

GRANT-IN-AID AWARDS Presentation 2018

HEARTKIDS GRANTS-IN-AID PROGRAM

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Perpetual



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HeartKids would also like to thank the James and Julia Luff Trust and all of our generous donors.

A WARM WELCOME

On behalf of the HeartKids Board and its Research Committee we warmly welcome you to the 2018 HeartKids Research Presentation.

Each year HeartKids takes this opportunity to showcase the impact of our investment in research and celebrate how this is making a practical difference in the lives of infants, children, young people and adults living with or impacted by childhood heart disease.

HeartKids mission of 'Supporting heart kids for life' recognises the burden of childhood heart disease now extends beyond the infant and early childhood years to encompass new and emerging challenges of a growing population of young people and adults living with the disease. Childhood heart disease, both congenital and acquired, has lifelong consequences which are not yet fully understood. It not only impacts the patient, but also their extended family and the broader Australian community.

Our research agenda is simple: 'To advance the diagnosis, treatment and prevention of childhood heart disease.' This commitment complements our understanding that childhood heart disease patients require lifelong and specialised care from an eco-system of health professionals including special CHD Cardiologists, Surgeons, Mental Health Professionals to name but a few.

A united HeartKids movement is committed to meaningful and sustainable research collaborations that can deliver timely outcomes which make a difference to those people impacted by childhood heart disease. Our future success lies in sustainable evidence-based support and information programs aligned with the most up to date childhood heart disease research.

Last year, HeartKids launched its partnership with the Congenital Heart Alliance of Australia and New Zealand (CHAANZ) to implement the bi-national CHD Registry and National CHD Survey. This strategic collaboration demonstrates the strength of health professionals and consumers working together and driving health sector reform through research and evidence.

Our 30-year commitment to funding world class research was substantially boosted by HeartKids Advocacy to the Australian Government with the announcement by the Hon. Greg Hunt, Minister for Health, that childhood heart disease would be the Medical Research Future Fund's Fifth Mission.



The HeartKids Board and the entire childhood heart disease clinical and research community acknowledges Minister Hunt's leadership and we owe him, as well as heart kid parent and Parliamentary Ambassador Mr Steve Ciobo, Minister for Trade, Tourism and Investment, a debt of gratitude for championing our cause.

The 'HeartKids Conquering Childhood Heart Disease Mission' is a game changing funding initiative with a commitment to accelerate research of a global significance. The fiveyear mission will see an initial investment of \$15 million by the Medical Research Future Fund, \$5 million by HeartKids and a further \$10 million to be donated by philanthropists, pharmaceutical and medical devices industry research and development contributions and/or by way of matched funding through international research collaborations. Over time, additional funding will also be sought from State and Territory Governments.

We warmly congratulate the seven research teams receiving 2018 Grants-in-Aid funding. We know that your research will make a substantial contribution to the childhood heart disease community and our research objectives.

HeartKids acknowledges and thanks Ms Jill Kinghorn, Perpetual Trustees, the James and Julia Luff Trust and the thousands of parents and supporters from across Australia for their donations and generous gifts to fund these research projects.

Our future is very bright with the announcement of the HeartKids Conquering Childhood Heart Disease Mission and the now underway National Childhood Heart Disease Action Plan funded by the Australian Government.

The acknowledgement by the Australian Government of childhood heart disease and its burden and the faith shown in HeartKids in leading these two programs is warmly welcomed and appreciated.

Jan McClelland, AM Chairperson HeartKids Limited

Dr Lisa Selbie, PhD Chairperson HeartKids Research Advisory Committee

DRIVING REAL IMPACT THROUGH GRANTS-IN-AID

The HeartKids Grants-in-Aid program is unique as it provides with childhood heart disease.

capacity specifically directed to congenital/childhood heart disease and enable pilot studies which may lead to larger, longer-term research projects. The critical seed funding

projects, to demonstrate the tangible impact of this research funding program.





