16 September 2020

Time To Move a Mountain

Cystic Fibrosis Australia (CFA) welcomes the Parliamentary Inquiry into new drugs and novel technologies. The Inquiry by the House of Representatives Standing Committee on Health, Aged Care and Sport will include a focus on access to treatment for rare diseases and conditions where there is high and unmet need. In other words, trying to eliminate the mountainous obstacles we so often see in front of us.

The Inquiry will be chaired by Trent Zimmermann MP and Dr Mike Freelander MP and CFA has written to both asking to be included in the HTA review. All CF drugs go through the Health Technology Assessment (HTA) process and we would all like it to be a swifter process. Let's have our say and try and move one enormous mountain out of the way because quite simply, it's time to level the playing field.

We believe that the Cystic Fibrosis (CF) Community has a wealth of knowledge and real-life experiences that should be shared. They will help ensure that the Inquiry's outcomes benefit the communities in need.

Consumer submissions addressing the Terms of Reference (ToR) must be submitted by Tuesday 13 October 2020 and CFA has developed key points to assist you in the process.

We have added suggested consumer comments for each ToR point below and we encourage the CF Community to use this information as a guide. Your own words will be so much more powerful.

The Inquiry's ToR points are designed to ensure Australia is well positioned to access new drugs and technologies in a timely manner in the future.

Your personal stories and heartfelt experience are incredibly important. These will really make the difference and have a considerable impact. With that in mind we encourage you to share this information if you feel comfortable about doing so.

It is also important when looking at the ToR Topics to consider what has not been included. CFA sees this as a vital component of the ToR review and we need to be sure the Inquiry is robust and inclusive. You do not have to address all the ToR topics. Just focus on those that have impacted your life.

As we live in a digital world the House of Representatives Inquiry will accept submissions online in the form of a letter, a short document, a more substantial proposal or a video. More information is available here [Preparing a submission to an inquiry]

ToR Topics

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.

Current Issues:

a. Speed to market in Australia when drugs are available overseas
b. Lack of transparency by pharmaceutical companies regarding their submissions
c. The HTA process and timeline relating to a specific drug or treatment should be agreed to by Government departments, Regulators and Sponsor and shared with consumers to manage expectations
d. Consumer consultation should be held at TGA stage and prior
e. Consumer co-design should be rewarded.

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.
   Current Issues:

a. Innovative trial should be encouraged and incentivised – N of 1, adaptive, organoids and basket trials are all available in Australia – approval pathways for rare diseases should be established
b. Double blind placebo clinical trials are no longer the only option or 'best practice' method especially in rare diseases
c. Treatments should be personalised, and precision medicine embraced and encouraged
d. Research into 'evidence gaps' for rare diseases should be funded
e. Repurposing of existing treatments should be incentivised, and acceleration pathways established.

3. Measures that could make Australia a more attractive location for clinical trials for new drugs and novel medical technologies
   Current Issues:

a. Support for disease specific clinical trial networks
b. Incentivise international clinical trials to include Australia by providing benefits such as an expedited HTA approval process when Australian data is available
c. National infrastructure for clinical trials
d. Consumer co-design
e. Streamlined national ethics approval process
f. Every Australian should have access to clinical trials
g. Not for Profit Consumer bodies should be supported to aid clinical trial participant recruitment
h. Embrace innovative clinical trials that include rural and remote communities.

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.
   Current Issues:

a. Open collaboration with FDA and EMA
b. Clinical registries should be accredited and then part funded by Government for use in the drug evaluation process
c. International reimbursement contact negotiations
d. Allow immediate access to life saving drugs following TGA approval and allow the PBAC process and negotiations to run while consumers benefit from treatments. This would also provide valuable data through post marketing surveillance
e. Set time limits for commercial (pricing) negotiations
f. New clinical trial techniques should be valued in the reimbursement process
g. Add consumers to the HTA process from the beginning
h. Require consumer comments to Pharma TGA and PBAC submissions
i. Provide support, educate and update consumers throughout the process
j. Incentivise compassionate access for a great number of people who are critically ill.

I realise this is a great deal of information to digest so as always CFA is here to help. If you have any questions, please email nickim@cfa.org.au and we will get back to you with a solution.

You can also contact the Government Inquiry Secretariat on 02 6277 4145 or Health.reps@aph.gov.au. There are three ways to deliver your submission…

1. create or log in to My Parliament and set up an account
2. email Health.reps@aph.gov.au
3. email Nicki at CFA and we will submit for you – her email is nickim@cfa.org.au.

Thank you to everyone who decides to support CFA's Health Technology Assessment advocacy. It will help eliminate the obstacles and pave the way for faster access to drugs in the future for people with CF. This is a mountain well worth moving because a level playing field is everyone's right and in every society's best interests.

Kind regards

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