




Research Review

The Australian Cystic Fibrosis
Research Trust 2017/2018



The LJ Hooker Foundation has a long history of supporting local communities and it takes pride in the way it gives back. LJ Hooker has raised over \$5.5 million for the Australian Cystic Fibrosis Research Trust (ACFRT) and Cystic Fibrosis Australia (CFA) over the past 20 years through its 'Partnership in Caring'.

LJ Hooker Founder, Sir Leslie Hooker, was a great believer in supporting the community. "Real estate is not about houses, it's about people" is a phrase that has been quoted for generations in the LJ Hooker family and still holds true today.

In September 2018, LJ Hooker celebrates its 90th birthday. CFA and the ACFRT are honoured to have such a wonderful relationship with The LJ Hooker Foundation and its many offices and franchises around Australia and wish them all the best for this incredible birthday milestone. It is a true testament to their ethic and community spirit.





TABLE OF CONTENTS

2	The ACFRT	History and Achievements
3	A Message from the President	
4	Dr Paul Robinson	Innovation Grant 2018
5	Dr Ama-Tawiah Essilfie	Innovation Grant 2018
6	Dr Jay Horvat	Innovation Grant 2017
7	Dr Shafagh Waters	Innovation Grant 2017
8	Dr André Schultz	Innovation Grant 2017
9	Professor Peter Wark	Mental Health Screening Trial
10	Associate Professor Michael Kelso	Special Grant
11	Professor Peter-John Wormald	Special Grant 2018
12	Chantelle Carpentieri	Special Grant 2018
13	Professor Scott Bell	Special Grant
14	Associate Professor David Parsons	Special Grant
15	Professor Stephen Stick	Little Lungs Big Futures
16	Dr Sharon Wong	Ann Maree Bosch Career Fellowship 2018
17	Dr Abdullah Tarique	Ann Maree Bosch Career Fellowship 2017
18	Zara Sheikh	ACFRT Top-up Scholarship 2018
19	Dr Simone Visser	ACFRT Top-up Scholarship 2017
20	Abbey Sawyer	ACFRT Top-up Scholarship 2017
21	Harry Tjondro	ACFRT Top-up Scholarship 2017
22	Samuel Montgomery	Hardie Foundation Scholarship 2017
23	Kelly Martinovich	CFWA Golf Classic Scholarship 2017
24	Meet the ACFRT Trustees	

History and Achievements



In 1989 the Australian Cystic Fibrosis Research Trust (ACFRT) was founded by a group of parents, patients and doctors to secure resources to support cystic fibrosis (CF) research in Australia.

Today after funding more than 300 research projects valued at over \$6 million, the ACFRT is recognised as Australia's largest and most prolific CF research body.

The ACFRT's remit is to secure funding from public, private and corporate entities and allocate funds for high quality peer reviewed research.

The ACFRT is passionate about ground breaking, innovative research concepts that consider new treatments and models of care that ultimately improve life expectancy and quality of life for people with CF.

Attracting the brightest young minds into cystic fibrosis medicine, allied health, mental health and research is also a key priority and former ACFRT grant recipients are a testament to this.

The ACFRT is administrated by Cystic Fibrosis Australia and supported by CF organisations from all states and territories.

A MESSAGE FROM THE PRESIDENT



It is a pleasure to introduce the Australian CF Research Trust (ACFRT) to the community. The ACFRT has been in operation since 1989, working hard to improve the lives of those affected by CF by funding innovative research and supporting young scientists to become involved in the search for answers.

The Trustees feel that while we have been supporting research and have funded hundreds of projects over the past 29 years, many of which have led to changes in treatment, we should take this opportunity to promote the work we have supported and the aims of the ACFRT.

The original Trustees, Mr Wal Riddell, Mr Conrad Guerra and myself, along with Cystic Fibrosis Australia (CFA) and all of the state and territory CF organisations believed that setting up a structure that could implement best practice in assessing projects and pooling research funds is vital. This would provide us with more and better opportunities to support the search to improve treatment and quality of life for people facing the daily challenges of living with CF.

As you will see in the following pages we have attained a number of successes and continue to support innovative projects and young researchers with our limited funds. This outlines our history and some of the wonderful outcomes that have come from the funding we have been able to provide through the generosity of the Australian community.

It is important to note that 100% of the funds raised or donated to the ACFRT are used to support research. The support of CFA in providing the operational funds to allow this is invaluable.

On behalf of the Trustees I commend this new resource to you.

