NFMRI

2022

ANNUAL REPORT

About Us

Founded in 1977, the National Foundation for Medical Research and Innovation (NFMRI) is a not-for-profit organisation that is entirely independent. It is not affiliated with any university, hospital, government or state body. The Foundation provides financial and in-kind, skill-based support to advance research projects whilst conserving and building its capital base.

The Foundation is classified as a health promotion charity and is endorsed as a Tax Concession Charity (TCC) with Deductible Gift Recipient Item 1 (DGR 1) status.

Our Mission

"To advance innovations in medical research related to the nature, prevention, diagnosis, treatment and incidence of disease and other health problems that have a significant impact on the health of humans."

Our Vision

NFMRI believes more than funding alone is needed to advance discoveries and innovations. Our culture is one where we look to make a social investment in medical research. By partnering with researchers to provide support and knowledge, and facilitating connections with industry, we aim to maximise the social returns from our grants. The Foundation is looking to become a partner of choice with both researchers and funders of research, and a national ambassador for medical research and innovation.

Our vision is to be recognised as the leading Foundation, efficiently and effectively supporting biomedical research, advancing innovations and creating impact.

Our Approach

NFMRI takes a uniquely proactive approach by partnering with grant recipients to provide support along the innovation pathway. It is a prerequisite that we only fund research of the highest quality. When reviewing applications and research projects, NFMRI looks for more than good science. We also assess the ability and willingness of the researcher and the institution to collaborate and to plan and manage research along the innovation pathway. Most importantly, we analyse the potential commercial and social success of the innovation.

NFMRI also considers the need for and size of any potential impact, the potential for the research and innovation to make a significant difference and whether the opportunity may become attractive to a potential next-step partner who can make a product or service accessible to the community. To do this, we harness skill sets from a variety of scientific, clinical, business development, commercial, industry and financial sources.

The Foundation is looking to increase its impact by partnering with other trusts and foundations, Private and Public Ancillary Funds (PAFs and PuAFs) and corporate donors. NFMRI does not proactively solicit donations, but we are always grateful for the donations and bequests that we do receive.

Message from our Chairman

NFMRI continues to be well placed in delivering community benefits through the research we support. Although the markets remained turbulent in 2022, the overall impact was carefully managed and didn't affect the Foundation's operations.

NFMRI further strengthened its commitment to encourage women to pursue careers in STEM and medical research by taking a leading position developing resources that could be utilised broadly across the school system, in universities, by philanthropic organisations and in the medical research sector. Two videos were created thanks to the help of leading researchers Prof Lenka Munoz, Prof Janet Davies, A/Prof Joanne Macdonald, Prof Wendy Cooper, A/Prof Branka Grubor-Bauk and the producer Phil Carey from Cornerstone Media.

The videos celebrate the success stories of our researchers with the hope of inspiring the next generation of aspiring researchers to make a difference to people in need. They explore the pathways, experience, challenges and opportunities experienced by female researchers. We hope that these videos help inspire women – both students and up-and-coming researchers – to consider pursuing a medical research career so that they can deliver benefits to the community.

Thanks to the help of our partners, we continued to support an increasing number of projects. Their support is instrumental in enabling NFMRI to scale its work. We thank The Mason Foundation, The NSW Community Foundation, the Nicholas & Phyllis Pinter Trust and the Vernon Sinclair Fund (all managed by Equity Trustees) for supporting a number of promising research projects in the areas of Alzheimer's disease and cancer. Our initial three-year pilot partnership with Equity Trustees was renewed for a further three years, enabling us to hold a continuous grant round in Alzheimer's disease. Equity Trustees are true collaborators in all aspects of our partnership and as always are a pleasure to work with.

Together with the State Trustees Australia Foundation and the Cure4CF Foundation we jointly supported research projects in the areas of cancer and cystic fibrosis during 2022. We thank them both for their support.

We are pleasantly surprised with the continuous high level of success stories and impact shared by researchers with us. This is testament to the effectiveness of our strategy, as well as the support and advice received from our RAC, Board and management.

Since the Foundation's establishment in 1977, a total of over $21.5 million in grants has been committed to support innovative research projects, covering various diseases and conditions throughout the country. Thanks to our partnerships, we have increased our grant commitments in 2022 and hope to continue growing this further over time. This funding and our work have been made possible thanks to our generous benefactors, supporters and partners, including individuals and organisations, who generously contribute their time and expertise.

We thank BT Financial Group and in particular, Mr. Scott Glover, for carefully managing the Foundation's investments in consultation with our Board. We also thank Mr. Mark Boyle from Nexia Sydney for his continued professional work carrying out the year's annual audit of our Foundation.

I would especially like to acknowledge our team's efforts and achievements over the past year. Dr. Noel Chambers and Mrs. Nancy Ranner who work exceptionally well together as a team and continue to raise the bar for the Foundation. We thank Ms. Di Moore for her services, who left NFMRI in October 2022 and welcome Mrs. Linda Hearne to our team as our bookkeeper and administrator.

Likewise, I wish to thank my colleagues and fellow Directors for the dedication and passion they have brought to the organisation. Their leadership, vision and guidance continue to be instrumental to the work and successes of our Foundation. Our Board greatly appreciates the ongoing support and advice from our expert Research Advisory Committee (RAC).

This year we farewelled one of our Founding Directors and longstanding RAC Member, Dr. John Dixon Hughes OAM, who sadly passed away in September 2022. John has contributed enormously to the Foundation and has been involved since its inception in 1977. We were also saddened to say goodbye to our Foundation Member, Mr. Peter Bowen, who passed away shortly after John in November 2022. Peter was a director from 1995-2011, and during this time he helped grow the Foundation's corpus significantly by way of bequests. The Foundation would not be what it is today were it not for John and Peter. Both will be greatly missed by all.

Furthermore, we are grateful to have received pro-bono legal advice from Ms. Alison Choy Flannigan of Hall & Wilcox throughout the course of the year, as well as complimentary venue access and administrative support from McGrathNicol. It has been great to again hold in-person meetings and we look forward to holding more of these in person in 2023.

We were delighted to finally be able to welcome our stakeholders at our long-awaited conference "Research with Purpose 2022" at the Anchorage Hotel & Spa in Port Stephens, NSW in November 2022. Delegates from across the country joined us to participate in our highly engaging educational event to hear from a spectacular line-up of speakers. Feedback was overwhelmingly positive, and we look forward to welcoming everyone again at our next conference in Queensland later in 2024. Stay tuned for an announcement.

We greatly appreciate and value our stakeholders' support and are confident both our donors and partners will be pleased with the high-quality research projects their gifts and assistance have enabled. Together with our partners, we continue to focus on achieving the vision and goals of our benefactors – both past and present. I hope you enjoy reading about our collaborative progress throughout the 2022 calendar year and I look forward to continuing to share with you some of our achievements throughout the course of 2023 and beyond.

Dr. Rob Sauer
Chairman

Message from our CEO

The overarching purpose of medical research is to make a beneficial difference to humanity. However, for medical research to eventually reach the community and create a positive impact, research must first navigate a complex pathway and ultimately translate across what is often referred to as the 'valley of death'.

The translation of research requires many different areas of expertise, including different fields of research and non-research skills. Harnessing the expertise required along the pathway can be a 'supply chain' challenge. Who can and is best to perform preclinical studies all the way through commercialisation, manufacturing, regulatory approval and solving the 'who pays for it' questions require a broader set of thinking and co-ordination.

NFMRI has built for-purpose systems, processes and specialist capability to identify and support potential innovations that may deliver community benefits including new medicines, vaccines, diagnostics, devices, tools and biologicals. Our goal is to address this need and therefore our strategic support fills an important gap in the funding and support ecosystem, removing barriers and enabling access to external research capabilities and capacity to answer important questions and attract next-step partners.

With a growing number of medical research success stories, NFMRI continues to de-risk innovations and assist them to become attractive and competitive to secure next-step partners – in turn, helping translate research from academic labs into the hands of patients in need.

An analysis of the progress of our projects funded under this strategy since its implementation in 2013 found more than 62% of projects supported secured next-step partners, five entered formal clinical trials and more than ten times the original funding was leveraged from other sources. We are pleased to report that two technologies have gone beyond securing next-step partners and have since been granted regulatory approval. We plan to update our Impact Report Card data over time to maintain up-to-date statistics.

