the value of the investment in the research and position Australia as a leader in AMR research. MOGA commented that 'Australia needs a National AMR research agenda to ensure that there is a focus on translatable research resulting in commercially available, novel antimicrobials and associated diagnostics. A national capability audit would be a first step.'[66]

10.75 Lowest cost comparators for new antimicrobials are often generic, so they are generally undervalued by reimbursement systems. In addition, they are often held in reserve by clinicians until resistance has emerged to older treatments, so the volumes used are small. This lack of commercial return means there are few companies still investing in antimicrobial research and

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63 DMTC, Submission 57, p. 2.

64 DMTC, Submission 57, p. 3.

65 AAMRNet, Submission 53, p. 1.

66 MOGA, Submission 50, p. 2.

development.[67] The dire need for new treatments has resulted in international funding agency support (e.g. CARB-X) to go some way to fill this gap.[68]

10.76 The Australian Antimicrobial Resistance Network (AAMRNet), which described itself as 'an industry-led, inclusive collaboration of stakeholders which is committed to addressing the growing problem of antimicrobial resistance,' made a submission to the Inquiry on this issue. It commented as follows:

> A number of countries around the world are investigating how to assess the value of novel antimicrobials to include the broader value they bring to society. In the United Kingdom, the Government has partnered with industry to pilot a model of reimbursement that will de-link the revenue of an antimicrobial from the volume sold, and base it instead on the antimicrobial's value to the NHS and wider public health. This means companies will be paid for antimicrobials based on how valuable they are rather than by the quantity being used or sold: the so-called 'Netflix subscription model'. This pilot will also help to reduce the financial uncertainty in antimicrobial research and promote responsible stewardship of antimicrobials.[69]

10.77 AAMRNet noted that other countries including the US and Sweden are also developing new payment models for these medicines.[70] It made three relevant recommendations:

- Regulatory pathways and incentives are required to facilitate the registration of novel antimicrobials and diagnostics, such as an adapted orphan drug category, a fast-track process specific to novel antimicrobials, strengthening Australia's regulatory data protection (RDP) provisions for new antimicrobials and the waiving of registration fees. An appropriate regulatory pathway is also required for the repurposing of existing antimicrobials.
- New reimbursement and procurement models should be explored such as socalled 'Netflix subscription models' where value is de-linked from volume, or a separate fund or formulary for novel antimicrobials.[71]

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67 Viiv Healthcare, Submission 80, p. 7.

68 MOGA, Submission 50, p. 2.

69 AAMRNet, Submission 53, pages 3-4.

70 AAMRNet, Submission 53, p. 4.

71 AAMRNet, Submission 53, p. 2.

- A review of Australia's HTA processes is required with the aim of expanding methodologies to capture the broader value of medicines. Particular consideration should be given to Australia running its own pilot scheme, based on a model where value is de-linked from volume.[72]

10.78 RESULTS International referred to the British, American and Swedish examples of responses to this issue. In addition it drew attention to a successful Australian pilot of a 'Netflix model' for hepatitis C drugs, which involved the Commonwealth Government making a lump $1 billion payment in return for unlimited access to these drugs for five years.[73] It recommended that the Government 'establish an incentive to introduce new drugs that rewards the expected value of a treatment to society, rather than the volume of drugs sold' and 'develop a roadmap for promoting the adoption of new antibiotics with milestones to launch such an incentive.'[74]

10.79 Merck Sharp & Dohme noted this issue and the British response to it.[75] AusBiotech similarly identified this as an area of concern and while not referring to any specific overseas examples, it submitted that the Government should:

> …continue to build and implement on the national strategy and actively work to support research development and commercialisation of new therapies to address AMR through:
>
> - Evaluation of international funding models for novel antimicrobials and evaluate the potential for implementing a similar model in Australia.
> - Review and assess existing medical and pharmaceutical funding streams in relation to antimicrobials and consider implementation of a national system for reimbursement.[76]

10.80 Pfizer argued that 'novel regulatory and reimbursement policies are needed to bring back investment by [large pharmaceutical] companies to ensure the antibiotic pipeline is refilled to prevent the predicted AMR crisis,' and made two recommendations on this issue:

- Designated pathways and incentives are required to facilitate the registration of novel antimicrobials and diagnostics, such as an adapted

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72 AAMRNet, Submission 53, p. 4.

73 Results International Australia, Submission 106, pages 4-5.

74 Results International Australia, Submission 106, p. 5.

75 MSD Australia, Submission 63, pages 2-3.

76 AusBiotech, Submission 114, pages 8-9.

orphan drug category. An appropriate regulatory pathway is also required for the repurposing of existing antimicrobials.

- New reimbursement and procurement models, at both state and federal level, should be explored encompassing both in-patient and out-patient use. This should include consideration of funding models where value is de-linked from volume, or a separate fund or formulary for novel antimicrobials.[77]

10.81 When asked whether there is any potential for Australia to join in the British pilot scheme, Ms Louise Graham, Director and Head of Market Access, Pfizer, replied that:

> From what I know about that trial, I don't see it as a joined up trial, but I think there are elements of the trial framework that could be applied here to run similar kinds of trials….the concept is that you value so highly the opportunity of having the antibiotic when you need it. It's like saying, 'Break glass in case of emergency.' You have it there and you have a system of incentives around it so that, in case of emergency, you've got these things.[78]

Manufacturing

10.82 The Committee was interested to find out how Australia could encourage more innovation in the medical health sector especially for local manufacturing.

10.83 Throughout the inquiry it was noted that the COVID-19 pandemic had challenged the Australian Government to reconsider whether Australia should be considering more local manufacturing of medicines and medical devices.

10.84 Adjunct Professor John Skerritt, Deputy Secretary, Health Products Regulation, Department of Health (Adjunct Prof Skerritt), who leads the Therapeutic Goods Administration (TGA), told the Committee that the TGA has responsibility for managing but not solving the issue of medicine shortages:

> Our role is in providing advice to those companies about what is required to manufacture according to the requirements of the Therapeutic Goods Act and, therefore, for a product that can be supplied. … But the bigger question of whether the government should invest a couple of billion dollars in a

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77 Pfizer Australia, Submission 137, pages [9-10].

78 *Committee Hansard*, Sydney, 12 March 2021, p. 8.

> greenfields manufacturing site is really one for industry policy, for government. [79]

10.85 Mr Jason Aldworth, Chair, 3DMEDiTech said 'Australia has the opportunity to build on this leadership position and become a global centre for development and manufacturing and even as a gateway for global regulatory market access pathways for patient-matched medical devices.'[80]

10.86 The Centre for Law and Genetics, highlighted that the current uncertain HTA pathways of precision medicine was a barrier for commercialising some products in Australia:

> The nature of personalised medicine means that making decisions about taking clinically important products forward are based on their commercial viability and to a large extent, opportunism. A number of interviewees were cognisant of the fact that decisions to fund translation is often premised on the likelihood a Pharmaceutical Benefits Scheme (PBS) listing. Given that this scheme (and decisions to list products) has its own quirks and problems, this is an important aspect that will affect manufacturers' decisions to develop products. [81]

10.87 MTAA discussed the potential to increase development and local manufacturing of new medical devices within Australia.

10.88 Mr Burgess, from the MTAA illustrated this point:

> … we do believe that there is opportunity to increase local manufacturing, recognising that more than 80 per cent of medical technology is imported to Australia. We have quality global supply chains that, despite the pressures of COVID-19, did work. We were certainly planning around global supply chains for worse outcomes in Australia than we, fortunately, did face. So, we do have a quality global supply chain, but, in the context of COVID-19 but also more broadly, there is substantial opportunity to strengthen local manufacturing and improve patient access at the local level. Strengthening the local capacity of the industry also improves the whole medtech industry in Australia, but ultimately for the purposes of providing patients quicker and better access.[82]

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79 *Committee Hansard,* Canberra, 3 September 2020, p. 8.

80 *Committee Hansard*, Melbourne, 22 April 2021, p. 50.

81 Centre for Law and Genetics, Submission 179, pages 6-7.

82 *Committee Hansard*, Sydney, 11 March 2021, p. 15.

10.89 Dr Clancy from the MTAA discussed with the Committee the challenges of manufacturing and keeping intellectual property here in Australia. She commented that 'the initiation is not the hard part,'[83] and went on to say:

> The harder part is going from commercialisation to manufacturing, but also procurement. Who will buy it? If access to a particular device is dependent on a tender at the state level, then it doesn't matter how well we give a tax incentive or protect the IP.
>
> We need to look at the whole system to address long-term outcomes and be more focused on improvements in the whole system rather than just the price of a device or the fact that it was invented here. That brings me to value based procurement. To say it more clearly, Queensland might be inventing a pacemaker that doesn't have any wires, which is better for infection control et cetera, but, if it's not being purchased in Australia, then that's a barrier for manufacturing.[84]

10.90 The MTAA added in their submission:

> The measures to support medical research and advanced manufacturing are welcome. However, there is opportunity to further enhance the local industry's ability to address unmet clinical need. In particular, the current strong financial incentives at the early stage research end need to be matched with better incentives at the commercialisation end.[85]

10.91 At a public hearing, Mr Burgess commented that 'there is substantial opportunity to strengthen local manufacturing and improve patient access at the local level. Strengthening the local capacity of the industry also improves the whole Medtech industry in Australia, but ultimately for the purposes of providing patients quicker and better access.'[86]

10.92 One suggestion Mr Burgess made was to improve Australia's procurement processes to support local manufacturing:

> It's about ensuring that there can be appropriate incentives provided for local production and there is appropriate recognition of that in procurement processes. As I mentioned, 80 per cent of our devices are imported and that quality global supply chain won't change, in terms of its relative importance in

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83 *Committee Hansard*, Sydney, 11 March 2021, p. 17.

84 *Committee Hansard*, Sydney, 11 March 2021, p. 17.

85 MTAA, Submission 148, p. 5.

86 *Committee Hansard*, Sydney, 11 March 2021, p. 15.

> Australia, but there are still opportunities to improve our procurement processes to support local manufacturing.[87]

10.93 AusBiotech commented:

> The policy relationship between advanced manufacturing and innovation is important. While comprising just eight per cent of the economy, manufacturing is one of the major sources of innovation in Australia, responsible for a quarter of all investment in R&D. In addition, the current pandemic has revealed that we have significant gaps in capability locally and this leaves (and has left) our country in a vulnerable position in term of supply of key medical products.[88]
>
> Research funding along the value-creation 'pipeline': Competition for Business Expenditure on Research and Development (BERD) as it relates to advanced manufacturing will become increasingly intense, as policymakers all over the world seek to use micro and macro-economic levers to compete for job-creating investments and skills.[89]

10.94 At a public hearing, Professor Alexander from the Children's Medical Research Institute commented that leaders in Australia must be engaged with an entrepreneurial spirit:

> You'd be aware that the New South Wales department of health has put $25 million into a GMP viral vector manufacturing facility. That process is still in the early days, but, for example, around that, there are big biotech companies that might be the suppliers of the manufacturing equipment or ones that could benefit from a stake in the facility. Those sorts of initiatives, configured and initiated in the right way, have massive potential to engage the international community. You could get very big players coming in and looking to be involved in that. But you have to have attractive enterprises happening. Skills are a big attractant; they want to come and work with people they know are cutting-edge, and we've got lots of those.[90]

10.95 Stryker commented that Australia's HTA pathways were not 'fit for purpose' and therefore despite the many comparative advantages that Australia has for R&D and manufacturing, many international companies

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87 *Committee Hansard*, Sydney, 11 March 2021, p. 17.