A handwritten signature in white ink, which appears to read 'Mitch Messer', is positioned above the printed name.

Mitch Messer
President, ACFRT

PROJECT: Detailed Characterisation of Structure-Function Relationships in Mild Cystic Fibrosis Lung Disease and Validation of an Ultra Low Dose High Resolution Computerised Tomography (HRCT) Scanning Protocol

Innovation Grant - 2018

INSTITUTION: Children's Hospital, Westmead & The Airways Physiology and Imaging Group at the Woolcock Medical Research Institute, NSW

\$80,000 | 1 Year

Improving survival outcomes relies heavily on the accurate detection and assessment of early CF lung disease in children with CF by earlier treatment intervention and the consequent prevention of lung damage.

Traditionally, primary assessment of lung function has been through the use of spirometry. HRCT has now confirmed that progressive scarring of the lungs (bronchiectasis) can be present despite normal spirometry and that by the time a child has reached school age, lung damage is well established.

Newer, more sensitive techniques such as Multiple Breath Washout and HRCT now exist to help but the optimal way to use them requires further investigation. In addition, radiation associated with CT scans remains a concern for physicians.

This project will generate data to compare novel peripheral airway function tests with structural changes evaluated using HRCT and assess the potential to significantly reduce the radiation dose associated with HRCT screening.



RESEARCHER

Dr Paul
Robinson

RESEARCHER

Dr Ama-Tawiah Essilfie



PROJECT: Novel Multi-Omic Insight into Evolution of Antibiotic Resistance in *Pseudomonas aeruginosa* (PA) in CF and Relationship to Clinical Outcomes

Innovation Grant - 2018

INSTITUTION: Lung Infection and Inflammation Group at QIMR - Berghofer Institute of Medical Research, QLD

\$80,000 ACFRT, CFSA, CFACT, CFTAS & CFCC (Vic) | 1 Year

Multi-antibiotic resistant PA is commonly found in CF patients. Persistent infection with PA is associated with worsening lung disease and more frequent hospitalisations. Resistance may be naturally occurring or be acquired following years of exposure to antibiotic therapy, making PA very difficult to treat.

Many people with CF are infected with epidemic, antibiotic multi-resistant strains of bacteria. Despite progress in understanding antibiotic resistant mechanisms, little has changed in how antibiotics are prescribed. Using state of the art gene sequencing techniques, this project will investigate why antibiotic resistance is so common in epidemic PA compared to non-epidemic strains and whether this increased resistance causes more severe disease.

Additionally, the project will investigate the potential of a new technique for quantifying antibiotic resistance in sputum as an improved method of antibiotic selection compared to conventional methods. If successful, this could lead to improved clinical outcomes and shortened hospital stays.

RESEARCHER

Dr Jay Horvat

PROJECT: Investigating the Role and Therapeutic Targeting of Iron in Cystic Fibrosis

Innovation Grant - 2017

INSTITUTION: University of Newcastle, NSW

\$80,000 | 1 Year

More effective strategies are desperately needed to improve efficacy for the treatment of *Pseudomonas aeruginosa* (PA) infection in CF to improve patient outcomes.

Dr Jay Horvat is working with Professors Philip Hansbro and Peter Wark exploring high iron levels in the lungs of people with CF, which leads to increased susceptibility to infection with PA, increased pulmonary exacerbations and subsequent disease progression.

Previous studies have shown that in clinically stable CF patients there is a strong positive correlation between sputum iron and PA levels. These findings suggest increased iron in CF patients is a potential causal factor of PA persistence.

A novel combination of experimental models of increased iron loading in the lung and PA infection in conjunction with clinical investigations will be used to better understand the role of iron, possibly identifying it as a biomarker for CF exacerbations. In addition, it is hoped to determine whether therapeutically suppressing increased iron levels in the airways improves the clearance of PA infection from the lungs.





PROJECT: RNA Therapeutics: Novel Paradigm in Mutation Independent CF Therapy (BGas gene manipulation)

Innovation Grant - 2017

INSTITUTION: School of Women's and Children's Health, UNSW -
Australasian Centre for Personalised Cystic Fibrosis Medicine, NSW

\$70,000 Cystic Fibrosis Research Limited | 1 Year

RESEARCHER **Dr**
Shafagh
Waters

Recent development of new molecular therapies for the treatment of CF that restore function to the defective Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) protein are only effective in some people with CF. This study examines how a gene (called BGas) can be genetically manipulated for CF therapy. When BGas is removed from the cell, CFTR function is restored. This therapy will be beneficial to the majority of CF patients.