Our partnerships continue to strengthen and grow. Equity Trustees renewed our initial pilot partnership for another three years and because of this we have been able to run an ongoing Alzheimer's disease grant round with set cut-off dates. Through support from The Mason Foundation (also managed by Equity Trustees), we have been able to support a growing number of innovative Alzheimer's research projects. We were pleased to announce three new Alzheimer's disease research projects: Dr. Jonathan Danon (The University of Sydney), A/Prof. Peter van Wijngaarden (Centre for Eye Research Australia) and Dr. Prashant Bharadwaj (Edith Cowan University). The other researchers supported in 2022 through this partnership include Dr. Lesley Cheng (La Trobe University), Prof. Ralph Martins AO (Edith Cowan University), A/Prof. Lyndsey Collins-Praino/Dr. Andrew Care (University of Adelaide), Prof. Stuart Dashper (The University of Melbourne), and Prof. Michael Parker (St Vincent's Institute of Medical Research). More projects will be announced later in 2023.

Thanks to generous support from the NSW Community Foundation, the Nicholas and Phyllis Pinter Trust and the Vernon Sinclair Fund (all managed by Equity Trustees), we are supporting A/Prof. Vivien Chen's cancer research project at the ANZAC Research Institute.

Together with the Cure4CF Foundation, we supported two cystic fibrosis research projects during 2022 including A/Prof. Sarah Vreugde (University of Adelaide) and A/Prof. Leszek Lisowski (Children's Medical Research Institute).

Lastly, we were pleased to be able to support Prof. Stephen Fox (The University of Melbourne) cancer diagnostic project with the help of the State Trustees Australia Foundation. Together with a growing list of partners, we are supporting an increasing list of successful case studies, and I hope you enjoy reading more about these projects in this report.

All of this would not be possible without our Research Advisory Committee (RAC) Members, whom we thank for contributing an enormous amount of time reviewing expressions of interest, applications, reports and acquittals throughout the course of the year. The composition of our RAC is unique and includes clinicians, academics, research translation and commercial science experts. Each member has a different background and set of skills that helps provide a multi-lens approach in our reviews and support.

I also wish to thank our mentors and supporters, whose pro-bono support in IP, research translation, marketing, commercialisation, media and access to networks helps to ensure researchers and their innovations have the maximum ability of achieving the desired outcomes.

Our fifth conference, 'Research with Purpose 2022', took place at The Anchorage Hotel & Spa in Port Stephens, NSW on the 22nd-24th November 2022. Together, we collectively discussed challenges faced by the research community and explored possible solutions. We thank all speakers who generously contributed their time and shared their expertise with our audience. We look forward to welcoming you at our next event in Queensland later in 2024.

Dr. Noel Chambers,
Chief Executive Officer

Our Legacy

The Foundation was established in 1977 on the initiative of the late Dr Frank Ritchie who had several patients wishing to donate to medical research and for the capital to be preserved. Fundraising activities were conducted under the auspices of the initial Chairman of the Board of the Foundation, Sir Peter Abeles, and Lady Sonia McMahon.

A patient of Dr. Frank Ritchie bequeathed a substantial sum, the Stern Estate, to be divided equally between Sydney Hospital and the Foundation. The Foundation was to maintain the capital and use income to fund and facilitate ongoing medical research in perpetuity. Over the years, by way of further bequests and donations, the Foundation has built up significant capital reserves to provide income to facilitate continuing important medical research. The funds of the Foundation and the management of those funds have always been totally independent of the hospital, as has been its management structure. Following an extensive review of the sector, the Foundation updated its mission and changed its name from the Sydney Foundation for Medical Research to the National Foundation for Medical Research and Innovation in 2014.

Past Directors and Major Benefactors

Our Foundation owes its legacy to the following Directors who have served as part of its Board and to those who contributed to the Foundation so generously. Without their vision, foresight and commitment to the Foundation, it would not be where it is today.

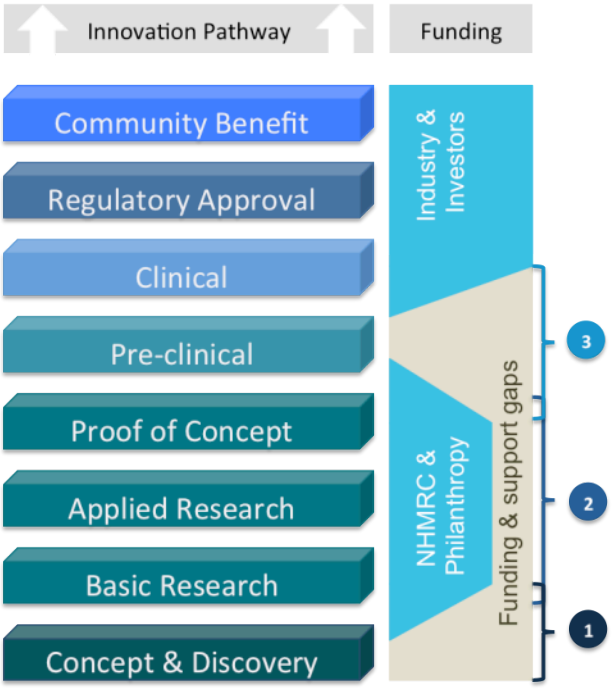
1979-1982	Sir Peter Abeles (Founding Chairman)	1984-1987	Mr. JW MacBean
1979-1983	Mr. ED Cameron	1984-1985	Sir William W Pettingell
1979-1983	Mr. JP Ducker AO	1987-2003	Mrs. SE Ball
1979-1983	Mr. MJ Inglis	1987-1999	Mr. RH Minter (Chairman)
1979-1982	Lady Sonia McMahon	1987-2017	Dr. V Cowlishaw Shortell
1979-1990	Mr. TE May (Former Chairman)	1995-2011	Mr. PM Bowen
1977-1982	Dr. FL Ritchie C.B.E.	2000-2003	Prof. AJ Young AO
1977-1995	Mr. BF Rose	2002-2017	Dr. J Graham OAM
1979-1982	Dr. HH Spiegel	2002-2018	A/Prof. R Garrick AM
1979-1982	Sir Ian Turbott C.M.G, C.V.O	2006-2017	Ms. J Schwager AO
1982-2007	Dr. J Raftos AM	2010-2019	Mr. K Drewery
1984-1990	Sir Gordon Jackson	2014-2019	Dr. A Bates
1984-1991	Mr. TL Lewis	1977-2022	Dr. J Dixon Hughes OAM

The Stern Estate
Josephine White and Hiltbrunner Fund
Estate Late Celia Margaret Paine
Estate Late Daqmar Wilhemine Halas
Estate Late Blanche Elizabeth Turner
Estate Late Mary Althouse
The Mason Foundation (managed by Equity Trustees)
NSW Community Foundation – Nicholas and Phyllis Pinter Trust (managed by Equity Trustees)
The Vernon Sinclair Fund (managed by Equity Trustees)
Cynthia & Patricia Gaden Fund
Tempe Mann Fund
Estate Late Bill & Shirley Westbrook
Estate Late Gloria Ida Prejeant
Estate Late Beatrice Gordon Joske
Estate Late James Hoadley
NSW Community Foundation
NSW Department of Primary Industries
Cure 4 Cystic Fibrosis Foundation
State Trustees Australia Foundation

Our Strategy

Historically, funding of medical research in Australia has been determined by outputs – research papers and citations validated by scientific peer review. Whilst these factors are important, the advancement of innovation, the formation of collaborations and the ability to deliver impact are the outcomes NFMRI's funding delivers.

To maximise impact, NFMRI focuses on advancing innovation. By looking outwards and supporting the gaps along the innovation pathway and applying resources, networks and knowledge, NFMRI helps philanthropy make a difference. NFMRI supports medical research in three key gap areas we call social investment portfolios.



3 Bridging the 'valley of death'. Supporting research required to facilitate collaborator uptake and investment

Often referred to as the 'valley of death', this is the area where strategic research studies are required to attract potential investors and industry collaborators.

Traditional funding mechanisms do not support or motivate researchers to contract research activities necessary to answer some research questions necessary to form these collaborations.
These research questions are often not attractive to publications as they are 'less newsworthy' and not research undertaken by the chief investigator and their team.

By supporting small incremental studies, NFMRI can manage risk and make innovations more attractive to potential commercial partners and investors.

2 Support for strategic collaborative research activities focussed on advancing research and validating directions

Providing access to the additional research skills not obtainable through currently available funding mechanisms.

Support for strategic collaborative research activities focussed on advancing innovations and validating directions is needed. NFMRI is uniquely positioned to add value to the advancement of research and innovations in preparation for potential collaborations.