88 AusBiotech, Submission 114, p. 7.

89 AusBiotech, Submission 114, p. 8.

90 *Committee Hansard*, Sydney, 12 March 2021, p. 26.

won't invest in Australia. Mr Maurice Ben-Mayor, President, Stryker, illustrated this systematic problem:

> I'm often asked, 'Why doesn't Stryker bring more R&D and manufacturing to Australia?' There are many reasons why we should. We've got a great hospital system. We have great clinicians and universities. The trends in individualised medicine favours us. There are favourable government grant programs such as the MMI and the MRFF. But the biggest issue is the uncoordinated and not-fit-for-purpose access pathways. At the moment, it's just too risky to invest when we have a system that could require eight years' worth of evidence before we can even begin supplying in our local market, if we're lucky.[91]

10.96 Ms Jaime McCoy, of Gilead Sciences, suggested that the access challenges to medicines and devices was a barrier to local manufacturing as well as the unpredictable access to novel medicines such as cell therapy.

> I think it's really hard, in my mind, to talk about manufacturing and also remove it from the access challenges that we face in Australia, because the reality is that all countries want manufacturing of pharmaceutical in their country. It's incredibly competitive. Certainly for Gilead and for most pharma companies, Australia is not considered a major market. We are one of the smaller markets. When we have unpredictability of access to things like cell therapy it's very hard for me or somebody in my position to be positioning Australia as a priority for manufacturing of cell therapies because, of course, there are other countries that also would like to have manufacturing locally.[92]

10.97 During a final public hearing, Adjunct Prof Skerritt, told the Committee:

> The final area where people felt that we needed to have clearer guidance, especially around clinical trial requirements and manufacturing standards, was medicines made through gene technology. We've commenced a targeted stakeholder consultation to identify those issues and to see if there's a need for regulatory changes or explaining the current system better.[93]

10.98 Professor Alexander, CMRI discussed the need for more of a research and funding to target gene and phage therapy.

> I'd like to see that whole pathway in Australia massively strengthened, because you not only get the patient benefit, but you get the economic benefits

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91 *Committee Hansard*, Sydney, 12 March 2021, p. 30.

92 *Committee Hansard*, Sydney, 12 March 2021, p. 8.

93 *Committee Hansard*, Canberra, 18 June 2021, p. 17.

> that flow with that—jobs and Australian IP being commercialised—and you get the economic returns.
>
> In the space of GMP manufacturing of a recombinant virus, say, our workforce is not well developed. People who are interested in developing themselves in that space have many more opportunities overseas, and this is an area that we would really like to improve. We had a lot of trouble recruiting people for our efforts in that space. That was one of the major challenges we had in getting the right people for advanced therapeutics manufacturing.[94]

10.99 Professor Skerritt, discussed with the Committee during a public hearing the idea of the Australian Government offering vouchers as a research incentive:

> What some governments have done is look at things like vouchers. In the same way we have an R&D tax concession and now we've got the patent box lower tax rate, it would be open to government to say, 'If you're a start-up here, why not get a voucher for your first couple of products?' That's a decision by government, of course.[95]

Translational partnerships

10.100 The Committee received evidence from two translational partnerships Monash Partners Research and Translation Centre and the Monash Institute of Medical Engineering (MIME) together with the Sydney Partnership for Health, Education, Research and Enterprise (SPHERE). These translational partnership groups provide advice and networks within Australia to support the translation of a new drug or device going through the commercialisation process, including clinical trials, registration and reimbursement.

10.101 Monash Partners Research and Translation Centre and the MIME is a unique collaboration of university cross faculty collaboration along with government and industry that seeks to identify clinical unmet needs that are developed to deliver healthcare innovation for the Australian community. Its submission stated:

> From modest start-up funds, we have delivered products now valued at $30 million and created IT innovation, now integrated into routine healthcare around the world.[96]

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94 *Committee Hansard*, Sydney, 12 March 2021, p. 28.

95 *Committee Hansard*, Canberra, 18 June 2021, p. 22.

96 Monash Institute for Medical Engineering (MIME), Submission 158, p. 1.

10.102 The aim of the MIME translation hub is to:

> …turn real-world problems into solutions that are then out to market and into care. It's been in place for seven years and it models off international models that are very effective. It essentially, effectively, puts engineers in scrubs alongside a surgeon while he or she is doing an operation to see how they could do it better. Engineers see things very differently to what clinicians do. That's just one example. They see firsthand what the clinical problems are and how they could innovate and do it differently.[97]

10.103 MIME provided an example of what they can achieve using the translational approach:

> An example of the sorts of things we've done is in our trauma departments, a new pulmonary decompression device, when people collapse their lungs in trauma. It used to be prehistoric what we did to them in terms of trying to get the air and fluid out of their lungs. Clinicians knew there must be a better way. Our engineers helped design a better procedure. There was a $50,000 seed grant to develop that device. It has just been valued at $30 million, and that has taken 18 months. The reason it happens is that we start with a problem. We don't start with a research issue; we don't start with a manufacturer. We start with a clinical unmet need, and then they pull that out to practice very quickly.[98]

10.104 SPHERE was established in 2016 as a collaborative partnership across 14 academic and healthcare delivery partners, including four health services, three universities and seven medical research institutes.

10.105 SPHERE aims to integrate research and initiate top-quality education and professional practises across its partnership organisations to improve health outcomes, deliver better health care, generate economic benefits and be a magnet for recruitment, retention and investment.

10.106 In 2020, SPHERE launched the HealthHatchery, a collaborative program to source, facilitate and fund the development of innovative healthcare technologies.

10.107 Dr Peter Spencer, HealthHatchery informed the Committee that 'our projects include products and services aimed at improving childhood and adult brain cancer outcomes, reducing post-operative pain for children undergoing tonsillectomy, enabling better outcomes for people with

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97 Professor Helena Teede, Co-Director, MIME, *Committee Hansard,* Melbourne, 23 April 2021, p. 15.

98 Professor Teede, MIME, *Committee Hansard,* Melbourne, 23 April 2021, p. 15.

defecation disorders and optimising the clinical pathway for managing childhood genetic disorders.'[99]

10.108 Noxopharm suggested more support was needed from Government in connecting a number of biotech start-ups with universities, venture capitalists and business advisors:

> Investment in the extension of this concept, tailored to specifically support early stage drug and MedTech companies, would benefit government and the companies alike. For government, the risk is lowered as the companies have completed the early proof of their scientific, design and business concepts, but still have a long road ahead in the clinical trial, registration and reimbursement processes. For companies, the benefits of an interconnected supportive development environment with access to clinical trial, registration and reimbursement expertise would streamline the development and commercialisation process.[100]

10.109 It suggested the following incentives could be offered to pharmaceutical companies seeking to develop new drugs in Australia:

> Offer better benefits to the OS partner 'if' they team up with local companies – this may be biotech, vendors, CRO's; extend to clinical studies. These biotech/vendors would then get the exclusivity with the investment money coming in.
>
> Offer a financial incentive (broader funding opportunities and Australian market exclusivity) that specifically supports repurposing the off-patent and/or novel formulation of existing drugs
>
> Set up an 'automated' network of collaborators - linking Advocacy groups with Biotech groups, Corporations and academic institutions to facilitate research in specialised areas such as orphan drugs, rare diseases or tailored therapies. By automated we mean using AI to link and notify collaborators of projects and funding opportunities.[101]

10.110 MIME highlighted the need for better coordination of the medical research sector across Australia. Professor Teede stated:

> We have all these initiatives on a dartboard and there are very few people whose job it is to sit above them and link it up. We have spectacular bits of that dartboard in Australia. It is about how we create a system to bring it

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99 Dr Peter Spencer, Co-founder, SPHERE, Sydney, 12 March 2021, p. 43.

100 Noxopharm, Submission 70, p. 2.

101 Noxopharm, Submission 70, p. 2.

> together in a timely way and out into manufacturing. There are many opportunities: the Modern Manufacturing Strategy, the $1.5 million, the road map that has been designed. But, again, it is putting money in silos; it is funding individual products.[102]

10.111 The Australian Cardiovascular Alliance proposed that:

> Financial incentives or bonuses could be developed for Universities and Research Institutes, rewarded on the translational capacity of the research and level of partnership with translators, including industry, rather the current, primary measure of success- peer-reviewed publication.[103]

Work visas

10.112 Several stakeholders called for a change to the current restrictive 457 visas. The Australian Cardiovascular Alliance called for an increase to the Global Talent Independent Program:

> Recruitment of talent in the specific area of pharmaceutical manufacturing is restricted with the current 457 VISA. The Global Talent Visa Program, also known as the Global Talent Independent Program, is an excellent streamlined visa pathway for highly skilled professionals to work and live permanently in Australia and covers 7 areas, including MedTech. However there are only 5,000 available.[104]

10.113 AstraZeneca also called for new work visas to attract global talent, since it is currently restricted with the 457 Visa. [105]

Horizon scanning

10.114 In recent years, there has been an emergence of medicinal products based on genes, tissues or cells. These emerging technologies provide new treatment options, including potential for patient-centric and preventative medical interventions.

10.115 The Department of Health (the Department) discussed the importance of horizon scanning and highlighted the need for it to ensure the Department has the capability to review emerging technologies.

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102 *Committee Hansard*, Melbourne, 23 April 2021, p. 16.

103 Australian Cardiovascular Alliance, Submission 76, p. 10.

104 Australian Cardiovascular Alliance, Submission 76, p. 10.

105 AstraZeneca, Submission 42, p. 3.

> Horizon scanning to determine the therapies that are likely to be submitted for regulatory review in the short to medium term is important, so that the Department (through both the Therapeutic Goods Administration and the Health Resourcing Group) has the right capability and capacity to either review, or commission for review, products based on new technologies.[106]

10.116 Mr Dale, MTAA, informed the Committee that in the past there had been a Council of Australian Governments horizon scanning unit that was called 'HealthPACT':

> Its role was to essentially scan and look for new technologies that were on the horizon, particularly in the device area, and were likely to have an impact on state healthcare systems. They did an analysis—an initial analysis, if you like—of what the technology was, how it was used, and also some of the evidence base that was supporting it. In some cases, they went into much greater detail and those reports were actually published. I'm not completely sure the year that they stopped, but I think it was around 2011. Subsequent to that, the process continued, but their results weren't published. I assume they were shared within the state authorities. I've recently been advised by one of the state health departments that, in the most recent National Health Reform Agreement, which has many elements that we would welcome, the idea is that it will be reinstated in some form. Many submitters agreed there was an immediate need for an horizon scanning unit for all novel drugs and medical devices within the Department of Health.[107]

10.117 Mr Stuart Knight, General Manager Roche, was very supportive of the need to be more strategic and engage with companies that are developing new products early in the process before they go through the regulatory process. Mr Knight stated:

> I certainly think there would be benefit in greater focus on horizon scanning—perhaps on showing what the areas of research are that we have and what might then be of particular clinical or medical interest to Australia. For example: we could then look at attracting greater clinical trials in areas that are seen as strategically important, or begin to think about what we would need to do to introduce new technologies or medicines in areas of disease priority that we'll have to overcome more quickly.[108]

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106 Department of Health, Submission 15, p. 9.