Miniaturised versions of a CF patient gut (known as mini-gut), using the patient's own cells as starting material are created in the lab. BGas-removing compounds are then added to the mini-gut to identify those responsive to BGas therapy.

The invaluable results from this project could drive future trials, delivering BGas-removing compounds directly to CF patients. A similar approach has had promising outcomes for children with spinal muscular atrophy.

PROJECT: BEAT CF – A Web-Based Platform to Support an Adaptive Model of Care for Managing CF

Innovation Grant - 2017

INSTITUTION: Telethon Kids Institute, WA

\$30,000 | 1 Year

How to best manage infectious exacerbations is not known despite dozens of trials over the past four decades involving hundreds of patients. With every infection roughly a quarter of people with cystic fibrosis lose lung function that is never recovered.

Optimising therapy is essential if we are to extend life expectancy.

The Innovation Grant supports the building of a BEAT CF data entry and randomisation web-interface. This web-based tool will capture both clinician reported outcomes plus patient reported symptoms as well.

The platform will have three separate portals catering to the needs of clinicians, researchers and patients.

Patient reported outcomes and feedback from families and carers is a highly desirable feature and will be clinically informative and time saving for multidisciplinary teams and in turn – and of paramount importance – advancing the care of people with CF.



RESEARCHER

Dr André
Schultz

RESEARCHER

Professor Peter Wark

PROJECT: Mental Health Screening Trial

Special Grant sponsored by The Cystic Fibrosis Foundation (CFF)

INSTITUTION: John Hunter Hospital and University of Newcastle, NSW

\$140,000 | 2 years



Following publication of the International Guidelines for screening mental health symptoms in 2016, CFF has funded 120 Mental Health Coordinators (MHC) in the USA.

Based on the US experience this project aims to intensively train two pilot CF centres in Newcastle (paediatric - 105 children; and adult - 110 adults) to implement the mental health screening protocol and collect data over a two-year period. A MHC will be appointed at each site, trained by US-based consultants.

Data will be recorded and automatically scored using software via preprogrammed iPads in each of the clinics, also determining the frequency and intensity of repeat testing based on results from the previous visit.

The centres will also be trained in the creation of a referral network of evidence-based providers who can offer counselling to parents or patients who request it. At the end of the first year the US-based trainers will assist the centres in developing a survey of patients and providers to evaluate their perceptions of the experience.

PROJECT: Multi-Action Antibiotics
to Treat Chronic Biofilm Infections

Special Grant

INSTITUTION: University of Wollongong, NSW

\$588,687 | 3 years

Biofilms from the bacterium *Pseudomonas aeruginosa* (PA) often build up in the lungs of people with CF. They contain large populations of bacterial cells and are encapsulated within gum-like materials which protect bacteria against the action of antibiotics and against the action of cells in the patient's immune system. Antibiotic resistance can be increased up to 1,000-fold in biofilms.

The UOW research team were the first to discover that low concentrations of nitric oxide (NO) can act as a signal that triggers bacteria in biofilms to disperse. When this happens the bacteria become more sensitive to antibiotics and to the body's immune system.

This project investigates the effects of combinations of NO-releasing compounds with antibiotics (cephalosporins) to develop a new way of targeting delivery of NO to biofilms. These compounds have the potential to be used as 'all-in-one' anti-biofilm antibiotics.

RESEARCHER

Associate Professor
**Michael
Kelso**





RESEARCHER

Professor
Peter-John
Wormald

PROJECT: A Novel Treatment Strategy
for CF *Staphylococcus aureus*
Associated Chronic Rhinosinusitis (CRS)

ACFRT in conjunction with CFSA Special Grant - 2018

INSTITUTION: Adelaide and Flinders Universities, SA

\$60,000 | 1 Year

CRS is one of the most common manifestations in patients with CF contributing to CF lung disease.

The frequent and often long-term use of antibiotics to treat relapsing airway infections significantly contributes to the threat of Multi Drug Resistant (MDR) pathogens. There is an urgent need to develop new treatments that are effective at eliminating infections with MDR pathogens.

Bacteriophage (phage) is a virus that targets and kills one specific bacterial species. Recently, in collaboration with Amplphi Biosciences, Professor Wormald's team successfully completed a clinical trial to test the safety and efficacy of a phage cocktail to treat CRS caused by *Staphylococcus aureus* in humans. The treatment has been further improved to develop a novel phage-based formulation that is now able to eradicate rather than reduce MDR *Staphylococcus aureus*.