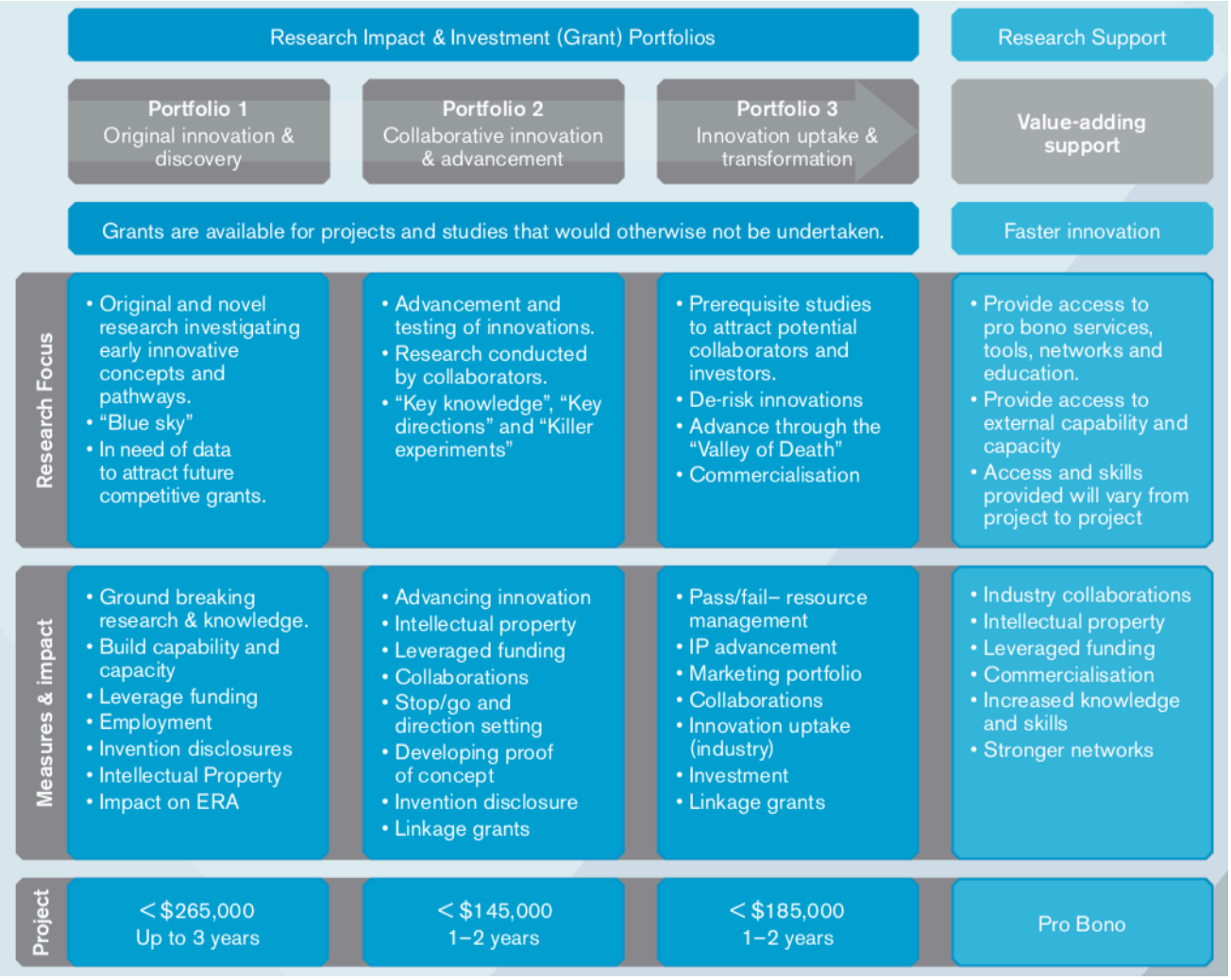
By partnering with researchers, NFMRI supports collaborative research activities undertaken by other

research groups that expedite the advancement of the innovation and are important for attracting potential industry partners and investors.

1 Original Australian innovation and discovery. Frontier research not competitive for NHMRC grants

Supporting the validation of new concepts, discoveries and intellectual property creates the foundation for innovations and community benefits of tomorrow. Young researchers, early discoveries and new paradigms need support to become competitive and stand on their own two feet.

Portfolio Summary



Grant amounts and durations are a guide only.

Our Supporters

We wish to acknowledge and thank the following organisations and individuals who have supported the Foundation during 2022. Their assistance has greatly contributed to the Foundation's growth and success.

McGrathNicol

Over many years, McGrathNicol has generously provided support to the Foundation. NFMRI is very grateful to McGrathNicol for kindly providing administrative support and use of office facilities.

BT Financial Group

BT Financial Group has been supporting the Foundation for several years, providing strategic guidance to the organisation and management of our investment portfolio.

Equity Trustees

A partnership with Equity Trustees was formed thanks to generous funding from The Mason Foundation to help support mutually aligned research into Alzheimer's Disease. Additional funding from The NSW Community Foundation, the NSW Community Foundation – Nicholas and Phyllis Pinter Trust and the Vernon Sinclair Fund has been provided to support cancer research. This funding has helped support the following grants in 2022: A/Prof. Vivien Chen, Prof. Ralph Martins AO, A/Prof. Peter van Wijngaarden, Dr. Lesley Cheng, Dr. Sanjaya Kuruppu, A/Prof. Lyndsey Collins-Praino (A/Prof. Andrew Care), Prof. Stuart Dashper, Prof. Michael Parker, Dr. Jonathan Danon and Dr. Prashant Bharadwaj.

State Trustees Australia Foundation

Funding from the State Trustees Australia Foundation has helped support Prof. Stephen Fox's research project.

Cure 4 Cystic Fibrosis Foundation (Cure4CF)

A partnership was formed with Cure4CF to support research into potential therapies or a cure for cystic fibrosis. This partnership supported A/Prof. Sarah Vreugde and A/Prof. Leszek Lisowski in 2022.

Hall & Wilcox

Hall & Wilcox kindly continues to provide pro bono legal and secretarial advice to the Foundation and has supported many of our past events.

IP Australia

IP Australia kindly provided pro bono patent analytics research to several of our research projects.

Nexia Sydney Audit Pty. Ltd.

We wish to thank Nexia Sydney Audit Pty. Ltd. for being our auditors since 2016.

Our Governance

The National Foundation for Medical Research and Innovation (ABN: 85 001 422 895) is endorsed as a Tax Concession Charity and Deductible Gift Recipient (Item 1). The Foundation is also recognised as a Health Promotion Charity and has fundraising licences in relevant Australian states.

The Directors of the Foundation and management are committed to achieving and demonstrating the highest standards of corporate governance. The Directors of the Foundation continually seek to adopt best practice policies and procedures.

In accordance with the Foundation's strong focus on sound governance, the Board has adopted a Governance Charter that supplements its Constitution and details the policies, processes and expectations for the Directors, Research Advisory Committee (RAC), staff and contractors of the Foundation. It outlines a code of conduct, which all members are required to agree to, as well as conflicts of interest disclosures and management procedures.

The annual review of the Foundation's governance frameworks considers best practice guides, including those published by the Australian Securities Exchange and Standards Australia.

The Foundation has continuous improvement processes and adopts a governance review schedule, which includes the review of its skills-based Board, RAC and Staff.

Our Board's Responsibilities

One of the primary responsibilities of the Board is to be the custodian of the purpose of the Foundation as set out in the mission statement within the Foundation's Constitution.

Our Mission

"To advance innovations in medical research related to the nature, prevention, diagnosis, treatment and incidence of disease and other health problems that have a significant impact on the health of humans."

Specific responsibilities include:

- Continually develop and drive the vision of the Foundation;
- Identify any critical gaps in medical research funding in the community;
- Achieve a greater profile within the research community;
- Grant funding to applicants whose research supports the mission of the Foundation;
- Provide guidance to the Research Advisory Committee in respect of the type of research project that the Foundation may fund;
- Increase the Foundation's ability to give via partnerships, bequests and any other suitable avenues; and
- Grow and monitor the financial capital base of the Foundation.

Our Management's Responsibilities

The Board has formally delegated day-to-day management of the company's operations and the implementation of the Foundation's strategy and policy initiatives to the Chief Executive Officer and senior executives.

Our Partnerships

NFMRI seeks to engage with like-minded stakeholders that enable the Foundation to fulfil its mission. The Foundation values its stakeholders and believes that for its partnerships to be successful, both parties must be involved and have mutual expectations.