107 *Committee Hansard*, Sydney, 11 March 2021, p. 12.

108 *Committee Hansard*, Sydney, 7 May 2021, p. 26.

10.118 Professor Andrew Wilson, Chair, Pharmaceutical Benefits Advisory Committee (PBAC) commented that:

> Currently there is an absence of a nationally coordinated approach to horizon scanning for new important medicines that should be considered for health technology assessment. Given the boundaries between hospital and ambulatory care are becoming increasingly blurred, there is an increased need with some medicines to consider the shared benefits and costs between state health systems and the Commonwealth PBS/MBS. The PBAC notes the agreement of the Australian and State and Territory governments to explore a nationally cohesive health technology assessment approach and recommends medicines horizon scanning be included in this approach.[109]

10.119 Research Australia suggested that the Government should fund a joint Commonwealth and state and territory horizon scanning program:

> This is essential to allow for Australian HTA process to have an up to date understanding of new medical technologies and devices. This will bring the latest medicines and technologies to Australians at a time when health technologies and therapies are rapidly changing and bringing exciting new treatments to patients worldwide.
>
> It requires a regulator that is well placed to collect information about emerging trends in Australia and overseas and is able to consult quickly and effectively with product manufacturers, innovators, health professionals and consumers.[110]

10.120 Luminesce Alliance informed the Committee about the International Horizon Scanning (BNeLuxA) Initiative and suggested that Australia consider joining.

> This is currently a pilot project involving eight European countries, that aims to seek successful ways of collaborating on pharmaceutical policy, anticipating the impact of high cost medicines. By utilising a central database to continuously gather data, analyse research and literature and facilitate information sharing about new and developing medicines, the framework serves to enable policymakers to identify future challenges, set priorities, improve insight in expected costs, and facilitate timely decision and joint negotiations for lower drug prices.[111]

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109 Department of Health, Submission 15.3, p. 3.

110 Research Australia, Submission 78, p. 6.

111 Luminesce Alliance, Submission 32, p. 20.

Repurposing drugs

10.121 The repurposing of 'old' drugs to treat new conditions presents a regulatory challenge for government. The Department explained to the Committee what some of these challenges are:

- the medicine has broader regulatory approval in other countries than in Australia and the only limitation is an Australian sponsor's willingness to pursue an application to match the TGA approval with the overseas one by collating existing evidence; and b) the formal evidence to support a broadening of the registration needs to be generated.
- There may be little incentive for a sponsor to generate the data required to support an application to register a new indication, and to pay regulatory fees for extension of indications to cover an additional indication. This is because some medicines are routinely used 'off-label' for other conditions, and have become part of the standard clinical paradigm without having formal regulatory approval. If the medicine is cheap, or used in in-patient situations there may not be sufficient incentive to seek TGA registration for the particular indication (and thus possible PBS reimbursement).[112]

10.122 Rare Voices Australia (RVA) informed the Committee that there are many examples of a medicine approved for a more common condition also demonstrating benefits for a rare disease. However, due to small numbers, it is not always commercially viable for companies to seek reimbursement for a rare disease indication. RVA stated:

> It is difficult, if not impossible, for a non-pharmaceutical sponsor to submit an application. Without government reimbursement, many rare disease medicines are unaffordable for people living with a rare disease and their families.[113]

10.123 Medicines Australia (MA) suggested there needs to be incentives, to encourage innovative companies to invest in the level of research required to enable older, legacy products to be repurposed for new uses:

> The costly and uncertain regulatory and reimbursement requirements contribute to this dilemma which ultimately denies patients benefit...

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112 Department of Health, Submission 15, p. 22.

113 Rare Voices Australia (RVA), Submission 86, p. 2.

> There needs to be a framework to rapidly update and/or repurpose older medicines via a simplified regulatory and reimbursement pathway to facilitate improved clinical outcomes for Australian patients.[114]

10.124 Amicus Therapeutic commented that for many Australians living with rare diseases, the promise of repurposing therapies is a symbol of hope that new treatments will be discovered and lead to better health outcomes:

> In repurposing therapies, effective treatments for diseases may be discovered in a process that is faster and less expensive than starting from scratch, and often with a reduced risk of failure as the safety profile of the medicine is typically well-established.

10.125 Amicus Therapeutics added that there are currently no incentives in Australia to encourage the repurposing of medicines:

> While mechanisms such as orphan drug designation reduce the cost to register and reimburse a new indication, it doesn't support the medicines industry to undertake critical R&D work that enables us to repurpose a medicine.[115]

10.126 The Committee asked Professor Skerritt if it would be possible for someone, other than a sponsor, to put in a submission for a drug to be reimbursed on the PBAC. He commented:

> For repurposed drugs, for an indication where a company may not be interested, but the patient group and the prescriber group are certainly interested. I wouldn't want to bury the possibility of seeking public expressions of interest, but I do realise that companies are protective of their intellectual property and there would be a need for legislative and regulatory changes. There were other things, such as working on evidence generation.[116]

10.127 MOGA suggested making allowances for clinicians to make submissions to HTA processes for off-patent drugs:

> We strongly recommend legislative reform that makes it easier for clinical groups like MOGA make submissions to regulatory bodies for approval of drugs especially for older, off-patent drugs with new indications, or for drugs to treat rarer conditions for which the budget impact is expected to be minimal.[117]

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114 Medicines Australia, Submission 141, p. 23.

115 Amicus Therapeutics, Submission 31, p. 3.

116 *Committee Hansard*, Canberra, 18 June 2021, p. 30.

117 MOGA, Submission 50, p. 4.

10.128 MA pointed out that the Australian Government is unable to compel a sponsor to make an application under the current provisions of the *Therapeutic Goods Act 1989* (Cth). MA stated:

> It is possible for non-commercial entities, such as clinical colleges or patient organisations to become a sponsor of a product but they would need to take on the medico-legal responsibilities for product stewardship that sponsorship of a particular medicine involves.[118]

10.129 MA added that 'without a sponsor application to repurpose an established medicine, there is no basis upon which to initiate a formal assessment.'[119]

10.130 Adjunct Prof Skerritt informed the Committee that the TGA had recently explored different options on the issue of repurposing of drugs, including that the TGA held several workshops with industry and intends to propose options to Government:[120]

> Some people said it actually needs to start at the other end. If its repurposed use doesn't have the potential for reimbursement then, if it's an expensive drug, you wouldn't put it into the regulatory system. Another idea was to provide exclusivity periods. These are all the ideas, the kites that have been flown. Many of them will require changes to either laws or at least government decisions. A further idea was open access to real-world usage data and an international evidence basis.[121]

10.131 Mrs Nicole Millis, Chief Executive Officer, Rare Voices Australia highlighted the need for a new pathway to encourage drug repurposing for people living with rare disease:

> For the average person on the street, where they have a common condition, the doctor will say, 'Take medicine A.' Maybe they've got a healthcare card and it costs them $6. For the person with a rare condition who takes medicine, their doctor will say, 'This will help.' It's not reimbursed. It might cost them $6,000. It sets up an alternative system which is uncertain and inequitable.[122]

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118 Medicines Australia, Submission 141.1, p. 23.

119 Medicines Australia, Submission 141.1, p. 5.

120 *Committee Hansard*, Canberra, 18 June 2021, p. 16.

121 *Committee Hansard*, Canberra, 18 June 2021, pages 29-30.

122 *Committee Hansard*, Sydney, 11 March 2021, p. 7.

10.132 Ms Delaine Smith, CEO, Australasian Leukaemia and Lymphoma Group encouraged the Government to establish a new pathway that would include Government support for research and data collection:

> For therapies and technologies that have insufficient evidence to support reimbursement in the Australian market, enable a pathway whereby the PBAC or MSAC could commission the research on that new agent only, in order to collect and support the reimbursement data.[123]

10.133 MA suggested to the Committee that the Australian Government should hold a series of workshops with industry to co-design a way forward for repurposing drugs. This included to:

- explore the full scope of repurposing that would be of benefit to Australian patients e.g. including closely related indications for oncology medicines
- fully identify the barriers to repurposing
- co-design policy, regulatory and reimbursement solutions to overcome these.[124]

10.134 Ms Sharon Winton, CEO Lymphoma Australia shared her concern that some patients are turning to the black market to purchase medicines:

> We also feel that we need a pathway for the repurposing of some medicines, for example, biosimilars. They are proving to be more cost effective and can bring wider access to a much broader cohort of patients.[125]

Pharmaceutical perspective

10.135 Several of the pharmaceutical companies explained that there are a number of challenges with the repurposing of medicines. Essentially, the repurposing of a medicine occurs late in its lifecycle, with multiple brands and a price that reflects very low margins. This, combined with the uncertainty of pathways and high costs of TGA and PBAC submissions, act as disincentives for repurposing medicines.[126]

10.136 Roche stated:

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123 *Committee Hansard*, Melbourne, 23 April 2021, p. 51.

124 Medicines Australia, Submission 141.1, p. 3.

125 *Committee Hansard*, Brisbane, 18 May 2021, p. 3.

126 Roche, Submission 92, p. 22; Johnson and Johnson, Submission 134, p. 16; Bristol Myer Squibb, Submission 118, p. 4.

> There are number of hurdles including long assessment timeframes, high costs to the sponsor in multiple hundreds of thousands of dollars with no guarantee of fee waivers, or successful evaluation due to lack of conventional clinical trial evidence. These challenges need to be addressed through further system changes to make repurposing medicines for rare conditions a more viable option for sponsors.[127]

10.137 Roche made the following suggestion in relation to potential changes that could be made to incentivise sponsors to repurpose medicines for rare disease. This included reviewing real world evidence (RWE) and using it to support indication expansions:

> A regulatory and HTA review could be expanded to consider both the evidentiary requirements and how evaluation processes need to change to ensure that the value of repurposing medicines for rare conditions is recognised and that price reductions aren't a disincentive to providing repurposed treatment options. This review could include how RWE could be used as sufficient evidence to support further indication expansions of current medicines.[128]

10.138 AstraZeneca suggested promoting the registration of new and off-patent indications for rare diseases and giving consideration to the following incentives:

- Voucher Program: Register an indication for a rare or less common disease in return for a priority review of a subsequent marketing application.
- Market exclusivity: A period of market exclusivity will renew interest in medicines/ devices that are either off-patent or have potential for repurposing.
- Where IP is expired, as in the case of re-purposed medicines, a cost neutral approach to meet an unmet need is appropriate.
- Better clarity on how PBAC/ MSAC committees treat real world evidence (RWE) and secondary tiered data sources when addressing off-label and/or pan-cancer treatments.[129]

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127 Roche, Submission 92, p. 22.