This project is a collaborative effort between Professor Wormald's Rhinology team with CF centres in Adelaide and at the Royal Brompton Hospital in the UK. The team aims to optimise the delivery of the new formulation for topical delivery to the sinus region in CF patients.

PROJECT: Lentiviral Mediated Airway Gene Therapy:
Is Lysophosphatidylcholine Required for Safe and
Effective Gene Transfer in a CF lung?

ACFRT in conjunction with CFSa Special Grant - 2018

INSTITUTION: The University of Adelaide, & Women's and Children's Hospital, SA

\$30,000 | 3 years

Regardless of mutation type, current treatments for CF are neither preventative nor curative. Correction of the faulty CF transmembrane conductance regulator (CFTR) gene is recognised as the most likely method to prevent, halt or treat CF airway disease.

In previous work on mouse cells using a unique, two-step treatment protocol the group was able to demonstrate successful facilitation of a properly functioning CFTR gene transfer directly into mouse nasal CF airway cells. Step one prepares the airway cells using an airway conditioning compound and once receptive, step two involves administration of a single dose of the specially designed lentiviral CFTR gene vector to produce functional CFTR correction.

Using an established, highly specialised CF rat colony which will act as an appropriate CF animal model, this project aims to examine the safety and efficacy of the newly developed gene transfer methods for potential future use in the treatment of CF lung disease.



RESEARCHER

Dr
Chantelle Lee
Carpentieri

RESEARCHER

Professor Scott Bell



PROJECT: Antimicrobial Resistance
In CF-International Taskforce

Special Grant

INSTITUTION: Department of Thoracic Medicine, The Prince Charles
Hospital and QIMR Berghofer Medical Research Institute, Brisbane, QLD

\$5,000 | 1 year

Given the high use of antibiotics in CF patients, antimicrobial resistance (AMR) is commonly encountered in bacteria and fungi isolated from the airways of people with CF. As survival of people with CF increases, so will their exposure to antibiotics.

With international and multidisciplinary representation the Taskforce will develop guidelines on the interpretation of AMR for clinical care in CF and provide guidance on the role of AMR in clinical trials while addressing the following objectives:

- Understand how chronic infections differ from acute infections with respect to the microbiological assumptions regarding AMR.
- Describe current and developing methodologies for determining AMR.
- Assess the value of current susceptibility testing including its frequency and timing.
- Offer guidance for the use of AMR testing in the conduct of clinical trials by the pharmaceutical industry and regulatory agencies.
- Set key research priorities for the development of appropriate future application of AMR diagnostics to improve patient outcomes.
- Explore how monitoring antibiotic usage impacts on AMR in people with CF.

PROJECT: Determining Factors that Influence the Acceptance and Adoption of a Gene Therapy for CF Airway Disease

Special Grant

INSTITUTION: University School of Health Sciences, Flinders University & The Cystic Fibrosis Airway Research Group at the Women's and Children's Hospital, SA

\$5,000 | 1 Year

This project aims to use the combined expertise and resources of the groups at both institutions to assess the perceived acceptability of and public support for gene therapy for CF.

The primary focus of the WCH group is on developing a gene therapy treatment for CF using their expertise in molecular analysis and animal models. Over the last 15 years the WCH group has developed an extremely promising viral gene vector delivery system using a modified lentivirus designed to produce a lasting and effective treatment for CF airway disease. Flinders University provides expertise in health psychology and the understanding of attitudes and beliefs in relation to treatments and health outcomes.

Findings will contribute to the effectiveness of future applications for funding by including assessment of the feasibility of the approach from both the scientific/biological perspective and CF community acceptance. It also aims to develop education programs to better inform the CF community about the risks and benefits of this type of treatment.

RESEARCHER

Associate Professor David Parsons

Dr Martin Donnelly & Dr Ivanka Pritchard





RESEARCHER

Professor Stephen Stick

PROJECT: Little Lungs Big Futures

Little Lungs Big Futures

INSTITUTION: Telethon Kids Institute, WA

Ongoing Capital Campaign with a goal of \$10,000,000

Little Lungs Big Futures (LLBF) is the largest research project embarked upon by the CF Federation in Australia. It is a multi-pronged, multidisciplinary approach that aims to reduce structural lung damage in infants under five years of age by 50% within five years and add 20 years to life expectancy.

The research is being undertaken by The Australian Respiratory Early Surveillance Team for Cystic Fibrosis (AREST CF), a global network of cystic fibrosis researchers led from Perth.