HeartKids Grants-in-Aid is funding 7 new projects in 2018 to the total value of \$272,958

SPOTLIGHT: RESEARCH IN TRANSLATION

Direct and timely support for families

In 2016, HeartKids provided Grants-in-Aid funding to Prof Alun Jackson of the Australian Centre for Heart Health (ACHH) to undertake a research project entitled "Families coping with childhood heart disease". ACHH implemented the findings from this initial study and worked with HeartKids Support Teams at the Royal Children's Hospital (RCH), and families, to develop a deeper understanding of the psychosocial impact of a child's condition on the whole family unit. Consultation and interviews also explored families' adaptation, coping and parenting challenges.

Areas covered in the program included:

- the positive psychology of parenting;
- adjusting to a family life with chronic illness;
- strengthening family's coping strategies;
- helping your child to express feelings in early years;
- tuning in to siblings;
- empowering your child to cope;
- couples working together towards improving relationships;
- adjusting, accepting and healing through mindfulness;
- and the role of grandparents in the heart child family.

A Family Coping Pilot Program run by HeartKids, the Australian Centre for Heart Health and Melbourne University was delivered in 2017 to 23 parents of heart kids in metropolitan and regional Victoria with overwhelmingly positive feedback from families. "I'm currently doing a 5 week Heart Child Parent Coping Program run by HeartKids, the Australian Centre for Heart Health and Melbourne University. I can't stress how amazing it has been and has helped me already (it's week 3!). If you see it being run at a location that suits you...PLEASE try and do it. It aims to give you coping strategies when you have children like ours with a chronic illness." Julia Davies, VIC. HeartKids is currently seeking funding in order to roll out this important program nationally. Thanks to the outstanding fundraising efforts by VF Siciliano and Sons, MarketPlace and A&S Wholesale Fruit & Vegetables at the Fruit Auction in Melbourne in November 2017, HeartKids has been able to significantly advance these plans.



From these various sources, a family intervention manual was designed based on a family coping program with five years' worth of evidence of its effectiveness.

• using assertiveness to advocate for yourself and your child;



SPOTLIGHT: RESEARCH IN ACTION

In 2017, HeartKids provided Grants-in-Aid funding to Associate Professor Dr Luregn Schlapbach of The University of Queensland to undertake a research project entitled "Nitric oxide during cardiopulmonary bypass to improve recovery in infants with congenital heart defects (NITRIC trial): a randomised controlled trial."

Despite improvements in survival of patients with congenital heart disease, the perioperative period remains associated with significant morbidities. Given the adverse effects of cardiopulmonary bypass on early recovery and long-term neurodevelopmental there is an urgent need for clinical trials evaluating novel therapies to address these problems.

Two previous pilot studies demonstrated the potential of nitric oxide (NO) – a commonly used medical gas with a very good safety profile - used during cardiopulmonary bypass to reduce bypass-mediated inflammation and fasten post-surgical recovery in children undergoing heart surgery.

In this large multicentre trial planning to enrol 1,320 children below two years of age needing heart surgery, we now aim to confirm whether nitric oxide used during bypass leads to improved patient centred outcomes. The study started in July 2017 at Lady Cilento Children's Hospital Brisbane and all five major paediatric cardiac surgical centres in Australia and New Zealand are now enrolling. The study will be the largest trial done in paediatric cardiac surgery and has the potential to change practice leading to better patient outcomes.

Further follow-up on the study cohort to investigate the impact on long-term outcomes is in planning.

Thanks to initial seed-funding provided by HeartKids Grants-in-Aid, this CTG-endorsed study (Clinical Trials Group of the ANZ Society of Intensive Care) has been successful in obtaining NHMRC funding in 2017. This is a first for the Paediatric Study Group of the ANZ Intensive Care Society. The study is expected to be completed within less than three years.

HeartKids has since worked with the research team and funded an animated video resource to inform parents of the study.