128 Roche, Submission 92, pages 22-23.

129 AstraZeneca, Submission 42, p. 3.

10.139 Amicus Therapeutics provided the following international examples of repurposing incentives to highlight ways in which the Government could encourage repurposing drugs for rare diseases:

- The incentives includes an Orphan Disease Tax Credit, which offers a tax credit equal to 25 per cent of clinical trial costs for the development of orphan drugs, seven-year market exclusivity arrangements that prevents generics entering the market for these orphan indications, as well as fast-tracked and accelerated approval processes including for paediatric indications. The FDA states the program has successfully enabled the development of over 600 therapies for rare diseases.[130]
- The European Union offers 10 years of market exclusivity with an additional two years if the company complies with a paediatric investigation plan at the time of orphan medicine designation. Companies also receive reduced fees for regulatory activities as well as protocol assistance and access to the centralised procedure.[131]
- Taiwan provides a three-to-five-year data exclusivity period for a new indication; depending on whether clinical trials were conducted in Taiwan, as well as a 10-year exclusivity period for orphan drugs.[132]
- The Republic of South Korea offers de-facto data-exclusivity through its post marketing surveillance requirements, effectively combining the need for a local phase 4 study with a period of data exclusivity that can last up to 10 years, depending on the medical need.[133]
- Hong Kong provides an eight-year data exclusivity period as part of its patent system to provide incentives for new uses.[134]

10.140 Amicus Therapeutics suggested that the government could consider the 'waiving of PBAC and TGA submission fees for orphan drug designations to five years [as opposed the 12 months] to encourage the repurposing of medicines.'[135]

> Additionally, we would suggest extending the waiving of all PBAC and TGA submission fees related to orphan drugs from the current timeframe of 12 months to the length of the exclusivity period as we believe this would

130 Amicus Therapeutics, Submission 31, p. 3.

131 Amicus Therapeutics, Submission 31, p. 3.

132 Amicus Therapeutics, Submission 31, p. 3.

133 Amicus Therapeutics, Submission 31, p. 3.

134 Amicus Therapeutics, Submission 31, p. 3.

135 Amicus Therapeutics, Submission 31, p. 4.

> encourage and support companies to continue investigating and following up with expanded populations such as paediatric indications for a therapy.[136]

10.141 Johnson & Johnson suggested extending data exclusivity could incentivise sponsors to repurpose certain drugs:

> There is an existing mechanism, currently only for medicines, data exclusivity, which provides for a form of non-patent intellectual property protection for new data required to list a new treatment. This concept could be extended, where required, to ensure any new use of an existing product would be protected for a period sufficient to secure investment sufficient to produce new data. We note that Australia currently provides only 5 years of data protection whereas comparable jurisdictions provide between 8 and 12 years to better support research and development.[137]

10.142 Noxopharm suggested the Government could 'offer a financial incentive (broader funding opportunities and Australian market exclusivity) that specifically supports repurposing the off-patent and/or novel formulation of existing drugs.'[138]

Off-label prescribing

10.143 Western Australia's Department of Health told the Committee there is currently no mechanism that captures off-label prescribing practices or patient outcomes. It suggested:

> This is untapped knowledge and there is potential for clinical risk. We recommend consideration be given to building a national mechanism to accurately capture off-label use and reasons for use, as well as to capture the outcomes (analogous to post-market surveillance).
>
> Clinicians who have patients with rare diseases or clinical unmet need may prescribe off-label, however without a mechanism for data capture, the knowledge is lost and the benefit for future similar patients remains unknown.
>
> It is suggested that any requirement to input and capture data of off-label prescribing needs adequate workforce resourcing and options investigated, such as mining of natural language processing and automatic data capture

136 Amicus Therapeutics, Submission 31, p. 4.

137 Johnson & Johnson, Submission 134, p. 16.

138 Noxopharm, Submission 70, p. 2.

> from electronic medical records, to ensure burden on clinical staff is minimised.[139]

10.144 PFIC Network were frustrated with the Australian system in that off-label medicines for PFIC patients can be very expensive and take a long time to be approved. PFIC raised the following two issues:

> For rare diseases, lack of transparent and equitable pathways for repurposing existing treatments that are reimbursed for more common conditions.
>
> Lack of clear pathways and length of time for rare disease patients to access orphan drugs, new treatments and personalised medicine.[140]

10.145 The PBAC commented that it is 'aware of and concerned about situations where condition-specific clinical practice guidelines recommend medicines listed on the PBS but which are not PBS listed for those indications. This includes both on- and off-patent medicines. Such situations may lead to inequity in access to treatments.'[141]

10.146 The Children's Cancer Institute explained that 'in paediatric oncology there is a heavy reliance on off-label use of drugs, [however], appetite for risk of off-label use of drugs differs at different centres, again resulting in equity/access issue for patients nationally.'[142] The Medical Oncology Group of Australia likewise submitted that 'the different coverage of on-label and off-label indications in hospital and PBS formularies may affect the continuity and affordability of treatment for patients.[143]

10.147 The Western Australian Department of Health expressed concern that 'there is currently no mechanism that cumulatively captures off-label prescribing practices or patient outcomes,' and that without such a mechanism while some patients can access the medicines, 'the knowledge is lost and the benefit for future similar patients remains unknown.' It recommended this be remedied by the creation of 'a national mechanism to accurately capture

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139 WA Department of Health, Submission 129, p. 4.

140 PFIC Network, Submission 19, p. 3.

141 Professor Andrew Wilson, Chair, PBAC, Submission 15.3, p. 7.

142 Children's Cancer Institute, Submission 84, p. [3].

143 MOGA, Submission 50, p. 3.

off-label use and reasons for use, as well as to capture the outcomes (analogous to post-market surveillance.'[144]

10.148 The Centre for Law and Genetics noted that off-label use poses a number of difficulties due to the lack of evidence for the safety, efficacy and cost-effectiveness of the medicines in question, and the potential for it to 'undermine regulatory and research processes' by bypassing the TGA and diverting patients away from clinical trials.[145]

10.149 At the same time the Centre for Law and Genetics argued that it has a number of benefits, including helping to fill the gaps in medicine development caused by the commercial imperatives that drive the work of pharmaceutical companies, and 'provid[ing] clinicians with the ability to respond dynamically to clinical challenges.' Their proposed solution to the off-label dilemma was to enable a medicine's registered indications to keep pace with its clinical use better.[146]

Updating indications

10.150 The challenge of keeping a medicine's registered indications up-to-date, thereby minimising off-label use, was discussed by several submitters. In their joint submission the Medical Oncology Group of Australia and the Private Cancer Physicians of Australia wrote that:

> TGA indications do not keep pace with evidence development. This is due to many factors including the complexity of the approval process; only drug sponsors can lodge an application for a new indication; lack of commercial incentives; off-label prescribing is clinically acceptable if supported by evidence; and, new evidence can be developed without the involvement of the original sponsor. Addressing these issues may improve the responsiveness of the registration process to changes in the clinical setting.[147]

10.151 The Centre for Law and Genetics commented that regulators should 'take a more proactive approach' to aligning a medicine's registration with current clinical practice. It proposed 'facilitating stakeholders other than the original drug sponsor to apply for extended uses of a medicine.'[148]

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144 WA Department of Health, Submission 129, p. [4].

145 Centre for Law and Genetics, Submission 179, p. [11].

146 Centre for Law and Genetics, Submission 179, p. [12].

147 MOGA, Submission 50, p. 1.

148 Centre for Law and Genetics, Submission 179, p. [24].

10.152 The Australian Amyloidosis Network identified the same difficulty with updating indications, and suggested providing an inexpensive process relying on overseas approvals for sponsors to use, or a pathway 'without undue cost or complexity' for clinician and patient groups.[149]

10.153 Medicines Australia argued that 'there needs to be a framework to rapidly update and/or repurpose older medicines via a simplified regulatory and reimbursement pathway to facilitate improved clinical outcomes for Australian patients.'

Committee Comment

10.154 The Committee understands that Australia has the potential to grow and capitalise on its research and development (R&D) sector for novel medicines and technologies over the next decade. Australia has a highly skilled workforce, a strong university research sector, valuable translational innovation hubs, and supportive cross jurisdictional funding and R&D initiatives.

10.155 There was overwhelming industry support for the recent updates to the RDTIs for the healthcare sector in the 2020-21 Federal Budget. The Committee recognises that uncertainty around RDTIs or continuous tax adjustments creates disincentives for industry to invest in Australia. The Committee recommends the Australian Government ensure that the RDTIs remain stable and competitive internationally. In addition, the Committee encourages the Australian Government to promote the newly instated RDTIs to industry around the world.

10.156 The Committee congratulates the Australian Government for introducing the Patent Box scheme in the 2021-22 Federal Budget. This is a strong measure that will attract international industry to invest in Australia for years to come. The Committee recommends the Australian Government review the implementation of the patent box every two years to ensure it is operating effectively.

10.157 The Committee believes the Australian Government should focus on several more initiatives to continue to strengthen Australia's R&D sector for health care. This includes the better coordination of the R&D sector to commercialise novel drugs and medical technologies. The Committee encourages the Australian Government to undertake a review of the sector

149 Australian Amyloidosis Network, Submission 98, p. [6].

across Commonwealth, state and territory funding with a view to having funding initiatives distributed in a methodical way throughout all stages of the R&D pipeline. Noting the work underway through the Modern Manufacturing Program. This review would feed into the development of an updated roadmap to facilitate the manufacturing and commercialisation of novel drugs and technologies in Australia.

10.158 The Committee believes there is merit in extending the data exclusivity for orphan drugs and vaccines. The Committee encourages the Australian Government to investigate the benefits of increasing data exclusivity for orphan drugs and vaccines to a period of up to 10 years. This would encourage on-shore innovation and manufacturing of novel drugs and medical technologies.

10.159 Manufacturing of medicines and medical devices requires further critical consideration in light of the COVID-19 pandemic. The Committee believes there are significant opportunities for Australia to capitalise on our world class research and university sector. This includes supporting the commercialisation and manufacturing of novel therapeutics and medical technologies. The Committee encourages the Australian Government to consider funding initiatives to support translational partnerships and the manufacturing of health care products for all Australians over the next decade with long-term funding initiatives.

10.160 Overall, generic drugs have been beneficial for Australian consumers. However, the Committee heard evidence that suggested generic drugs were inhibiting innovation of certain medicines such as antimicrobials. The Committee commends the Australian Government's pilot scheme for payment for hepatitis C drugs, which appears to have been successful, and urges the Government to consider implementing a similar pilot for payment of antimicrobials, in cooperation with the states and territories.

10.161 The Committee believes there is an immediate need for the Australian Government to fund an horizon scanning unit within the Department of Health. The Committee suggests the Department of Health consider the creation of a partnership with the National Institute for Health and Care Excellence (NICE) in the United Kingdom to develop learnings that will assist Australia to establish an appropriate horizon scanning unit within the Department of Health, which is linked appropriately into the HTA process.

10.162 The Committee acknowledges submitters' concerns about the role that off-label use currently plays in the treatment of Australian patients for many diseases, and the inconsistencies and uncertainties that result. The

Committee hopes that the recommendations it makes throughout this report to improve patient access, particularly access for patients with rare diseases, will go a large way to alleviating this problem. The Committee's view is that, while it has its downsides, the system of off-label use provides necessary flexibility to clinicians and should be retained in its current form.

10.163 The Committee acknowledges that reforms are needed to the way in which indications of already listed therapeutic goods are updated under the current system. The Committee believes that its recommendation for the Australian Government to establish an annually capped fund to support submissions without a commercial sponsor, and a recommendation to establish a new pathway that incentivises the repurposing of drugs for all diseases, will improve the way already listed indications can be updated in the future. The Committee believes that this should be considered as part of the broader rethinking of how the system supports repurposing.

10.164 The repurposing of drugs has been an issue that interested stakeholders have been grappling with for several years. The Committee is emphatic that a solution needs to be arrived at that satisfies all parties, with the outcome ensuring that there is equitable access to drugs listed on the PBS for rare diseases.