Our processes facilitate discussions to align expectations, establish a governance framework and develop a partnership that benefits both parties to achieve the desired results. To maximise success, our focus is on identifying and working with groups that have aligned interests, where each party stands to mutually benefit from the partnership. Over recent years, we have had the pleasure of partnering with the following organisations:

Equity Trustees (The Mason Foundation, NSW Community Foundation, NSW Community Foundation – Nicholas and Phyllis Pinter Trust and the Vernon Sinclair Fund): Equity Trustees extended our partnership to assist with distribution of medical research funding for a further three years. This includes an annual contribution of approximately $520,000 towards Alzheimer's disease research and approximately $80,000 from other partners to support cancer research. NFMRI was seen as a partner of choice due to its strategy and focus on outcomes, independence, ability to value-add to research projects and its ability to support innovation successes that will lead to tangible community outcomes. Together we have supported many projects featured in this report.

Cure4CF Foundation: NFMRI was selected by the Cure4CF Foundation board and team to assist the Foundation with its giving. We ran specific cystic fibrosis grant rounds to source the most innovative projects across the country. Together we have supported Prof. Marc Pellegrini, A/Prof. Sarah Vreugde and A/Prof. Leszek Lisowski.

State Trustees Australia Foundation: NFMRI is assisting the State Trustees Australia Foundation in supporting innovative cancer research in Victoria. Together we are supporting Prof. Stephen Fox's diagnostic research project at the Peter MacCallum Cancer Centre.

NFMRI's Impact

Following the introduction of our strategy focused on supporting the advancement of early biomedical research innovations to enable them to attract next-step partners, NFMRI carried out an analysis of its strategic outcomes to date in 2021.

Although a number of research projects are ongoing and expected to succeed in attracting next-step partners, an analysis of projects commencing between 2013-2019 yielded better than expected results. Sixty-two percent of projects supported achieved our primary measure of success in attaining next-step partners leveraging over $10 of external funding for every dollar of grant funding – a total of $45.3 million. Five innovations have commenced clinical trials and one innovation is delivering benefits to cancer patients. Analysis of our strategic outcomes provides evidence that what NFMRI does is reproducible and effective, irrespective of indication, disease or innovation (medicine, vaccine, biological, device or diagnostic).

62% of projects supported by NFMRI attained next-step partners

Strategic success measures	Strategic success outcomes
Attract next-step partners including industry and venture capital	Sixty-two percent of projects (16/26) achieved our primary measure of success in attaining next-step partners
Become clinical trial ready	Five innovations have commenced clinical trials and one innovation is delivering benefits to cancer patients.
Leverage the funding, expertise and experience of next-step partners	Leveraging over $10 of external funding for every dollar of grant funding – a total of $45.3 million.

"The gap between basic research and commercial support for the discovery and development of new therapies, the so called "valley-of-death", is not just a funding gap but it is also a skills gap. Notwithstanding its notable 62% success rate in helping researchers bring their programs to next-step partners, NFMRI funding is 100% successful in helping train the next generation of Australian innovators and industry-ready researchers and leaders. The impact of this output is immeasurable." -

NFMRI's Impact: Project Updates

Below are a few examples of grant updates arising from the support provided by NFMRI and our partners over recent years. Our researchers share their outcomes and outputs directly with us utilising our online progressive reporting system. With an increasing amount of success stories, we are planning to update our impact report statistics soon.

Prof. Nicholas Opie, The University of Melbourne and Synchron

Safety validation of the Stentrode: a biomedical device for paralysis that converts thoughts into computer commands **(2018, $390,000)**

- NHMRC project grant, $1,651,685
- NHMRC development grant, $810,382
- DARPA & ONR Global, $1,331,325
- Defense Health, $154,823
- Funding from other external sources, $193,000
- Spin-off company Synchron
- Synchron (Series C) brings total raised since inception to $212,000,000. Company is backed by Bill Gates' venture fund and Amazon founder Jeff Bezos' 'Bezos Expeditions'
- Platform product has been implanted successfully into patients to treat paralysis

Dr. John Raftos AM Medal **(2022, $50,000)**

- Grant is currently underway

A/Prof. Branka Grubor-Bauk, The University of Adelaide

Novel T cell-based DNA vaccine against Zika virus infection **(2021, $220,000)**

- Industry collaboration with Vaxxas (Australia)
- Industry support from Enesi Pharma UK
- Industry collaboration with PharmaJet (CO, USA)

Prof. Mark Kendall, Australian National University and WearOptimo

Dr John Dixon Hughes OAM Medal **(2017, $50,000)**

- Led to the establishment of microwearables company, WearOptimo
- WearOptimo granted a $30,000,000 manufacturing project in QLD
- Multi-million funding round from Aspen Medical

Prof. Bernard Flynn, Monash University and Cincera Therapeutics

Novel sphingolipid targeted agents for the treatment of cardiac fibrosis **(2016, $100,000**)

- Monash Research Innovation Fund (MRIF), $50,000
- NHMRC Development Grant, $684,000
- Spin-off company Cincera Therapeutics, $7,000,000

Sphingosine Kinase-1 inhibitors for the treatment of pulmonary hypertension **(2020, $110,400**)

- Spin-off company AnkeRX, $10,791,639

Dr. John Raftos AM Medal **(2020, $50,000)**

Gastrointestinal-directed S1P1-receptor modulators in the treatment of inflammatory bowel disease (2021, $135,700)

- Monash MIPS Incubator Investment, $50,000
- Monash MRIF investment, $50,000
- Term sheet for a VC-backed seed funding start-up company, $2,500,000

Prof. Lenka Munoz, The University of Sydney

Improving chemotherapy response rates in brain cancer **(2013, $396,104)**

Dr. John Raftos AM Medal **(2020, $50,000)**

- NHMRC Ideas Grant, $1,100,000
- NHMRC Development Grant, $550,000
- Lin BioScience funding grant, $1,200,000
- Promoted to Professor
- Substantial IP and out-licensing portfolio of neuro-oncology therapeutics she developed
- Licensing agreement

Prof. Roger Chung, Macquarie University & Celosia Therapeutics

Preclinical evaluation of novel therapies for clearance of TDP-43 in amyotrophic lateral sclerosis **(2019, $183,488)**

- Fight MND Foundation Drug Development Grant, $1,000,000
- Fight MND Foundation Impact Grant, $250,000
- Spin-off company, Celosia Therapeutics

Prof. Steven Wise, The University of Sydney and NanomedX

Durable Treatment of Peripheral Artery Disease **(2019, $95,022)**

- Sydney Local Health District - Accelerating Translation of New Cardiovascular Devices and Materials - $361,752
- Heart Foundation Future Leader Fellow Level 3, $593,126
- NSW Cardiovascular Initiative, $727,423
- MTPConnect TTRA Research Project, $739,128
- Spin-off company NanomedX with private seed funding of over $790,000
- Promoted to Professor of Cardiovascular Bioengineering

A/Prof. Peter van Wijngaarden, Centre for Eye Research Australia and Enlighten Imaging

***Translating an eye imaging biomarker for Alzheimer's disease to the clinic* (2019, $250,000)**

- BioMedTech Horizons 2.0 grant, $1,000,000
- NHMRC research grant, $1,400,000
- Funding from multiple external sources, $2,600,000
- Well advanced camera prototypes
- Strong IP position
- Spin-off company Enlighten Imaging

***A retinal imaging biomarker of Alzheimer's disease* (2022, $40,000)**

- Project currently underway

Prof. Wendy Cooper, Royal Prince Alfred Hospital

***Personalised medicine for lung cancer and mesothelioma* (2012, $125,000)**
***Personalised medicine in lung cancer – massively parallel sequencing of lung tumors enriched for actionable mutations* (2014, $190,000)**
***Dr. John Raftos Medal* (2018, $50,000)**

- Cancer mutation testing being used to characterise tumour pathology and identify suitable and personalised drugs for patients
- Approval of new therapies and concurrent diagnostics by PBAC and MSAC
- Promoted to Professor

Prof. Michael Good AO, Griffith University Institute for Glycomics

***Producing a GMP grade peptide conjugate vaccine for a Phase 1 trial to prevent infection with group A streptococcus* (2015, $251,000)**

- Leveraged $3,500,000 from the Heart Foundation
- Leveraged over $750,000 of funding from external sources, including Lowitja, Snow Foundation etc.
- $550,000 in funding and over $500,000 in kind to fund a Phase 1 clinical trial from the Li Ka Shing Institute (Canada)
- Investment from industry
- Currently in Phase 1 clinical trial overseas
- Anticipated commercialisation through Australian NewCo establishment in 2023/24

***Manufacture and evaluation of a chemically attenuated Plasmodium falciparum whole parasite blood-stage malaria vaccine* (2018, $200,000)**

- Rotary Health Grant, $1,035,648
- Medical Research Future Fund, $500,000
- Private trust funding, $570,000
- Private philanthropic donor, $372,027
- Griffith University funding, $334,968
- Collaborating with industry & currently in clinical trial

New Grants Approved in 2022

From grant rounds held during 2022, a total of seven new grants were approved (a total of $1,063,904) and announced in 2022:

Dr. Jonathan Danon **University of Sydney** **$143,904 (2022-2024)**

Innovative molecules for imaging neuroinflammation in Alzheimer's disease

Dr. Danon and his team have recently developed a set of novel radiotracers designed to detect neuroinflammation with unprecedented sensitivity in humans of all genetic backgrounds. This grant is funding a crucial collaborative study that will be performed at the Centre for Advanced Imaging at the University of Queensland. They will validate the ability of these compounds to image neuroinflammation in preclinical animal models of Alzheimer's disease using positron emission tomography (PET), generating data that will strengthen their IP position and take the innovation another step closer to preclinical first-in-human trials, commercialisation, and global distribution. These studies will be fundamental for translating their preliminary results into clinically useful tools that could help millions of people globally who live with Alzheimer's disease.