"The start-up funding by HeartKids was essential to get us going, and provide the necessary feasibility data to NHMRC allowing to secure this large grant – so thank you on behalf of the whole team!!!!" Associate Professor. Dr Luregn Schlapbach, Paediatric Intensive Care Unit - Lady Cilento Children's Hospital



EVERY SCAR HAS A STORY

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STRATEGIC AND PRIORITY DRIVEN RESEARCH

HeartKids funds vital research alongside the Grants-in-Aid program. An example of this is strategic investment in long term projects and initiatives such as CHAANZ.

What is CHAANZ?

The Congenital Heart Alliance of Australia and New Zealand (CHAANZ) is a consortium of paediatric, adult and surgical congenital heart disease (CHD) researchers from Australia and New Zealand. CHAANZ was established with initial funding from HeartKids.

The purpose of CHAANZ is to facilitate focused research, promote the need for greater resources and to build a lifelong continuum of care for all people with CHD. We hope that by working together, we can better understand the complexity and burden of CHD across the lifespan.

The National Congenital Heart Disease Survey

The National Congenital Heart Disease Survey is an initiative that aims to capture selfreported information directly from the community. The National Congenital Heart Disease Survey involves an online self-registration portal which collects information on basic demographics, point prevalence and perceived health burden of living with CHD.

The Survey is the first step for gathering pivotal information to help provide a clearer picture of the entire population in Australia impacted by CHD. Patients and their carers can register and participate at **chaanz.org.au**

Strategically it is hoped that this data will help drive policy on the provision of optimal and sustainable health services. Core aspects of this project will include understanding early and late outcomes, transitional care requirements, and describe access to ongoing care for all patients.

Congenital Heart Disease Registry of Australia and New Zealand

The National Congenital Heart Disease Survey portal is the first step in developing an Australia and New Zealand Congenital Heart Disease Registry that we hope will be a mandatory tool to provide best care to a growing population.

The CHAANZ Steering and Governance Committee includes the following clinical experts:

Professor David Celermajer (Chair), Dr Tom Gentles, Dr Rachael Cordina, Dr James Ramsey, Dr Andrew Bullock, Dr Gavin Wheaton, Dr Patrick Disney, A/Prof Michael Cheung, Prof Yves d'Udekem, A/Prof Christian Brizard, A/Prof Robert Weintraub, A/Prof Leeanne Grigg, Dr Clare O'Donnell, A/Prof Gary Sholler, A/Prof Julian Ayer, A/Prof Nadine Kasparian, Dr Mugur Nicolae, A/Prof Rob Justo, A/Prof Nelson Alphonso, Prof Simon Stewart, Dr Lisa Selbie, Mr Mark Brooke and A/Prof Geoff Strange.

CHAANZ has been funded by HeartKids with support from The Kinghorn Foundation, The Pinnacle Charitable Foundation, Tata Consultancy Services and other generous sponsors and donors. artKids | Grants-in-Aid Awards Presentation 2018



RECIPIENTS OF THE 2018 GRANTS-IN-AID PROGRAM

Specimen collection cataloguing and re-classification **Principle Investigator:** Dr Bryn Jones Institution: Royal Children's Hospital Additional Investigator: Prof Jim Wilkinson

Dr Bryn Jones is Deputy Director of Cardiology and Clinical Lead in Cardiac MRI at the Royal Children's Hospital (RCH). He also consults at the Fetal Management Unit at the Royal Women's Hospital. He graduated from the University of Queensland and completed his Paediatric Training at the Royal Children's Hospital, Brisbane prior to training in Paediatric Cardiology at The Prince Charles Hospital in Brisbane and the Royal Children's Hospital, Melbourne. He undertook further Cardiac MRI training at the Royal Brompton Hospital and Great Ormond Street Hospital in London before returning to RCH.