10.165 The Committee sees merit in the suggestion from Medicines Australia that encourages the Australian Government to undertake workshops with industry to co-design a way forward for the repurposing of drugs. Although some initial workshops have already been held by the Department of Health on this, the Committee believes it would be worthwhile undertaking a more comprehensive review to allow for an acceptable co-design option to be agreed upon between government and industry.

11. Recommendations

List of Recommendations

Establish a Centre for Precision Medicine and Rare Diseases

Recommendation 1

11.1 The Committee recommends the Australian Government establish a Centre for Precision Medicine and Rare Diseases within the Department of Health.

- **The objective of the Centre should be to ensure that the capacity of the Department of Health is enhanced to provide Australians with timely access to new drugs and novel medical technologies, including for rare diseases, and that the HTA process and government research agenda aligns with this outcome.**

- **The Centre should provide advice to the Department of Health and the Australian Medical Research Advisory Board on research priorities.**

- **The Centre should provide education and training information including support for patients and a comprehensive horizon scanning unit for new medicines and novel medical technologies.**

- **The Centre should provide advice to governments on the establishment of a dedicated regulatory Health Technology Assessment pathway for cell and gene technologies, in consultation with state and territory governments, industry, patients and other**

relevant stakeholders. The Centre should regularly provide advice to government on the effectiveness of those pathways and areas for further reform.

Establish a National Genomics Testing Program

Recommendation 2

11.2 The Committee recommends that, consistent with Recommendation 1 and the establishment of a Centre for Precision Medicine and Rare Diseases, the Health Technology Assessment (HTA) process for cell and gene therapies be simplified to establish a clear and certain pathway for such therapies.

- **This simplified process should be considered together with a new HTA pathway for cell and gene therapy.**
- **Building on the Medical Research Fund Genomics Mission, the Australian Government and state and territory governments should establish a jointly funded national genomics testing program to provide equitable access to genomic testing nationwide. As part of the program, governments should ensure the provision of genomics counselling for all patients.**
- **The Australian Government should prioritise and simplify the regulation of cell and gene therapy pathways for clinical trials in Australia.**

Establish an Office of Clinical Evaluation

Recommendation 3

11.3 The Committee recommends the Australian Government establish an Office of Clinical Evaluation within the Department of Health to assess the best and most effective care for patients in the context of new and emerging health technologies.

- **The Office should enable evaluation of both pharmacological and non-pharmacological interventions, combination products and products with different sponsors. It should also establish a "living**

evidence" function to ensure Health Technology Assessment is based on the most up-to-date global health practices.

- **The Office, in consultation with relevant stakeholders, should conduct a review of how the Department's Health Technology Assessment system assesses combination products, particularly combinations with different sponsors, with a focus on:**
  - **Value attribution between the different products**
  - **Challenges to cooperation between sponsors due to competition law**
  - **Disincentives for a sponsor with an already listed product to participate in its combination listing**
- **The Office should consider collaboration with the National Institute for Health and Care Excellence (NICE) in the United Kingdom to establish similar clinical evaluation processes in Australia that links in with Australian Health Technology Assessment processes.**
- **The Office should cooperate and share information with the state and territory governments to ensure that patients receive treatment where it is safest and most efficacious for them and that there are no gaps in continuity of care.**

Improving the Life Saving Drugs Program

Recommendation 4

11.4 The Committee recommends that the assessment process for the Life Saving Drugs Program (LSDP) be streamlined and delays in access to treatments be reduced by ensuring that a sponsor only need lodge one application for one Health Technology Assessment pathway. The Committee recommends either:

- **Providing sponsors with an immediate pathway to the LSDP Expert Panel (instead of waiting for a PBAC determination), or**
- **Providing a pathway by adjusting the Pharmaceutical Benefits Scheme section 100 program, with specific criteria, as with other section 100 programs.**

The Committee believes it is critical that consideration be given to how the LSDP will integrate with an increasing number of precision medicine applications into the future.

Health Economists

Recommendation 5

11.5 The Committee recommends that the Australian Government develop a labour market and skills strategy to expand the number of health economists in Australia. This could include encouraging training within Australia as well as seeking expertise from overseas.

Improving Education and Engagement for the TGA and the HTA processes

Recommendation 6

11.6 The Committee recommends that the Department of Health increase its efforts to educate and engage with patients, clinicians, industry and the public and develop education campaigns on all aspects of the regulation and reimbursement system.

11.7 The Committee recommends that the Department of Health improve information available on the websites of the Therapeutic Goods Administration (TGA) and its Health Technology Assessment (HTA) bodies for all users including patients, clinicians, industry and the public. This would include:

- **Using plain English language, infographics and videos to explain general processes and timelines**
- **Explanations on the TGA and all HTA's websites of how that entity fits into the overall regulation and reimbursement system, similar to the Medical Services Advisory Committee's *Australian Government HTA Processes* factsheet.**
- **The Department of Health expanding the Pharmaceutical Benefits Scheme Medicines Status website to include technologies funded through the Medicare Benefits Schedule or create an equivalent website for such technologies.**

Improving National Blood Authority Alignment

Recommendation 7

11.8 The Committee recommends that the Department of Health and the National Blood Authority, in consultation with state and territory governments, reform the Health Technology Assessment processes for blood products to provide better alignment with the Health Technology Assessment system, including:

- **Publication of guidance documents for applicants**
- **Establishment of timelines for applications, and publication of an assessment cycle calendar**
- **Creation of a parallel Therapeutic Goods Administration and Health Technology Assessment process.**

Submission Fee Waivers

Recommendation 8

11.9 The Committee recommends that the Australian Government make the following changes to submission fees for the Therapeutic Goods Administration (TGA) and the Pharmaceutical Benefits Advisory Committee (PBAC) and where appropriate Medical Services Advisory Committee (MSAC) assessments in the following separate circumstances:

- **Replace the current orphan drug fee waivers with a HECS-style fee waiver, in which orphan drug application fees are payable on successful application, only once the drug has earned the sponsor a certain amount of revenue. The Department of Health should determine this threshold value in consultation with industry**
- **To support smaller companies, HECS-style fee waivers for any sponsor company with revenue at or below $50 million per annum**
- **HECS-style fee waivers for Australian start-up companies with a specified amount of revenue in the Australian market to promote innovation.**

The Committee also recommends introducing a sliding scale for fees for resubmissions, with fees being lower for resubmissions.

Funding for Submissions without a Sponsor

Recommendation 9

11.10 The Committee recommends that the Australian Government establish a fund to support patients, clinicians and non-profit organisations to sponsor registration and reimbursement applications where there is no realistic prospect of a company serving as sponsor, and where the Department of Health is otherwise supportive of the application.

- **Such a fund should be targeted at treatments for conditions where low patient numbers in Australia serve as a market barrier and where there is a clinical demand and need. The fund should be available for applications to repurpose previously listed medicines and technologies.**

- **The fund should be annually capped with clear and transparent eligibility rules.**

The PBAC and Managed Access Programs

Recommendation 10

11.11 The Committee recommends that the Australian Government amend the *National Health Act 1953* (Cth) to give the Pharmaceutical Benefits Advisory Committee the power to authorise Managed Access Programs. The eligibility criteria for these Managed Accessed Programs should be aligned as far as possible with the eligibility criteria for the Therapeutic Goods Administration's provisional registration.

Review Repurposing of Drugs

Recommendation 11

11.12 The Committee recommends that the Department of Health conduct a comprehensive consultation process with industry to establish a more flexible way forward for the repurposing of drugs in Australia. This should include:

- **Establishing a new pathway that incentivises the repurposing of drugs for all diseases, not just rare disease.**

TGA Reform

Recommendation 12

11.13 The Committee recommends that the Therapeutic Goods Administration make the following changes to its Orphan Drugs Program:

- **Provide automatic access to the Priority Review Pathway for all medicines granted an orphan drug designation**

- **Treat paediatric patient populations as separate to adult patient populations for the purposes of the eligibility criteria**

- **Better account for the extra costs incurred by a sponsor in expanding its medicine to paediatric indications, for the purposes of assessing commercial viability as part of the eligibility criteria**

- **Where the prevalence of a disease is unknown in Australia, accept evidence of prevalence in other comparable countries or, in diseases of extremely low prevalence, worldwide for the purposes of the eligibility criteria.**

Molecular Indications

Recommendation 13

11.14 The Committee recommends that the Department of Health reform its regulatory and reimbursement processes to enable therapeutic goods to be registered and reimbursed by molecular indication in addition to by disease indication. This should include legislative change if necessary.

Funding for TGA

Recommendation 14

11.15 The Committee recommends that the Australian Government reconsider the current cost recovery funding model for the Therapeutic Goods Administration, paying attention to future staffing and IT infrastructure

needs in an environment where demand on its services and systems are expected to increase in future years. The Committee recommends funding specifically for:

- **IT systems upgrades, to modernise and match the IT capability of other overseas Tier 1 regulators.**
- **An expansion of its staffing capacity in areas of new medical and technological advances including for horizon scanning.**
- **The release of TGA Australian Public Assessment Reports at the same time as a prescription medicine is listed.**
- **The implementation of the HECS-style fee waivers outlined in Recommendation 8.**

Membership of the PBAC and MSAC

Recommendation 15

11.16 The Committee recommends that the Australian Government ensure the membership of the Pharmaceutical Benefits Advisory Committee and Medical Services Advisory Committee provides the appropriate expertise for all applications. This should include the possibilities of enhanced cross-membership between the two committees and the appointment of temporary members to consider individual applications.

- **Recognising the nature of health challenges in Indigenous communities, membership should include representation from Aboriginal and Torres Strait Islander Peoples.**

Increase International Collaboration

Recommendation 16

11.17 The Committee recommends that the Department of Health investigate further opportunities for the formation of an international Health Technology Assessment consortium similar to the Access Consortium to streamline the regulatory process for certain medicines and medical technologies. This investigation should include discussions with representatives of the Health Technology Assessment bodies of the

United Kingdom, Canada and other countries with systems similar to Australia's.

- **The Committee recommends that the Therapeutic Goods Administration work with the United States Food and Drug Administration and other overseas regulators to establish an equivalent of Project Orbis for non-cancer rare diseases, or to expand Project Orbis to include such diseases.**

Breakthrough Devices Program

Recommendation 17

11.18 The Committee recommends that the Australian Government establish a scheme that supports the domestic medical technology sector, similar to the Food and Drug Administration's Breakthrough Devices Program in the United States.

Review the NIP

Recommendation 18

11.19 Recognising the vital role that vaccines play in addressing many diseases, including its importance in providing protection against Covid-19, the Committee recommends that the Department of Health conduct a review of the National Immunisation Program. This review should focus on reforming existing approaches used to value vaccines to ensure early and rapid deployment of vaccines in Australia.

Reform the Prosthesis List

Recommendation 19

11.20 The Committee recommends that the Australian Government continue to address the following matters in its reforms to the Prostheses List:

- **The lack of coverage for non-implantable devices under the current arrangements.**

- **Improving coordination between the Medical Services Advisory Committee and the Prostheses List Advisory Committee to provide faster access for patients.**

Supply of Medicines on the PBS

Recommendation 20

11.21 The Committee recommends that the Australian Government establish a last resort mechanism for directly securing ongoing supply of medicines that meet a high clinical need and lack suitable alternatives that are at risk of being delisted from the Pharmaceutical Benefits Scheme.