This project is funded in partnership between The Mason Foundation (managed by Equity Trustees) and NFMRI.

A/Prof. Peter van Wijngaarden **Centre for Eye Research Australia** **$40,000 (2022-2023)**

A retinal imaging biomarker of Alzheimer's disease

This project seeks to generate key experimental data to show that the retinal signal that they are measuring in Alzheimer's disease is attributable to amyloid beta. To do so, they will modify a new type of retinal camera that they have developed for human use to image the eyes of mice that have been genetically modified to demonstrate features of Alzheimer's disease. This will enable A/Prof. van Wijngaarden and his team to compare their imaging findings in living mice with comparable images from humans and with mouse retinal tissue studies that will measure the levels and subtypes of amyloid beta. This evidence will be important to gain approval for the use of this imaging technology in the clinic. Doing so would mean that it will be possible to detect people with or at risk of Alzheimer's disease during routine eye examinations. Detecting the earliest stages of the disease may eventually enable the commencement of disease-modifying treatments at the right time before irreversible brain damage occurs.

This project is funded in partnership between The Mason Foundation (managed by Equity Trustees) and NFMRI.

Prof. Denise Doolan **James Cook University** **$290,000 (2023-2024)**

Development of a multi-antigen T-cell malaria vaccine

Despite the dramatic decline in mortality and morbidity during the past decade, there is now a resurgence of malaria. Vaccination with the current lead malaria vaccine candidate can induce partial protection, but efficacy is low (<35%) and short-lived. To address this need, Prof. Doolan and her team have been pursuing an innovative approach to malaria vaccine development and together have identified four novel high-potential targets for a malaria vaccine to protect against all species of malaria. Each of these antigens can reduce liver-stage and blood-stage parasite burden against same-species and cross-species sporozoite challenge in mice, when administered in either of two vaccine regimens. Funding for this project will be used to enhance the pre-clinical package to support the translation of these lead antigens through the pre-clinical pipeline to phase 1/2a clinical testing, strengthen the IP portfolio, and attract a commercialisation partner.

A/Prof. Linda Wakim **Doherty Institute, The University of Melbourne** **$145,000 (2023-2024)**

A novel universal influenza virus vaccine that provides long term protection against the flu

Vaccination remains the best way to prevent human influenza disease, a highly contagious and rapidly spreading acute respiratory disease. However, current influenza vaccines are sub-optimal relying on annual reformulation and delivery to the population to maintain protective immunity. Current vaccines also offer no protection during a pandemic outbreak, where the emergence of novel viruses from animal reservoirs spread rapidly worldwide, having a devastating impact on global health. This project unites A/Prof. Linda Wakim, Prof. Greg Qiao, Prof. Bernd Rehm together with the WHO Collaborating Centre for Reference and Research and Influenza (CCRRI) to develop a novel “one-shot’ intranasal influenza vaccine that provides long term, cross-strain protection against seasonal and newly emerging pandemic strains.

Funding will be used to develop an improved vaccine formulation and perform key in-vitro and in-vivo pre-clinical testing. While the initial focus of this project is to develop an intranasal influenza virus vaccine, it is envisaged that the formulation will be developed with flexibility so that it can be easily and rapidly adapted to target other clinically relevant respiratory viruses (ie SARS-CoV-2).

Dr. Livia Carvalho **Lions Eye Institute** **$145,000 (2023-2024)**

Investigating novel bile acid nanocapsules carrying neuroprotective agents for the treatment of retinitis pigmentosa

Retinitis pigmentosa is a rare, inherited degenerative eye disease that causes severe vision impairment. Dr. Livia Carvalho and her team are developing a safe and effective nanocapsule system that can support sustained drug delivery into the eye for prolonged beneficial effects to help treat retinitis pigmentosa. Their preliminary research has shown that intravitreal administration into the eye of a neuroprotective drug can provide a transient protective effect in mouse models of inherited retinal diseases. In these animals, treatment was capable of reversing cell death temporarily in the first few days after treatment, but the effect was lost after a week. This project will establish a multidisciplinary collaboration between Dr. Carvalho’s vision research lab and Dr. Al-Salami’s bio-nanotechnology development lab to address this unmet need. Funding for this proof-of-principle project will be used to test the long-term treatment efficacy of this neuroprotective drug using a novel bile acid-based nanocapsule system compatible with direct eye delivery.

Dr. Prashant Bharadwaj **Edith Cowan University** **$250,000 (2023-2024)**

Analysis of neurofilament biomarkers for Alzheimer's disease, Parkinson's disease, Multiple sclerosis and childhood dementia

Currently, the diagnosis of Alzheimer's disease (AD) is difficult, expensive, and there are no reliable biological indicators of the disease. Dr. Bharadwaj and his team aim to develop a blood test for cognitive loss in AD by identifying a signature of the disease based upon specific proteins. The NFL protein has been shown to be a good indicator of many forms of neurodegeneration and not specific to AD only. Recent findings suggest the occurrence of distinct NFL variants in the brain and blood. Furthermore, NFL variants in AD blood appear to be different compared to healthy individuals. This project is a novel concept and aims to characterise these variants in different types of dementias. This funding will also support the use of mass spectrometry to help determine if specific variants are differently expressed in AD and identify whether these modifications form a signature unique for AD. If successful, findings from this study could ultimately improve the accuracy of AD diagnosis.

This project is funded in partnership between The Mason Foundation (managed by Equity Trustees) and NFMRI.

Dr. Joshua Ooi **Monash University** **$50,000 (2023)**

Dr John Dixon Hughes OAM Medal

In a landmark 1st author Nature paper, Dr. Ooi showed that regulatory T cells (Tregs) specific for self-proteins can be used to specifically treat the cause of autoimmune diseases, like lupus. Dr. Ooi then started his own independent laboratory and devised a platform that can genetically engineer GMP-ready Tregs specific for any self-protein. These cell products are called Targeted Tregs.

Dr. Ooi has applied his unique platform to develop Targeted Tregs specific for lupus. Lupus patients suffer from multi-organ dysfunction due to an autoimmune response that attacks its own tissues. Using lupus patient blood samples to devise a novel humanised mouse model of lupus, Dr. Ooi has shown that the lupus-specific Targeted Treg product effectively stops the pathogenic autoimmune response and effectively cures disease.

In addition to the lupus-specific Targeted Treg product (which led to a large research collaboration and commercialisation deal with a multinational pharma company), Dr. Ooi has developed and filed patents for Targeted Tregs specific for other severe autoimmune diseases including Sjogren's syndrome and autoimmune vasculitis.