Project Description

The use of heart specimens for the teaching of paediatric cardiology and cardiac surgical trainees is well established and is widely regarded as being an essential component of education and training. The cardiac specimen collection at RCH has been accumulated over more than fifty years and is an invaluable resource for such research into and for teaching of the pathology of congenital heart defects. Currently the pathology collection at RCH is under-utilised and is in urgent need of careful re-evaluation and comprehensive cataloguing in order to ensure that specimens are properly preserved and are available for these purposes.







RECIPIENTS OF THE 2018 GRANTS-IN-AID PROGRAM

Precision medicine in CHD: Genetic variants guiding post-operative clinical management

Principle Investigator: Prof David Winlaw MBBS, MD, FRACS

Institution: The University of Sydney

Additional Investigators: Dr Gillian Blue, Dr Eleni Giannoulatou, Dr Jon Egan and Prof Seema Mital

David completed an MD (Surgery) supervised by Professor Peter Macdonald at St Vincent's Hospital Sydney and UNSW in 1996, publishing in Lancet and Transplantation. He commenced consultant practice in paediatric cardiac surgery in 2003 after a Fellowship year in the UK. He was appointed as Professor in Paediatric Cardiac Surgery at University of Sydney in 2014. He has been Head of Cardiothoracic Surgery since 2009.

David was a Career Development Fellow of the Heart Foundation of Australia, 2006 to 2010. He is currently a CI on 2 NHMRC Project grants, is CI on an NHMRC Partnership Grant and AI on a Program Grant.

Project Description

Neonatal cardiac surgery for transposition of the great arteries (TGA) is a success story, with most babies now surviving well into adulthood with a 'normal' circulation. Outstanding concerns are early post-operative cardiac dysfunction and other serious complications requiring prolonged intensive care in up to a third of patients. Our aim is to use patient genotype to target therapy and achieve better outcomes. We will identify individual variation in genes associated with the development of low cardiac output and length of intensive care stay that will form the basis of a personalised approach to post-operative care.

This project is proudly supported by Kiwanis Charitable Foundation.

Identifying the underlying genetic cause of inherited arrhythmia syndromes in early childhood

Researcher: Dr Jodie Ingles

Institution: Cententary Institute

Dr Janine Smit, Dr Christian Turner and Dr Andrew Davis

Dr Jodie Ingles is an early career researcher (NHMRC/National Heart Foundation co-funded Early Career Fellow, 2012-2015 and National Heart Foundation Future Leader Fellow, 2016-2019). Her strong interest has focused on patients and families with a variety of genetic heart diseases and the most effective ways to manage these families, motivated by her 14 years' experience as a cardiac genetic counsellor.

She leads the Clinical Cardiac Genetics Group, Molecular Cardiology Program. Since 2014 she has independently funded her group, currently including: 1 PhD, 2 master's research students, 1 full-time post-doctoral researcher and 1.6FTE research assistants. At almost 6-years post-PhD, she has 75 publications and 4 book chapters. She completed a Master of Public Health, University of Sydney.

Project Description

Heart disease in children results in significant symptoms and can ultimately lead to arrhythmias and premature death. One form of heart disease in children relates to abnormalities in the electrical activity of the heart, many of which are inherited. Inherited arrhythmia syndromes can be difficult to diagnose on standard clinical tests, making clinical screening of family members challenging.

Better understanding of the genetics may be useful in predicting clinical outcomes of affected children, but most importantly, can more accurately determine risk to other family members. The proposed study will use the latest genetic technologies to identify the genetic causes and to use these results in clinical care.