Improve Newborn Screening Program

Recommendation 21

11.22 The Committee recommends:

- **The federal, state and territory health authorities complete the standardisation of newborn screening across Australia**
- **As part of that process, the Australian Government work with states and territories to expand the newborn screening program based on new understandings of genomic testing for conditions and international best practice**
- **That the Australian Government in collaboration with states and territories, conduct reviews every two years to determine whether the screening program should be further expanded based on new Australian and international scientific and medical knowledge.**

While not in the terms of reference for this inquiry, the Committee recognises and supports the calls from rare disease patient groups for more funding for treatment pathways for actionable disorders across states and territories, where identified through newborn screening.

Improve the Clinical Trial System in Australia

Recommendation 22

11.23 The Committee recommends that all levels of government prioritise and implement with urgency the harmonisation of Human Research Ethics Committee (HREC) and Site-Specific Assessment submissions into one Australian online platform and enable parallel review by HRECs and Research Governance Offices.

- **The platform should be developed within the purview of the Australian Commission on Safety and Quality in Health Care.**

- **This work should be a continuation from the work prepared as part of the National Clinical Trials Governance Framework.**

Recommendation 23

11.24 The Committee recommends that all levels of government jointly provide funding for the development of a national clinical trial register. It should include:

- **Development of a sophisticated digital platform to collect and facilitate patient identification, patient recruitment, patient retention and completion rates for clinical trials.**

- **Linked data from existing national registers and consideration should be given to whether the register is best operated by a government agency or an existing Non-Government Organisation, or an academic body with appropriate experience.**

Recommendation 24

11.25 The Committee recommends the Australian Government develop policies that encourage modernising digital technologies and practices to position Australia as the premier destination for international clinical trials. This would include developing national standards for the use of e-consent, e-signature, and electronic medical records to enable remote monitoring and participation in clinical trials across Australia.

- **National standards should include standardising clinical costs and fees that are competitive with international fees.**

Recommendation 25

11.26 The Committee recommends the Australian Government should develop a national standard approach, including nationally agreed systems and standard operating procedures to support and strengthen the capacity to conduct clinical tele-trials in rural, regional and remote areas.

- **This approach should be developed in consultation with industry and allied health workers.**

- **This would include the need for education and training opportunities for General Practitioners and all allied health workers engaging in clinical trials using tele-trials and multi-centre trials.**

Recommendation 26

11.27 The Committee recommends the Australian Government should continue to fund Clinical Trial Networks with a particular focus on developing seed funding for Indigenous Health Clinical Trial Networks.

Research and Development

Recommendation 27

11.28 The Committee recommends the Australian Government reform data exclusivity provisions in Australia with a view to extending data exclusivity for orphan drugs and vaccines to a period of up to 10 years. The Australian Government should:

- **Develop additional reforms to data exclusivity timeframes to support research and development into new drugs and novel medical technologies in areas of unmet need.**

- **Consider future funding initiatives for novel drug discovery and support research and development partnerships in Australia. This would assist new drugs and novel medical technologies in early stage and pre-commercial development.**

- **In partnership with the states and territories, develop and implement a pilot scheme for value-based payments for new antimicrobial drugs. This pilot should apply the lessons learned from the Australian Government's pilot scheme for payment for Hepatitis C drugs, as well as from overseas antimicrobial drug schemes.**

- **Promote the recent research and development tax initiatives internationally as a way of encouraging industry to look to Australia for future investments in the healthcare sector.**

- **Conduct a full review of the patent box scheme every two years after implementation to ensure it is operating effectively and driving increased expenditure and innovation within Australia.**

- **Collaborate with the states and territories to review the funding of the research and development sector in health care to distribute funding in a methodical way that provides sufficient support throughout the research funding 'pipelines'.**

  - **Noting the work underway through the Modern Manufacturing Program, the Committee supports the development of an updated roadmap to facilitate the manufacturing and commercialisation of novel drugs and technologies in Australia.**

The Patient Voice

Recommendation 28

11.29 The Committee recommends that:

- **The Department of Health integrate the patient voice upfront into the Health Technology Assessment system. Earlier patient engagement with the Health Technology Assessment system would include:**

  - **Representation from peak patient bodies that is refreshed every three – five years**
  - **Representation of Aboriginal and Torres Strait Islander Peoples.**

- **The Department of Health implement a notification system for all HTA bodies and the TGA to advise relevant patient groups of the receipt of an application.**

- **The Department of Health provide patients and stakeholders with a concise sponsor's submission summary to help facilitate their own involvement in the Health Technology Assessment process.**

- **The Department of Health should consider making patient evidence compulsory for certain applications, and should consider the role of patient evidence in the decisions of the Therapeutic Goods Administration.**

- **The Department of Health should notify relevant patient groups of the outcome of the assessment process by all HTA bodies.**

- **The Department of Health be funded to implement these recommendations.**

- **The Australian Government provide funding for organisations to support participation in the HTA process, including for very rare disease patient groups that have limited capacity for fundraising or access to alternative funding.**

Improving the HTA process

Recommendation 29

11.30 The Committee recommends that:

- **The Committee recommends that the Australian Government amend the *National Health Act 1953* (Cth) to formalise the role and powers of the Pharmaceutical Benefits Advisory Committee Executive. The scope of the Executive's role and powers should be determined by agreement between the Executive and the Department of Health.**

- **The Department of Health produce a pre-submission advice framework for submissions to the Therapeutic Goods Administration, Pharmaceutical Benefits Advisory Committee, Medical Services Advisory Committee and other Health Technology Assessment bodies, explaining the interaction between those bodies and their evidentiary and other requirements, to be provided to sponsors before they make their submissions.**

- **The independent Health Technology Assessment Review reassess relevant aspects of the Health Technology Assessment process to ensure there are future pathways for treatments and therapies that do not fit neatly into the current system such as rare cancers, antimicrobials, orphan drugs, and precision medicines.**

  - **It is imperative that appropriate clear pathways are considered for inclusion for paediatric medicines and technologies.**
  - **The Committee is of the clear view that precision medicine approval pathways will require a different application assessment than current approaches designed for treatments for common conditions, with large data sets and comparative evaluations.**

- **The Department of Health publish data on application processing times and positive recommendation rates for the Pharmaceutical**

Benefits Advisory Committee and other Health Technology Assessment bodies. In addition:

- **The Department of Health should publish Health Technology Assessment processing times annually, benchmarked against other nations with advanced HTA processes.**

- **The Australian Government, in collaboration with relevant stakeholders, develop a suite of clear and measurable benchmarks to track the Commonwealth's implementations of the recommendations made by the Committee and accepted by the Australian Government.**

  - **These agreed benchmarks along with measurable KPIs/metrics should be developed in such a way as to best facilitate the Department of Health, including its agencies and other relevant statutory bodies, in the tabling of an annual update to the Australian Parliament.**

Review of HTA

Recommendation 30

11.31 The Committee recommends that the Australian Government's independent Health Technology Assessment Review (which is scheduled to commerce in July 2022) consider and develop reforms in the following areas:

- **Reducing the frequency and need for applications to HTA bodies to be resubmitted.**

- **Streamlining the interaction between hospitals and the Health Technology Assessment system**

- **Streamlining the interaction of the Therapeutic Goods Administration, the Pharmaceutical Benefits Advisory Committee, the Medical Services Advisory Committee and other Health Technology Assessment bodies**

- **Cooperation and harmonisation between Australian Health Technology Assessment bodies and equivalent bodies overseas**

- **Improving the measurement of the performance of the Pharmaceutical Benefits Advisory Committee and the publication of data on that performance**

- **Improving the mechanisms for communication between sponsors and the Pharmaceutical Benefits Advisory Committee during the submission process**

- **Increasing the use of Managed Access Programs to facilitate earlier access to innovative medicines**

- **Increasing the use of Real World Evidence in Health Technology Assessment**

- **Improving flexibility when choosing a comparator in Health Technology Assessment**

- **Introducing a scoping process that includes patients and clinicians at an early stage to agree on the framework that the submission will be considered. This process could draw on the approach taken by the United Kingdom's National Institute for Health and Care Excellence**

- **Improving the independent review process for HTA decisions, including the potential for this to be made available to groups of patients and clinicians in addition to sponsors.**

MSAC

Recommendation 31

11.32 The Committee recommends that:

- **The Department of Health should consider, in consultation with state and territory governments, industry, patients and clinicians, the introduction of fees for Medical Services Advisory Committee applications on a cost recovery basis, if this is necessary to increase the speed and effectiveness of assessments. If fees are introduced they should have similar features to those recommended by the Committee for Pharmaceutical Benefits Advisory Committee fees (including those arrangements outlined at Recommendation 8).**

- **The Medical Services Advisory Committee increase the involvement of clinicians in its assessments of technologies with which its members lack relevant expertise.**

- **The Department of Health introduce an equivalent to the Managed Access Programs for medical devices. The details of this scheme including eligibility criteria and duration should be formulated in consultation with patient groups, clinicians and industry.**

- **The Therapeutic Goods Administration introduce parallel processing of applications with the Medical Services Advisory Committee.**

- **The Medical Services Advisory Committee increase opportunities for sponsors of particularly complex applications to present to it at its meetings and expand the opportunities for pre-submission meetings.**

- **The Medical Services Advisory Committee consider developing international collaboration for complex assessment proposals.**

- **The Department of Health expand the independent Health Technology Assessment Review in July 2022 to include Medical Service Advisory Committee processes.**

- **The Medical Services Advisory Committee publish a full calendar timeline of meeting agenda and outcomes, including dates when minutes and Public Summary Documents will be made public.**

- **The Medical Services Advisory Committee publish additional guidance for sponsors of digital health technologies.**

- **The Department of Health establish a benchmarking system for MSAC assessments, including benchmarking against comparable overseas organisations.**

Mr Trent Zimmerman MP

Chair

A. Submissions

1 Mr Robert Heron

2 Ms Rachel Rogers

3 Recordati Rare Diseases Australia

4 Fabry Australia

5 Ms Julia Burlison

6 Mrs Donna Greenhalgh

7 Specialised Therapeutics Australia

8 Cystic Fibrosis Australia

9 Mind Medicine Australia

10 Mirum Pharmaceuticals

11 Myeloproliferative Neoplasms Alliance Australia

12 Myeloma and Related Diseases Registry

- 12.1 Supplementary to submission 12

13 Mr Michael Smith

14 *Confidential*

15 Department of Health

- 15.1 Supplementary to submission 15
- 15.2 Supplementary to submission 15
- 15.3 Supplementary to submission 15
- 15.4 Supplementary to submission 15
- 15.5 Supplementary to submission 15
- 15.6 Supplementary to submission 15

- 15.7 Supplementary to submission 15

16 Australasian Sleep Association

17 Dr Falk Pharma Australia Pty Ltd

18 QIMR Berghofer

19 PFIC Network

20 *Confidential*

21 Myasthenia Alliance Australia

22 *Name Withheld*

23 Humane Research Australia

24 Migraine Australia Ltd

25 *Name Withheld*

26 Australian Pompe Association

27 Family Planning NSW

28 Stryker South Pacific

- 28.1 Supplementary to submission 28

29 Alpha-1 Organisation Australia Inc

- Attachment 1

30 Alexion Pharmaceuticals Australasia

- 30.1 Supplementary to submission 30

31 Amicus Therapeutics

32 Luminesce Alliance

33 Save Our Sons Duchenne Foundation

- Attachment 1

34 Merck Healthcare Pty Ltd

35 Sleep Disorders Australia & Hypersomnolence Australia

36 Sanfilippo Children's Foundation

37 Spinal Muscular Atrophy Association of Australia Inc.

38 Ms Fiona Mobbs

39 Eczema Support Australia

40 Miss Jessica Pace

41 ARCS Australia

42 AstraZeneca Australia

43 Australia and New Zealand Legal and Regulatory Affairs Committee of the International Society of Cell and Gene Therapy