Grants with Funding Continued in 2022

Following recommendations of our Research Advisory Committee, the Board approved $1,074,127 in grant payments supporting 18 projects during the 2022 calendar year:

Researcher	Institute	Focus Area	Total 2022	Total funding commitment
A/Prof. Sarah Vreugde*	University of Adelaide	Cystic fibrosis	$29,852	$145,000
A/Prof. James Chong	University of Sydney	Cardiovascular	$33,334	$200,000
Prof. Stuart Dashper**	University of Melbourne	Alzheimer's disease	$60,000	$160,000
A/Prof. Lyndsey Collins-Praino/A/Prof. Andrew Care**	University of Adelaide	Alzheimer's disease	$93,746	$249,990
Prof. Ralph Martins AO**	Edith Cowan University	Alzheimer's disease	$82,500	$170,000
A/Prof. Leszek Lisowski*	The University of Sydney	Cystic fibrosis	$71,442	$140,000
A/Prof. Branka Grubor-Bauk	University of Adelaide	Zika virus	$155,000	$220,000
Prof. Stephen Fox***	University of Melbourne	Cancer	$36,000	$144,000
Prof. Michael Parker**	St Vincent's Institute of Medical Research	Alzheimer's disease	$45,392	$192,374
Prof. Bernard Flynn	Monash University	Bowel Disease	$33,925	$135,700
Dr. Lesley Cheng**	La Trobe University	Alzheimer's disease	$20,200	$20,200
Prof. Nicholas Opie	The University of Melbourne	Paralysis	$37,500	$50,000
A/Prof. Vivien Chen****	ANZAC Research Institute	Cancer	$77,250	$193,000
Prof. Jake Shortt	Monash University	Cancer	$98,360	$190,000
Prof. Merlin Thomas	Monash University	COVID	$175,000	$175,000
A/Prof. Peter van Wijngaarden**	Centre for Eye Research Australia	Alzheimer's disease	$10,000	$40,000
Dr. Jonathan Danon**	The University of Sydney	Alzheimer's disease	$14,626	$143,904
			$1,074,127	**$2,569,168**

**Supported by NFMRI and the Cure4 Cystic Fibrosis Foundation*
***Supported by NFMRI and The Mason Foundation (managed by Equity Trustees)*
****Supported by NFMRI and the State Trustees Australia Foundation*
*****Supported by NFMRI, the NSW Community Foundation and the NSW Community Foundation Nicholas and Phyllis Pinter Trust & Vernon Sinclair Fund (managed by Equity Trustees)*

Projects Supported in 2022

A/Prof. Sarah Vreugde **The University of Adelaide** **$145,000 (2020 – 2022)**

A novel treatment for Non-Tuberculous Mycobacteria lung infections in cystic fibrosis patients

A/Prof. Sarah Vreugde is targeting Non-Tuberculous Mycobacteria (NTM) lung infections in cystic fibrosis patients, which cause severe infection and lung function decline. NTM lung disease is caused by bacteria that are common in the environment and are rapidly rising in prevalence, particularly in those with cystic fibrosis. NTM are naturally resistant to antibiotics and even disinfectants and so, are challenging to treat. Sarah's technology is unique because it is the only treatment in (pre)clinical development that targets the bacterial iron metabolism, which enables the bacteria to thrive and survive.

Funding provided by the Cure4CF Foundation and NFMRI is being used to further develop the proof-of-concept of the technology in a preclinical animal model of CF.

A/Prof. James Chong **The University of Sydney** **$200,000 (2020 – 2022)**

Development of novel recombinant human platelet derived growth factor therapy for prevention of ischemic heart failure

This project will develop a novel recombinant human Platelet Derived Growth Factor (PGDF) protein therapy for acute ischemic cardiovascular disease. Cardiovascular disease remains our greatest source of death and disability, accounting for billions of dollars in health care costs. The major single cause for this is "heart attack". Despite significant progress in medical and interventional therapies for heart attack, patients can still lose up to a billion heart muscle cells. This is due to the heart's inability to regenerate (unlike other organs such as the skin and liver) and down-stream health issues including heart failure, heart rhythm abnormalities and recurrent chest pain occur. A/Prof. Chong's results show that in both rodents and the more clinically relevant porcine model, human PDGF-AB treatment administered after heart attack decreases scar, increases heart function, decreases heart rhythm abnormalities, and increases new blood vessel formation. The overarching aim is to progress this experimental therapy into human patients suffering from heart attack and heart failure.

Funding provided by NFMRI is helping develop the therapy towards first-in-human clinical trials for patients with severe heart dysfunction after heart attack.

A/Prof. Bernard Flynn **Monash University** **$135,700 (2021-2022)**

Gastrointestinal-directed S1P1-Receptor Modulators in the Treatment of Inflammatory Bowel Disease

Inflammatory bowel disease (Crohn's and ulcerative colitis) affects 85,000 Australians and millions of people world-wide and there is currently no cure for this disease. Most current therapeutics are only partially effective, providing temporary relief to a subset of patients. There is significant interest in the development of orally bioavailable agents, with more significant and sustained efficacy and which treat a broader IBD patient group. While a number of small molecule immunomodulators are in use or development, the therapeutic utility of these is compromised by their systemic immunosuppressive effects (opportunistic infection and increased cancer risk through reduced

immunosurveillance) and off-target effects. Consequently, there is considerable interest in the development of GI-directed agents that can exert a GI-specific immunomodulatory effect. Recently, small molecule sphingosine-1-phoshate-1 (S1P1)-receptor modulators (eg ozanimod and etrasimod) have emerged as a new class of orally bioavailable immunosuppressive agents showing great promise in IBD clinical trials (Phase II/III). However, these agents suffer from dose-limiting adverse effects on non-GI organs.

Funding from NFMRI is supporting a proof-of-concept to a new class of orally administered, GI-directed S1P1-receptor modulators as more effective treatments of IBD with negligible systemic exposure and improved efficacy and safety profiles.

Prof. Stuart Dashper **The University of Melbourne** **$160,000 (2020-2022)**

A polymicrobial aetiology for Alzheimer's disease

L-R: Su Tolson, Deanne Catmull, Prof. Stuart Dashper, Rita Paolini, Prof. Neil O'Brien-Simpson

A growing number of studies are now linking bacterial infection and/or periodontitis with sporadic Alzheimer's disease (AD). Two recent studies have provided strong evidence for a potential causal link between the pathogenic oral bacterium Porphyromonas gingivalis and AD. There are also reports of oral bacterial proteolytic enzymes and genomic DNA, particularly those of P. gingivalis and Treponema denticola, in the brain tissue of AD sufferers. Prof. Stuart Dashper's and Dr. Catherine Butler's research will seek to demonstrate a causal link between specific oral bacteria and the onset and progression of Alzheimer's disease. If successful, it may open a whole new field of research into the bacterial aetiology of Alzheimer's disease.

Funding provided by The Mason Foundation (managed by Equity Trustees) and NFMRI will enable further development of this project.

A/Prof. Lyndsey Collins-Praino / A/Prof. Andrew Care **The University of Adelaide** **$249,990 (2020-2022)**

"Cage vs. Age": Development of an innovative nanotechnology to halt the spread of abnormal Tau protein in Alzheimer's disease

Dementia is a significant global problem affecting 50 million people worldwide, with a staggering 10 million new cases diagnosed each year (WHO). Alzheimer's disease (AD) is the most common cause of dementia, accounting for 60-70% of all cases. A major contributor to the spread of AD throughout the brain is the transmission of an abnormally folded protein called tau. Tau is released from diseased brain cells and taken up by healthy cells, triggering misfolding and aggregation of tau within those cells. Thus, AD spreads throughout the brain. The aim of the project is to engineer an innovative nanotechnology that can target and disrupt tau pathology. If successful, such a strategy would lead to modification of the brain mechanisms of AD and the potential development of a treatment strategy that would be of significant benefit to the millions of individuals currently suffering from AD. Funding from The Mason Foundation (managed by Equity Trustees) and NFMRI will allow for further engineering of the nanotechnology platform, as well as evaluation of its safety for neurological indications.

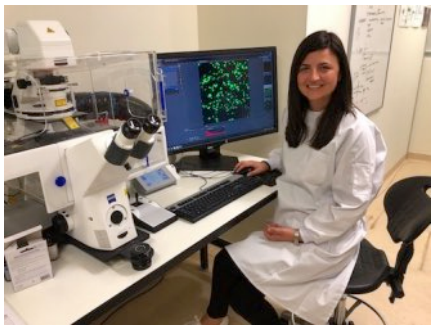
Prof. Ralph Martins AO **Edith Cowan University** **$170,000 (2020-2022)**

In-depth neurofilament analysis as potential biomarkers for Alzheimer's disease

Prof. Martins and his team's research is based on an in-depth analysis of neurofilament light chain (NFL) in plasma and cerebrospinal fluid (CSF) in Alzheimer's disease (AD) patients and healthy controls to determine whether AD affected individuals possess altered neurofilament light chain (NFL) and/or different binding partners. NFL levels in plasma and CSF have repeatedly been reported to be increased in AD patients, strongly supporting a link with the disease state. However, higher levels were also reported for other neurological diseases, making NFL levels a generic marker for neurodegeneration, rather than a specific marker for AD. A specific NFL protein signature found in plasma or CSF that closely associated with AD will allow more accurate and quicker detection of the disease, leading to early medical treatments known to be more effective. This project is supported thanks to funding from The Mason Foundation (managed by Equity Trustees) and NFMRI.