The strategy focuses on early detection and intervention, including new imaging techniques and personalised medicine to alter the trajectory of the disease.

The recently created Cystic Fibrosis Biobank (BANKCF) allows collaboration across the world using samples collected in Perth and Melbourne.

A new research platform has been developed to address antimicrobial resistance, including the use of bacteriophages, as an alternative to antibiotics.

The AREST CF program has been cited more than 1,500 times since 2014.

PROJECT: RNA-Based Therapeutics for
Mutation-Independent CF Therapy

Ann Maree Bosch Career Fellowship 2018

INSTITUTION: School of Women's and Children's Health, UNSW -
Australasian Centre for Personalised Cystic Fibrosis Medicine, NSW

\$10,000 | 1 Year

This project aims to develop an effective treatment for the general CF population, irrespective of their mutation types.

A personalised and predictive platform for testing potential drugs has been developed by Dr Wong in her laboratory using patient-derived tissue specimens. Known as CF AVATARS, miniaturised versions of each CF patient are created using their own stem cells from lung and gut tissue, which swell up when a potential new Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) drug restores the defective function of the CFTR protein.

A recent study notably showed the reduction or even absence of a novel human gene called BGas was shown to boost CFTR levels independent of CFTR mutation types. This project will also explore the therapeutic potential of a few small molecules targeting BGas in the patient-derived CF AVATAR. If successful, gene targeting may herald a new dawn in CF therapy.



RESEARCHER

**Dr Sharon
Wong**

RESEARCHER Dr
Abdullah
Tarique



PROJECT: Molecular Mechanism of Defective Anti-Inflammatory Responses by Macrophages in CF

Ann Maree Bosch Career Fellowship 2017

INSTITUTION: **The Centre for Children's Health Research University of Queensland, QLD**

\$10,000 | 1 Year

The body's immune system is designed to respond to and protect against environmental and infectious attacks by preventing infectious agents from entering the body, defending against pathogens that break through the first line of defence and finally by healing the damaged tissues following an infection. The immune system's defence mechanisms protect, defend and fight against infectious agents while the healing mechanism repairs wounds made by the invading microbes. The macrophage is one of a number of immune cells whose role is to protect, eliminate and heal.

In CF it has been observed that the healing mechanism does not work at all. As a result, wounds made by agents during infectious episodes are not repaired resulting in repeated damage to the lungs.

Earlier studies have shown that due to the mutation in the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) gene in CF patients, macrophages fail to efficiently fight and eliminate pathogens. This project aims to identify the molecular mechanism that underlies the defective healing process in CF.

RESEARCHER **Zara
Sheikh**

PROJECT: Understanding the Mechanism of Interactions between Anti-Inflammatories and Antibiotics for Optimal CF Treatment

ACFRT Top-up Scholarship – 2018

INSTITUTION: **University of Sydney, NSW**

\$15,000 | 3 years

The mainstay of current CF treatment is based on chronic antibiotic therapy delivered by inhalation as an adjunct to either oral/inhaled anti-inflammatory drugs and/or oral/intravenous antibiotics to treat pulmonary exacerbations.

This project will investigate the different combinations of anti-inflammatories and antibiotics used in CF treatment to unravel their interactions and understand if their combined use could potentially impact on their efficacy and treatment outcomes.

This project has the potential to improve the day-to-day lives of CF patients, reducing the treatment burden, shortening treatment times and decreasing treatment cost, ultimately improving quality of life.





RESEARCHER

Dr Simone
Visser

PROJECT: Bacterial Lifestyles and Response to Antimicrobials in Patients with Acute and Chronic Lung Diseases

ACFRT Top-up Scholarship - 2017

INSTITUTION: Royal Prince Alfred Hospital, NSW

\$15,000 | 3 years

This project aims to begin investigation of new antibacterial treatments for chronic lung infections in CF, examining novel, combination anti-biofilm treatment for emerging CF pathogens.

Emerging bacterial pathogens, many of which are multi-resistant, establish chronic lung infections in patients with CF by forming biofilms in the airways.

In previous work Dr Visser's group has found that innovative triple combination therapy (glutathione/DNase/antibiotic) is effective in reducing *Pseudomonas aeruginosa* biofilms and this project will allow expansion of that work into the emerging CF pathogens. These are typically under-studied and include *Achromobacter*, *Stenotrophomonas*, and methicillin-resistant *Staphylococcus aureus*.

PROJECT: Does High Intensity Interval Training Improve Fitness in People with CF?