Additional Investigators: Prof Christopher Semsarian, Dr Richard Bagnall,



GRANTS-IN-AID PROGRAM

Development in 8/9 year old children after major cardiac and non-cardiac surgery

Principle Investigator: A/Prof Karen Walker PhD, MClinNurs, BAppSc (Nurs), RN

Institution: Grace Centre for Newborn Intensive Care, Westmead Children's Hospital

Additional Investigator: Natalie Fairbairn

Associate Professor Karen Walker is a Clinical Research Fellow in the Grace Centre for Newborn Care at the Children's Hospital at Westmead. Karen completed her PhD in 2011 researching outcomes of infants admitted to neonatal intensive care units with her thesis entitled "Neurodevelopmental outcome at one year of age of infants who have undergone early major cardiac or non-cardiac surgery".

Project Description

The Development After Infant Surgery (DAISy) study is a world-first prospective longitudinal cohort study evaluating the developmental outcomes of infants who underwent cardiac and non-cardiac surgery compared to healthy newborns. We showed that at one and three years of age infants who underwent surgery as a baby had increased delay, with infants having cardiac surgery at the highest developmental risk. These children may continue to be at risk at school age, thus the third phase of our study (DAISy8) is currently underway evaluating the educational and developmental skills of the children in our original cohort at 8/9 years of age.

advanced MRI techniques

(EEG), FRACP

Institution: The Royal Children's Hospital

Additional Investigators: Prof Stuart Grieve and Prof Yves d'Udekem

Mark Mackay is a Monash University graduate. He trained in general paediatrics at the New Children's Hospital, Sydney and the Royal Children's Hospital, Melbourne from 1993-1996. He completed his neurology training at the Royal Children's and Austin Hospitals from 1997-1999. This was followed by a two year Paediatric Neurology and Epilepsy Fellowship at the Hospital for Sick Children, Toronto, Canada from 1999-2001, returning to the Royal Children's Hospital in July 2001. He is an Honorary Research Fellow with the Murdoch Children's Research Institute and the Florey Neurosciences Institute. He has over 10 years' experience in the conceptualisation of paediatric stroke and epilepsy research projects and has a publication and presentation profile in these fields. He has established a dedicated paediatric stroke program; the first of its kind in Australia and is a major contributor to the International Paediatric Stroke Study. His stroke research interests include application of advanced MRI imaging techniques in childhood stroke and the development of strategies to reduce time to stroke diagnosis.

Project Description

Children with complex heart defects are at increased risk of physical, learning and behavioural problems. We will investigate the utility of advanced MRI brain imaging techniques to investigate brain structure and function, in people with a single ventricle chamber, palliated with a Fontan operation. We will assess volumes of different brain structures, and the microstructural organisation of white matter fibre tracts, which are the brain's information highways. Subjects with better outcomes will be compared to those with worse outcomes. Understanding of imaging contributors to outcomes may help us develop ways to optimise management strategies including timing and type of surgery.





Investigating neural correlates of outcome in Fontan patients using

Principle Investigator: A/Prof Mark Mackay MBBS, PhD, DRANZOG, DCSCN



RECIPIENTS OF THE 2018 GRANTS-IN-AID PROGRAM

Establishing the Queensland Paediatric Cardiac Service CHD LIFE **Program Database**

Principle Investigator: A/Prof Robert Justo MBBS, FRACP, FCSANZ

Institution: Lady Cilento Children's Hospital

Additional Investigators: A/Prof Nelson Alphonso Ms Karen Eagleson, Ms Jess Suna and Ms Janelle Johnson

Dr Robert Justo is the Director of Paediatric Cardiology for Queensland Paediatric Cardiac Service. He was actively involved with the transition of this service to Lady Cilento Children's Hospital over the past decade. Interventional cardiology is his field of primary clinical interest, with significant involvement in care of cardiac transplant patients and indigenous cardiac health issues. Areas of research interest have examined long-term outcomes for children undergoing cardiac surgery, with a particular focus on neurodevelopmental and quality of life outcomes. He has actively supported the development of the CHAANZ Registry with other Australian clinicians and HeartKids.

Project Description

Children with congenital heart disease (CHD) are surviving into adulthood with little known about their developmental course over time. Each year over 100 Queensland children undergo open heart surgery before 12 months of age, placing them at risk of poorer developmental outcomes.