A/Prof. Branka Grubor-Bauk **University of Adelaide** **$220,000 (2021-2023)**

Novel T cell-based DNA vaccine against Zika virus infection



A/Prof. Grubor-Bauk has developed an innovative, patented and thermally stable Zika virus DNA vaccine that encodes Zika virus non-structural protein 1, NS1. Extensive evaluation of this vaccine in mouse models of Zika infection has shown that it induces strong immunity and confers complete protection against systemic Zika infection. Funding from NFMRI is helping to progress the development a novel Zika virus vaccine by completing pre-clinical evaluation of this Zika virus DNA vaccine enabling Phase I Human Clinical Trials and generating data for regulatory filing.

Prof. Michael Parker **St Vincent's Institute of Medical Research** **$192,374 (2021-2023)**

A new approach to tackle neurodegenerative diseases

Currently, the leading strategy to remove toxic proteins (namely Abeta and tau) associated with two types of brain deposits found in Alzheimer's disease is to treat people with antibodies that recognise these toxic proteins; this is called 'immunotherapy'. To date, all Abeta immunotherapy trials have had serious efficacy and/or safety concerns, in part because the body's immune response to the therapy has resulted in inflammation in the brain. Professor Michael Parker and his team at St. Vincent's have developed a new type of drug that has the advantage that it does not promote inflammation in the brain and can more effectively cross the 'blood-brain barrier', a barrier which protects the brain from infection but can also block the transport of drugs.

They have tested the drug in brain cells in the lab and found that their novel drug technology successfully increases the removal of toxic Abeta material without promoting increased inflammation. Funding provided by The Mason Foundation (managed by Equity Trustees) and NFMRI is enabling Prof. Parker to complete important pre-clinical trials in animals to take this therapy a step closer to the clinic. This technology holds great promise for future development and has drawn preliminary interest from industry partners.

A/Prof. Leszek Lisowski **The University of Sydney** **$140,000 (2021-2022)**

An all-in-one gene therapy treatment for cystic fibrosis

A/Prof. Lisowski's project aims to develop new gene therapy tools for the treatment of cystic fibrosis (CF). Together with his team, they aim to develop tools that carry the promise of achieving therapeutic efficacy following a single systemic administration of an adeno-associated viral (AAV) vector. Specifically, the project will enable development of two key tools: (1) novel, human lung-tropic AAV vectors for safe and efficient targeting of the basal cells, which give rise to human airway epithelium (HAE). To increase safety and efficacy, the AAV vectors will be specifically de-targeted from the human liver, which is the primary target of most AAVs following systemic delivery. (2) AAV-based gene editing strategy to correct CF causing mutations in the fibrosis transmembrane conductance regulator (CFTR) gene in the basal cells.

Combination of those two novel technologies will form the basis of a powerful gene therapy approach to cure cystic fibrosis. The project will also lead to the development of a novel dual liver-lung preclinical model that will enable preclinical studies not only in gene therapy, but also will be an invaluable tool to other researchers studying the disease and/or developing novel therapeutic options for CF. Funding provided by the Cure4CF Foundation and NFMRI is supporting the preclinical validation of the gene therapy approach to cure CF.

Prof. Stephen Fox **The University of Melbourne** **$144,000 (2021-2022)**

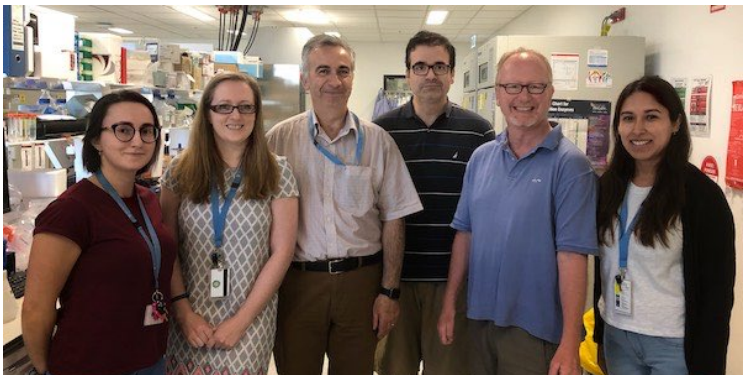
DNA nano biosensor for cancer diagnostics

Precision oncology, which matches a cancer patients' gene test to a specific drug, has transformed the treatment of cancer patients and led to substantial improvements in survival with fewer hospital admissions from therapies with less toxicity, as drugs are only given to patients that respond. Current molecular testing to identify changes in cancer DNA for the above clinical uses is expensive as specialist pathologists and scientists who rely on complex equipment are needed, which often affects patient outcome. It is also slow due to a combination of time for tissue transport to the central test lab and then time taken to perform the assay.

This research seeks to validate an inexpensive (<$100 compared with $1000s using current technologies), rapid and sensitive method to detect genetic mutations that can be used on blood from patients with any tumour type. The identification of such changes will enable screening, diagnosis, prognosis, selection of patients for specific therapies and monitoring of response to treatment. The basis of the assay is an innovative biosensor that detects the presence of abnormal cancer DNA on binding through a change in electrical current. The novel biosensor method Prof. Fox is developing will enable a reduction in the time-critical analyses by days to ensure timely reporting that will help realise the improved outcomes of precision oncology. Funding provided by State Trustees Australia Foundation and NFMRI is supporting further development of the biosensor as well as experiments to delineate the performance characteristics and capabilities of the biosensor to identify different types of mutant DNA.

Prof. Merlin Thomas **Monash University** **$175,000 (2022)**

Development of an inhaled RNA therapy for the prevention and treatment of coronavirus infections



L-R: Alexandra Dimitropoulos, Raelene Pickering, Christos Tiklellis, Carlos Rosado, Prof Merlin Thomas, Alejandra Zuniga Gutierrez

While global research has focused on directly targeting SARS-CoV-2, Prof. Thomas' team's work has focused on the cell-surface protein, Angiotensin-Converting Enzyme 2 (ACE2), which all strains of SARS-CoV-2 use like a 'door' to access and infect cells of the respiratory tract.

Over the last 16-months, they have discovered and developed an RNA therapy that triggers vulnerable cells of the lungs to preferentially generate soluble ACE2, a short form of ACE2 that can't be used by the virus to enter the body. Instead, it acts as a natural decoy-receptor to prevent SARS-CoV-2 infections. At the same time, the expression of the cell-surface door (ACE2), capable of facilitating virus entry, is reduced by their therapy. They have now proven this novel treatment works to prevent coronavirus infection in lung cells and have also shown that it is highly effective in inducing protective changes in ACE2 in the lungs of healthy mice. They also believe this strategy may also reduce the viral load in infected patients and therein protect people's health.

NFMRI funding will support proof-of-concept studies, including pharmacokinetic studies, to ensure the best dose can be efficiently and safely delivered using a nebulizer.

Prof. Jake Shortt **Monash University** **$190,000 (2022-2023)**

Dual-targeted inhibitors of BET bromodomains and PI3-kinase for cancer therapy

In partnership with Prof. Philip Thompson's team at the Monash Institute of Pharmaceutical Sciences, Prof. Shortt's group has developed a designer drug that targets PI3K and BET proteins at the same time in the same cancer cell – a dual-targeted PI3K and BET inhibitor. Both PI3K and BET inhibitors have been developed and show significant clinical activity, particularly in the context of blood cancers. However, the 'addiction' of a cancer cell to a particular pathway or process (ie. PI3K, BET) is not absolute. Therefore, the innovation of dual-targeting cancer drugs represents a potential step-change technology, taking the established models of cancer treatment, such as combination therapies, to a new level and attempting to defeat cancers on multiple fronts in a single compound. A dual acting drug puts the two activities in the 'same place at the same time', potentially enhancing drug synergy, reducing toxicity and drug resistance. The general concept is gathering traction in the research community and our studies to date of this dual targeting paradigm (PI3K and BET inhibition) shows great promise.

Prof. Shortt and Prof. Thompson's teams have demonstrated that targeting both PI3K and BET

proteins together has synergistic anticancer activity, in part due to the ability to prevent cancer cell adaptation and resistance to the inhibition of each target in isolation. NFMRI funding will help support further therapeutic efficacy studies.