ACFRT Top-up Scholarship - 2017

INSTITUTION: Department of Physiotherapy, Sir Charles Gairdner Hospital, WA

\$15,000 | 3 years



People with CF with increased fitness levels have improved quality of life and may live longer.

Currently, people with CF are encouraged to exercise for 30 to 60 minutes a day, in line with recommendations provided for people who are healthy. This is often difficult to achieve due to the high daily treatment burden and competing demands in life such as work, study and family.

This project will investigate whether 10 minutes of high intensity interval training over an eight-week period improves fitness and other important outcomes such as quality of life, mood, confidence to complete exercise and enjoyment in people with CF.

RESEARCHER **Abbey
Sawyer**

RESEARCHER

Harry Tjondro



PROJECT: Are Altered Carbohydrates Associated with Compromised Immune Cells in CF?

ACFRT Top-up Scholarship - 2017

INSTITUTION: Department of Chemistry and
Biomolecular Sciences - Macquarie University, NSW

\$15,000 | 2 years

This project will for the first time use biochemistry to explore the aberrant carbohydrate signatures and their functional significance on CF neutrophils relative to healthy neutrophils, an important type of innate immune cell in CF.

Advancing our understanding of the structural and functional alterations of carbohydrates in CF neutrophils will be valuable in order to unravel the underlying disease mechanisms.

This study will generate valuable, fundamental biochemical knowledge to support the generation of new immune-based therapeutics to alleviate the disease burden of affected individuals.

RESEARCHER

Samuel Montgomery

PROJECT: The Role of Interleukin (IL-1 α) and Necrosis Leading to Neutrophilic Inflammation in Children with CF.

CFWA, Hardie Foundation Scholarship 2017 managed by ACFRT

INSTITUTION: Telethon Kids Institute, WA

\$37,500 | 3 Years

Recent studies in children with CF have demonstrated that lung disease occurring in the first few months of life is often detectable on Computerised Tomography (CT) before the presentation of clinical symptoms. This early CF lung disease is characterised by airway mucus plugging and neutrophilic inflammation.

This study aims to determine whether inflammation resulting from cell death in the airway is increased in early lung disease in CF. It also aims to identify differences in specific gene pathways during inflammation resulting from cell death in the CF airway.

This research will determine whether IL-1 α released from dying airway epithelial cells is elevated in early lung disease and if it correlates with neutrophilic inflammation in the CF airway. In addition, using an in vitro primary airway cell model and system biology, this study aims to identify specific gene pathways that are dysregulated during exposure to cell death-inducing conditions.





RESEARCHER

Kelly
Martinovich

PROJECT: Personalised Antisense Oligonucleotide Therapy to Correct Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Function in CF Patients.

CFWA Golf Classic Scholarship 2017 managed by ACFRT

INSTITUTION: School of Paediatrics and Child Health,
Princess Margaret Hospital, WA

\$37,500 | 3 years

There are more than 2,000 different mutations that cause CF. Small molecule therapies currently available on the market are not helpful to all CF patients.

This project aims to develop therapies that target rare CF mutations. Many mutations change the message that is passed on from the CF gene, which results in a poorly functioning CFTR channel. This channel maintains the airway surface liquid in the lungs, helping to clear infections.

Potential therapies developed as part of this project are small molecules called antisense oligonucleotides (AOs). AOs can change the faulty message transferred from gene to protein and improve the function of the CFTR protein. AOs can also be designed to target a specific site in the CF gene according to the distinct location and mutation type of an individual.

It is hoped that this study will initiate a personalised medicine pathway, generating molecules that lessen the severity of disease caused by specific CF-causing mutations.

AUSTRALIAN CYSTIC FIBROSIS RESEARCH TRUSTEES

Conrad Guerra

Founder and Past President
1989 to present



Paul Dalby

2014 to present



Laurie Daly

CFA Treasurer
2018 to present



Mitch Messer

Founder and President
1989 to present



Patrick O'Connor

CFA President
2015 to present



Nettie Burke

Trust Secretary and CEO of CFA
2015 to present



Cystic Fibrosis Australia (CFA) would like to thank all those who have donated to ACFRT research projects over the past 29 years and in particular, LJ Hooker who continue to be a great supporter of cystic fibrosis research and have sponsored this Research Review.

CFA's ability to raise funds for the ACFRT is dependent on the generosity of the business community and the general public. Tax-deductible donations can be made by contacting the CFA office on +61 2 9889 5171 or visiting our website **cysticfibrosis.org.au/research**.



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cysticfibrosis.org.au