44 Queensland Genomics Community Advisory Group

45 Mrs Pamela Bird

46 The GUARD Collaborative

47 Myriad Genetics Pty Ltd

48 *Name Withheld*

49 Dr Bruce Baer Arnold and Dr Wendy Bonython

50 Medical Oncology Group of Australia and Private Cancer Physicians of Australia

51 Rare Disease Industry Working Group

52 JDRF Australia

53 Australian Antimicrobial Resistance Network

54 Mrs Melissa Jose

55 Narcolepsy Australia

56 Painaustralia

57 DMTC Ltd

58 *Name Withheld*

59 Albireo Pharma, Inc.

60 Ms Patricia Pontynen

61 Victorian Comprehensive Cancer Centre

62 Macquarie University Centre for the Health Economy

63 Merck Sharp & Dohme Australia

64 MND Australia

65 Ipsen Pty Ltd

66 Takeda Pharmaceuticals Australia Pty Ltd

67 Australian Patient Advocacy Alliance

68 Australian Healthcare and Hospitals Association

69 Mrs Janna Linke

70 Noxopharm Limited

71 Patient Voice Initiative

- 71.1 Supplementary to submission 71

72 Dr Haitham Tuffaha

73 ausEE Inc.

74 UCB Australia Pty Ltd

- 74.1 Supplementary to submission 74

75 Medicinal Cannabis Industry Australia

76 Australian Cardiovascular Alliance

77 Duchenne Australia

78 Research Australia

79 Myositis Association - Australia Inc.

80 ViiV Healthcare

81 XLH Australia Inc.

82 Amgen Australia

- 82.1 Supplementary to submission 82
- 82.2 Supplementary to submission 82
- 82.3 Supplementary to submission 82
- 82.4 Supplementary to submission 82
- 82.5 Supplementary to submission 82
- 82.6 Supplementary to submission 82
- Attachment 1

83 Edwards Lifesciences Pty Limited

84 Children's Cancer Institute

85 MS Australia

86 Rare Voices Australia

87 Kyowa Kirin Australia

88 Association of Australian Medical Research Institutes

- 88.1 Supplementary to submission 88

89 Ms Monica Kurth

90 Mrs Vanessa Brown

92 Roche Australia

93 New South Wales Government

94 Australian Prescriber

95 Australasian Association of Nuclear Medicine Specialists and the Australian and New Zealand Society of Nuclear Medicine

96 ClinTrial Refer

97 Melbourne Academic Centre for Health

98 Australian Amyloidosis Network

99 Sanofi

- 99.1 Supplementary to submission 99

100 *Name Withheld*

101 Gilead Sciences

102 Gene Therapy Advisory Steering Group, Sydney Children's Hospital Network

103 Leukaemia Foundation

- Attachment 1
- Attachment 2

104 Blood Cancer Taskforce

- Attachment 1

105 The George Institute for Global Health

- Attachment 1

106 Results International Australia

107 Australian Nuclear Science and Technology Organisation

108 The Pharmacy Guild of Australia

109 Metabolic Dietary Disorders Association

110 Prader-Willi Research Foundation Australia

- 110.1 Supplementary to submission 110

111 3DMEDiTech

112 Australasian Leukaemia and Lymphoma Group and Haematology Society of Australia and New Zealand

113 Mr Rod Longmire

114 AusBiotech

115 The Australian and New Zealand Headache Society

116 Melanoma & Skin Cancer Advocacy Network

117 Migraine & Headache Australia

118 Bristol Myers Squibb Australia

- 118.1 Supplementary to submission 118
- Attachment 1

119 Haemophilia Foundation Australia

120 Australian and New Zealand Children's Haematology/Oncology Group

121 Mrs Deb Bailey

122 Medtronic Australasia

123 Melbourne Children's Campus

124 Dr Fiona West

125 Mito Foundation

126 Dr Anneke Blackburn

127 SCN2A Australia

128 Allergy & Anaphylaxis Australia

129 Western Australia Department of Health

130 Biotronik Australia Pty Ltd

- 130.1 Supplementary to submission 130

131 *Name Withheld*

132 Ms Michelle and Mr Eliot Jones

133 The University of Melbourne

134 Johnson & Johnson

135 Ovarian Cancer Australia

136 White Coats Foundation

137 Pfizer Australia

138 Novartis Australia and New Zealand

139 ITP Australia

140 Eli Lilly Australia

141 Medicines Australia

- 141.1 Supplementary to submission 141

142 Immune Deficiencies Foundation Australia

143 Lymphoma Australia

144 Carers and Patients of Braf V600E Colorectal Cancer

145 CSL Behring

- 145.1 Supplementary to submission 145

146 Varian Medical Systems Australasia

147 Australasian Society of Clinical Immunology and Allergy

- Attachment 1

148 Medical Technology Association of Australia

149 Australian Clinical Trials Alliance Ltd

150 Dr Susanne O'Malley

151 Novo Nordisk Oceania

152 BioMarin Pharmaceutical Australia Pty Ltd

153 Foundation for Angelman Syndrome Therapeutics Australia

154 Juvenile Arthritis Foundation Australia

- Attachment 1
- Attachment 2

155 NeuroEndocrine Cancer Australia

156 National Allergy Strategy

157 Maridulu Budyari Gumal

158 Monash Institute for Medical Engineering

- Attachment 1

159 Fragile X Association of Australia

160 Better Access Australia

161 Psychedelic Research In Science and Medicine Inc.

162 *Name Withheld*

163 Australian Rheumatology Association

164 BXTAccelyon Limited

165 WMozzies

166 Rare Cancers Australia

- Attachment 1
- Attachment 2

167 Rare Ovarian Cancer Inc.

168 Ms Dianne Spillane

169 The CF Pipeline Patient Interest Group

170 *Name Withheld*

171 *Confidential*

172 *Confidential*

173 *Confidential*

174 *Confidential*

175 Bayer Australia and New Zealand

176 *Confidential*

177 *Confidential*

178 Pathology Technology Australia

- 178.1 Supplementary to submission 178
- 178.2 Supplementary to submission 178

179 Centre for Law and Genetics, University of Tasmania and Sydney Health Law and Sydney Health Ethics, Sydney University

180 AbbVie Pty Ltd

181 Shawview Consulting

182 Red Cross Lifeblood and the South Australian Health and Medical Research Institute

183 InteliCare

- Attachment 1

184 Omico: Australian Genomic Cancer Medicine Centre

- Attachment 1

185 Sydney Children's Hospital Network and the Children's Medical Research Institute

186 *Confidential*

187 TALi Health

- 187.1 Supplementary to submission 187

188 Medistar

189 MedReleaf Australia

- 189.1 Supplementary to submission 189

190 National Aboriginal Community Controlled Health Organisation

- 190.1 Supplementary to submission 190

191 Abbott Diabetes Care

192 T1D Hub

193 *Confidential*

194 Dr Diane Sheehan

195 AccessCR Pty Ltd

196 Australian Federation of AIDS Organisations

197 Private Healthcare Australia

198 Sleepfit Solutions Pty Ltd

199 Mrs Lyndal Lemmey

200 FSHD Global Research Foundation

201 HealthMatch

202 Leo Pharma

203 Pharmaceutical Society of Australia

204 The Royal Australian and New Zealand College of Radiologists

205 Consumers Health Forum of Australia

206 BioScience Managers

207 Australian Commission on Safety and Quality in Health Care

B. Exhibits

1 St Vincent's Health Australia

C. Hearings and Witnesses

Thursday, 3 September 2020 - Canberra

Department of Health

- Ms Penny Shakespeare, Deputy Secretary, Health Resourcing
- Adjunct Prof John Skerritt, Deputy Secretary, Health Products Regulation
- Dr Jane Cook, Fist Assistant Secretary, Medicines Regulation
- Ms Tracey Duffy, First Assistant Secretary, Medical Devices and Product Quality
- Ms Adriana Platona, First Assistant Secretary, Technology Assessment and Access
- Dr Allyson Essex, Assistant Secretary, Health Economics and Modelling Branch
- Dr Masha Somi, Assistant Secretary, Health and Medical Research Office

Friday, 5 February 2021 - Canberra

Department of Health

- Prof Brendan Murphy, Secretary
- Adj Prof John Skerritt, Deputy Secretary, Health Products Regulation
- Dr Jane Cook, First Assistant Secretary, Health Products Regulation, Medicines Regulation
- Dr Grant Pegg, Assistant Secretary, Prescription Medicines Authorisation Branch, Medicines Regulation Division
- Ms Hope Peisley, Assistant Secretary, COVID-19 Vaccine Taskforce

Australian Technical Advisory Group on Immunisation (ATAGI)

- Prof Allen Cheng, Co-Chair

Thursday, 11 March 2021 - Sydney

Rare Voices Australia

- Prof Adam Jaffe, Member of Scientific and Medical Advisory Committee
- Mrs Nicole Millis, Chief Executive Officer
- Dr Kaustuv Bhattacharya, Scientific Advisor

Medical Technology Association of Australia

- Mr Ian Burgess, Chief Executive Officer
- Mr George Faithfull, Advisory, Vice-Chair Regulatory Affairs Strategic Committee
- Mr Paul Dale, Policy Director
- Dr Merrilyn Clancy, Senior Policy Officer

Bowel Cancer of Australia

- Mrs Joni Thomes, Communications & Strategic Communications

JDRF Australia

- Mr Mike Wilson, Chief Executive Officer

Allergy & Anaphylaxis Australia

- Ms Maria Said, Chief Executive Officer

Fabry Australia

- Mrs Sheridan Campbell, Chair

Migraine and Headache Australia

- Mr Carl Cincinnato, Lead

Australian Pompe Association

- Mr Raymond Saich, President

Save Our Sons Duchenne Foundation

- Mr Lance Dale, Advocacy Officer

Cystic Fibrosis Australia

- Mrs Annette Burke, Chief Executive Officer

Alpha-1 Organisation Australia Inc.