A/Prof. Vivien Chen **ANZAC Research Institute** **$193,000 (2022-2024)**

Novel biomarker to predict thrombotic risk in myeloproliferative neoplasms

A/Prof. Chen's team have invented a diagnostic assay that identifies hyperactive platelets known as "procoagulant platelets". These are increased in patients with pathological blood clots, such as those causing heart attack and stroke, but are also increased in a blood cancer called essential thrombocythaemia (ET), in which clotting complications are common (40% of patients) and responsible for 40% of deaths. Until now, no blood test has been able to predict which ET patients will develop blood clots or progress to fibrosis/leukaemia.

This intellectual property protected assay is performed on standard patient blood samples on a clinical flow cytometer – thus has potential to be adapted for use in diagnostic labs around the world. The additional innovation involves coupling with their novel mathematical algorithm to enumerate a particular sub-population of platelets that appears highly predictive of further blood clots (including stroke) in ET patients.

This project is supported in partnership with The NSW Community Foundation, The NSW Community Foundation – Nicholas and Phillis Pinter Trust, the Vernon Sinclair Fund (all managed by Equity Trustees) and NFMRI. This innovation has successfully gone through a development phase proof of concept and internal validation using blood from ET patients, and funding provided will help support further validation studies across different flow cytometry platforms.

Prof. Nicholas Opie **The University of Melbourne** **$50,000 (2022-2023)**

Dr John Raftos AM Medal – Endovascular Neuromodulation

Following the completion of a successful first-in-human trial evaluating the safety and efficacy of the Stentrode to restore communication and independence to those severely paralysed by enabling direct brain control of a computer, Prof. Nicholas Opie is now working to expand the potential clinical applications of his technology through development of a Stentrode suitable for efficacious cortical stimulation.

Our People

A dedicated Board, Research Advisory Committee (RAC) and management team lead our Foundation.

Directors

Directors, qualifications and special responsibilities		Experience
Dr. Rob Sauer **Chairman**	2017-	• Chairman, Echoview Holdings Pty. Ltd. • Director, Biopharm Australia Pty Ltd. And Arthropharm Pty. Ltd. • Admitted as solicitor of the Supreme Court of New South Wales in 1974 • Admitted as Certified Practicing Accountant in 1980 Formerly: • A Founding Director and shareholder of ResMed • Partner, DibbsBarker (1978-2008) • Inaugural Chairman, 150% R&D Tax Concession Committee • Inaugural Chairman, Tassal Ltd (1984-1990)
Mr. John Harkness	1984 -	• NFMRI Chairman (2001-2018) • Partner of KPMG for 24 years and National Executive Chairman for five years • Former Chairman and/or Director of listed or unlisted companies from 2000-2018 including Goodman Group, Sinclair Knight Merz, Reliance Rail Group, Charter Hall Retail REIT and Crane Group • Fellow of the Institute of Chartered Accountants in Australia and the Australian Institute of Company Directors
Dr. John Dixon Hughes OAM *(retired Sept 2022)*	1977 - 2022	• NFMRI Director (1977 – 2022) • NFMRI's RAC Chairman (2000-2018) • NFMRI RAC Member (1977-2022)
Dr. Kevin Hellestrand	2001 -	• Cardiologist and Cardiac Electrophysiologist for 35 years • Co-author of more than 50 journal articles, reviews and book chapters • Fellow of the Royal Australasian College of Physicians, American College of Cardiology, Cardiac Society of Australia and New Zealand, Heart Rhythm Society, European Society of Cardiology

		• Member of the North Shore Heart Research Foundation
Mr. Anthony McGrath *Honorary Secretary and Director*	1997 -	• Founding Partner, McGrathNicol • Board Member, National Rugby League • Non-Executive Director, Servcorp Limited • Member, Institute of Chartered Accountants in Australia • Member, Australian National University Finance Committee • Board Member, 360 Capital Ltd.
Ms. Alison Choy Flannigan *Company Secretary*	2014-	• Company Secretary since 2014 • Partner, Leader, Health & Community, Hall & Wilcox • Member, NSW Law Society • Member, Australian Institute of Company Directors • Publications officer, Healthcare and Life Sciences Committee, International Bar Association • Member, AusBiotech
Emeritus Professor A. Ian Smith Chairman, Research Advisory Committee	2017-	• Emeritus Professor, Faculty of Medicine Nursing and Health Sciences Monash University (from 2020) • Director and Chair, Population Health Research Network (from 2017) • Chair, EuroBioimaging Scientific Advisory Board (from 2020) • Director and Chair, Queensland Cyber Infrastructure Foundation Limited (from 2020) • Member EMBL Council and Finance Committee (from 2020) • Managing Director AIS Life Science Consulting Pty Ltd (from 2021) • Chair, EMBL Australia Council (from 2021) Director, St Vincent's Institute for Medical Research (from 2022)
Prof. Elaine Saunders	2018-	• Adjunct Professor, Faculty of Health, Arts & Design, Swinburne University of Technology • Managing Director, Bingarra ScaleUp Solutions • Health subcommittee, ATSE • Non-Executive Director, Australian National Fabrication Facility • Deputy Chair, Victorian Committee, Pearcey Foundation • Non-Executive Director, Audeara • Advisory Board, Hearing Power

		Formerly: • Executive Chairman, Blamey Saunders hears • Co-founder & CEO, Dynamic Hearing Pty. Ltd. • Non-Executive Director, Alfred Health • Chair, Swinburne University's Innovation Precinct Advisory Board
Ms. Alison Gartner	2020-	• Co-founder, Evidentli Pty. Ltd. • Project Manager, Chimeric Therapeutics Ltd. • Project Manager, Radiopharm Theranostics Ltd. Formerly: • Portfolio Manager, Asia Union Investments Pty. Ltd. • Investment Manager, Bioscience Managers • Alternate Director, Saluda Medical
Emeritus Professor Douglas E. Joshua AO	2020-	• Professor Emeritus, The University of Sydney • Consultant Hematologist, Royal Prince Alfred Hospital • Scientific Advisor and Member, International Myeloma Foundation • Member Scientific Research Committee ARCBS Formerly: • Head of Clinical and Laboratory Hematology, Sydney Cancer Centre • Head of Sydney Local Health District in Hematology • Alan Ng Professor in Medicine at the University of Sydney • Director, Institute of Hematology, Royal Prince Alfred Hospital

Research Advisory Committee

Chairman **Emeritus Professor A. Ian Smith**	Emeritus Professor, Faculty of Medicine Nursing and Health Sciences Monash University
Dr. John Dixon Hughes OAM *(Retired Sept 2022)*	Consultant general surgeon with over 55 years' experience
Prof. Elizabeth Gardiner	Deputy Director of the John Curtin School of Medical Research at the Australian National University, Canberra

Emeritus Professor Douglas E. Joshua AO	Emeritus Professor in Haematology at the University of Sydney and Consultant Haematologist at RPHA.
Prof. Mark von Itzstein	Director of the Institute for Glycomics at Griffith University
Alison Gartner	Lifesciences and healthcare investment professional
Dr. Noel Chambers	CEO with over 35 years' experience in biomedical research, innovation, commercialisation and biotechnology
Dr. Andrew Cottrill	Medical Director, HCF
Prof. Lisa Horvath	Director of Medical Oncology and Director of Research, Chris O'Brien Lifehouse

Management and Administration

Dr. Noel Chambers	Chief Executive Officer
Mrs. Nancy Ranner	Grants, Communications and Engagement Coordinator
Mrs. Linda Hearne	Bookkeeper and Administrator

Dr John Dixon Hughes OAM
1926-2022

Vale Dr John Dixon Hughes OAM

We wish to recognise our Director, Dr John Dixon Hughes OAM, who passed away on Wednesday, 14 September 2022 at the age of 96. John has been an instrumental Founding Member of our Foundation and remained an active Director and Research Advisory Committee Member since 1977. His wisdom, companionship and unique insights will be missed by all.

John was passionate about NFMRI and its role in supporting research to deliver benefits to the community. He was the long term Chair of the Research Advisory Committee and has remained engaged as a member of the committee in recent years.

An astute consultant general surgeon with over 60 years of experience, John was an active, dedicated and passionate leader. Science, medicine and innovations were his passions and he continued to provide valuable advice and distinguished service to medicine until his passing.

He lived a remarkable life, supported by a beautiful family. His 'can-do' attitude, robust motivation and desire to learn supported him in achieving his enormous list of lifelong accomplishments. From being a pilot to a renowned skilled surgeon - nothing stopped John from persevering and succeeding. He was humble and kind, always keen to share his knowledge and history with others, as well as help wherever he could.

We are grateful to have received John's guidance and support throughout all of these years. His stewardship and leadership have been instrumental in not only establishing, but also shaping our Foundation into what it is today.

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