- Dr Gaynor Heading, President

Macquarie University Centre for the Health Economy

- Prof Martin Hoyle, Professor of Health Innovation and Evaluation

Australasian Association of Nuclear Medicine Specialists

- Dr Geoff Schembri, President

Australian and New Zealand Society of Nuclear Medicine

- Prof Dale Bailey

Australian Nuclear Science and Technology Organisation

- Mr Shaun Jenkinson, Chief Executive Officer (Acting)

Dr Susanne O'Malley, Private capacity

Mr Michael Smith, Private capacity

Shawview Consulting

- Dr Brendan Shaw, Principal

Medical Oncology Group of Australia

- Dr Deme Karikios, Deputy Chair

Friday, 12 March 2021 - Sydney

Merck Healthcare Pty Ltd

- Ms Leah Goodman, Managing Director Australia and New Zealand

Biogen Australia and New Zealand

- Dr Kylie Bromley, Managing Director

Pfizer Australia

- Ms Louise Graham, Director, Head of Market Access
- Ms Anne Harris, Country Manager

Merck Sharp & Dohme Australia

- Mr Michael Azrak, Managing Director

Amgen Australia

- Mr Ian Noble, Director, Value, Access & Policy

AbbVie

- Mr Chris Stemple, Vice President and General Manager, Australia and New Zealand

Novartis Australia and New Zealand

- Mr Richard Tew, Country President and Pharma Country Head

Sydney Children's Hospital Network and Children's Medical Research Institute

- Prof Ian Alexander, Head, Gene Therapy Research Unit

Kids Research, Sydney Children's Hospital Network

- Dr Wendy Gold, Chair Gene Therapy Advisory Steering Group & Head of Molecular Neurobiology Research Group

BXTAccelyon Limited

- Mrs Nicola Leavold, Commercial Director

Medtronic Australasia

- Ms Liz Carnabuci, Vice President & Managing Director, ANZ

Biotronik Australia Pty Ltd

- Ms Jan Ewert, Managing Director

Edwards Lifesciences Pty Ltd

- Mr Pat Williams, Managing Director

Stryker South Pacific

- Mr Maurice Ben-Mayor, President

Maridulu Budyari Gumal, Sydney Partnership for Health, Education, Research and Enterprise (SPHERE)

- Dr Peter Spencer, Medtech Innovation Advisor and HealthHatchery Co-Founder
- Rowena Tucker, Interim Executive Director

Friday, 26 March 2021 - Canberra

Medicines Australia

- Ms Elizabeth de Somer, Chief Executive Officer
- Dr Anna Lavelle, Chair
- Mr Jamie Snashall, Head of Government Relations
- Mr Mark Stewart, Manager Research and Data

Pharmacy Guild of Australia

- Mr Philip Chindamo, Group Executive Health Economics
- Mr Chris Flood, Senior Pharmacist and National Manager PBS Operations & Strategy

MND Australia

- Mr David Ali, Chief Executive Officer
- Dr Gethin Thomas, Executive Director Research

Painaustralia

- Ms Carol Bennett, Chief Executive Officer

Dr Bruce Baer Arnold, Private capacity

Thursday, 22 April 2021 - Melbourne

Australian Patient Advocacy Alliance

- Ms Deidre MacKechnie, Executive Officer

Myeloma and Related Diseases Registry

- Prof Andrew Spencer, Chief Investigator

Prader-Willi Research Foundation Australia

- Dr Elizabeth Patterson

Psychedelic Research In Science and Medicine Inc.

- Ms Stephanie Tzanetis, Member

GUARD Collaborative Australia

- Ms Monica Ferrie, Founder

SCN2A Australia

- Ms Kris Pierce, Founder

Metabolic Dietary Disorders Association

- Ms Louise Healy, Vice President

Specialised Therapeutics Australia

- Mr Carlo Montagner, Chief Executive Officer

ViiV Healthcare

- Mr Michael Graham, Country Manager

CSL Behring

- Ms Loretta Croker, General Manager

Australian Antimicrobial Resistance Network

- Dr Daniel Grant, Managing Director and Chief Executive Officer and Co-Chair

DMTC Ltd

- Dr Mark Hodge, Chief Executive Officer

Association of Australian Medical Research Institutes (AAMRI)

- Dr Jacqui Waterkeyn, Member

Ipsen Pty Ltd

- Mr Peter Koetsier, Head of Asia Pacific (excluding China) & General Manager of Australia and New Zealand

3DMEDiTech

- Mr Jason Aldworth, Chairman

Friday, 23 April 2021 - Melbourne

UCB Australia

- Ms Selina Clifford, Head of Neurology & Country Lead

Gilead Sciences

- Ms Jaime McCoy, General Manager

Bristol Myers Squibb Australia

- Mr Neil MacGregor, Managing Director

Monash Institute of Medical Engineering

- Prof John Forsythe, Co-Director
- Prof Helena Teede, Co-Director

Research Australia

- Mr Greg Mullins, Head of Policy

Melbourne Academic Centre for Health

- Prof Andrew Davidson, Medical Director, Melbourne Children's Trials Centre and Chair Clinical Trials Committee

University of Melbourne

- Dr Heather St John, Director, Research and Enterprise Development, Research, Innovation and Commercialisation

Melbourne's Children's Campus

- Prof Andrew Davidson, Medical Director, Melbourne Children's Trials Centre

White Coats Foundation

- Mrs Christine Zahren, Director

Victorian Comprehensive Cancer Centre

- Prof Grant McArthur, Executive Director

Melanoma & Skin Cancer Advocacy Network

- Ms Tamara Dawson, Director

Ovarian Cancer Australia

- Ms Jane Hill, Chief Executive

NeuroEndocrine Cancer Australia

- Ms Simone Leyden, Chief Executive Officer and Co-Founder

Leukaemia Foundation

- Mr Tim Murphy, General Manager, Blood Cancer Partnerships

Blood Cancer Taskforce

- Prof John Seymour, Co-Chair

Australasian Leukaemia and Lymphoma Group

- Ms Delaine Smith, Chief Executive Officer

Haemophilia Foundation Australia

- Ms Sharon Caris, Executive Director

Australian and New Zealand Children's Haematology/Oncology Group

- Acting Prof Jordan Hansford, Director, CNS Group Chair

Friday, 7 May 2021 - Sydney

Alexion Pharmaceuticals Australasia

- Ms Sara Trafford-Jones, Vice President - South Cluster Lead (Brazil, Colombia, Turkey); General Manager Australian/New Zealand/China

Kyowa Kirin Australia

- Mr Simon Dawson, General Manager

BioMarin Pharmaceutical Australia Pty Ltd

- Dr Kathryn Evans, Area Director, Australia and New Zealand

Recordati Rare Diseases Australia

- Mr Anthony Shelton, General Manager

Takeda Pharmaceuticals Australia Pty Ltd

- Mr Brad Edwards, General Manager, Australia and New Zealand

Sanofi

- Ms Vanessa Xavier, Head of Market Access, Australia and New Zealand

Varian Medical Systems Australasia

- Mrs Rebecca Cortiula, Senior Managing Director, Australasia

Amicus Therapeutics

- Dr Simon McErlane, Medical Director, APAC

Myriad Genetics Pty Ltd

- Ms Andrea Tesoriero, Associate Director, Asia Pacific

Roche Australia

- Mr Stuart Knight, General Manager

Eli Lilly Australia

- Mr Benjamin Basil, President and General Manager Australia, New Zealand and North Asia-Pacific

Novo Nordisk Oceania

- Mr Jeppe Theisen, Vice President and General Manager

Bayer Australia and New Zealand

- Mr Ashraf Al-Ouf, General Manager

Luminesce Alliance

- Acting Professor Michelle Farrar, Clinician representative from the University of New South Wales, a partner in the Luminesce Alliance

Rare Cancers Australia

- Mr Richard Vines, Chief Executive

Australasian Society of Clinical Immunology and Allergy

- Professor Connie Katelaris AM, Chair, Drug Allergy Committee and ASCIA HAE, CSU and CRNP Working Parties
- Ms Jill Smith, Chief Executive Officer
- Dr Theresa Cole, President Elect and Co-Chair, Immunodeficiency Committee
- Dr Melanie Wong, Co-Chair, Immunodeficiency Strategy
- Professor Jo Douglass, Co-Chair, Immunodeficiency Strategy
- Professor Michaela Lucas, President

Australia and New Zealand Legal and Regulatory Affairs Committee of the International Society of Cell and Gene Therapy

- Dr Gabrielle O'Sullivan, Co-Chair

Australian Clinical Trials Alliance

- Professor John Zalcberg OAM, Chair

The George Institute for Global Health

- Professor Bruce Neal, Executive Director, Australia

The University of New South Wales

- Professor Louisa Jorm, Professor, Faculty of Medicine and Director, Centre for Big Data Research in Health

Monday, 17 May 2021 - Brisbane

Patient Voice Initiative

- Jessica Bean, Chair

Migraine Australia

- Dr Raphaella Kathryn Crosby, Founder and Campaigns Director

Eczema Support Australia

- Mrs Ruth Duggan, Project Team Member

XLH Australia Inc.

- Mrs Naomi Elizabeth Ford, Vice President

Narcolepsy Australia

- Mrs Melissa Jose, President

Ms Amanda Vernon, Private capacity

Sleep Disorders Australia and Hypersomnolence Australia

- Mrs Michelle Chadwick, Chairperson

Foundation for Angelman Syndrome Therapeutics Australia

- Mrs Meagan Cross, Chairperson

QIMR Berghofer

- Professor Fabienne Mackay, Director and Chief Executive Officer

Results International Australia

- Mr Mark Rice, Policy and Advocacy Manager

Dr Haitham Tuffaha, Private capacity

MedReleaf Australia

- Mr Nathan Davis, Director - Head of Business Development
- Mr Russell Harding, Executive Chairman and Chief Executive Officer
- Mr Hugh Tait, Pharmacist

Medistar

- Ms Jeneth Boughen, Owner and General Manager

Tuesday, 18 May 2021 - Brisbane

ausEE Inc.

- Mrs Sarah Gray, President and Founder

Myasthenia Alliance Australia

- Mrs Susan White, Chairperson

ITP Australia

- Mrs Danielle Boyle, Chief Executive Officer

Lymphoma Australia

- Ms Sharon Winton, Chief Executive Officer

Australian Amyloidosis Network

- Dr Peter Mollee, Executive Committee Member
- Mrs Patricia Neely, Patient and Family Advocate and Member of the Australian Amyloidosis Network Management Team

Queensland Genomics Health Alliance Community Advisory Group

- Dr Erin Evans, Chair

Queensland Genomics Health Alliance Community Advisory Group

- Mr David Bunker, Executive Director
- Ms Louise Healy, Member

AusBiotech

- Ms Michelle Burke, Chair
- Ms Lorraine Chiroiu, Chief Executive Officer

Friday, 18 June 2021 - Canberra

National Aboriginal Community Controlled Health Organisation

- Dr Dawn Casey, Deputy Chief Executive Director
- Mr Mike Stephens, Director, Medicines Policy and Programs

Pathology Technology Australia

- Dr John Melki, Member
- Ms Susan Martland, Member
- Mr Dominic De Souza, Member
- Mr Dean Whiting, Chief Executive Officer

Department of Health

- Adjunct Professor John Skerritt, Deputy Secretary, Health Products Regulation
- Ms Tracey Duffy, First Assistant Secretary, Medical Devices and Product Quality, Health Products Regulation
- Ms Trish Garrett, First Assistant Secretary, Cancer Hearing and Program Support

- Ms Adriana Platona, First Assistant Secretary, Technology Assessment and Access
- Ms Thea Connolly, Assistant Secretary, Office of Health, Technology Assessment Branch
- Dr Megan Keaney, Principal Medical Adviser, Technology Assessment and Access

Thursday, 24 June 2021 - Canberra

Pharmaceutical Benefits Advisory Committee, Department of Health

- Professor Andrew Wilson, Chair
- Ms Jo Watson, Deputy Chair

Wednesday, 7 July 2021 - Canberra

United Kingdom National Institute for Health and Care Excellence

- Mr Meindert Boysen, Deputy CEO and Director of The Centre for Health Technology Evaluation