The CHD LIFE (Long-term Improvement in Functional Health) Program provides targeted developmental services, and through a partnership project developed a state-wide model of care to meet the developmental needs of these children. This project proposes establishing a centralised database to support long-term follow up and research activity, measure state-wide model success and inform local and international registries.

Funded by the James & Jutta Lauf Foundation and HeartKids' Grants-In-Aid

Cord blood cell therapy for babies with Hypoplastic Left Heart Syndrome

FAHA

Institution: Murdoch Children's Research Institute

A/Prof Christian Brizard

A/Prof Salvatore Pepe initially trained in clinical pharmacology and biochemistry. After completing a PhD in cardiac physiology he undertook four years of postdoctoral training at the National Institutes of Health, National Institute of Aging, and as a Fogarty Fellow at Johns Hopkins Bayview Medical Center, Baltimore, USA. Before joining Murdoch Children's Research Institute, he was Head of the Laboratory of Cardiothoracic Surgical Research, Department of Cardiothoracic Surgery, Alfred Hospital and Adjunct Senior Lecturer, Department of Surgery, Monash University. A/Prof Pepe's research has been focused on dysfunctional mitochondrial and cellular metabolism in heart injury, heart failure, and translation of cardioprotection therapies in heart transplantation and cardiopulmonary bypass surgery. In mid-2008 he was invited to move his laboratory to the Murdoch Children's at the Royal Children's Hospital, Melbourne, to undertake the challenge of translating paediatric heart research to therapy.

Project Description

This study tests the safety of our novel 'first in human' heart treatment with placental cord blood stem cells in 12 babies with hypoplastic left heart syndrome (HLHS) during their first of three surgical operations (at day three of life). Our strategy is to stimulate heart muscle growth as early as possible, and strengthen heart function to avoid the high incidence of heart failure and death that occurs before the second operation at three months. Demonstration of the safety of this new therapy will facilitate larger studies, including multicentre clinical studies to determine improvements to immediate and long-term post-operative recovery with fewer life-threatening complications.





Principle Investigator: A/Prof Salvatore Pepe, PhD, BScHonsMed, FCSANZ,

Additional Investigators: A/Prof Michael Cheung, Dr Ngaire Elwood and

2017 FUNDED PROJECTS

Project Title: ACE inhibitor cessation in the setting of well-functioning Fontan hearts **Principle Investigator:** A/Prof Yves d'Udekem Institution: Murdoch Children's Research Institute

Project Title: Clinical and genetic studies in children with left ventricular noncompaction cardiomyopathy (LVNC) Principle Investigator: Prof Chris Semsarian Institution: Centenary Institute

Project Title: Mechanisms and predictors of cardiovascular risk in children following Kawasaki disease Principle Investigator: Prof David Bugner Institution: Murdoch Children's Research Institute

Project Title: Modelling CHD in a dish using IPS cells and massively parallel sequencing **Principle Investigator:** Prof Richard Harvey Institution: Victor Chang Cardiac Research Institute

Project Title: Burden of rheumatic heart disease: comprehensive measurement to drive the Endgame **Principle Investigator:** A/Prof Judity Katzenellenbogen Institution: The University of Western Australia

Project Title: Nitric oxide to reduce cardiopulmonary bypass-induced inflammation after cardiac surgery in children **Principle Investigator:** A/Prof Luregn Sclapbach Institution: The University of Queensland

Project Title: Early detection of hypertension in aortic coarctation using genome sequencing Principle Investigator: Prof Fadi Charchar Institution: Federation University Australia

2016 FUNDED PROJECTS

Project Title: Families coping with child heart disease Principle Investigator: Prof Alun Jackson Institution: Australian Centre for Heart Health

associations with neurodevelopmental outcomes Principle Investigator: Dr Gillian Blue **Institution:** The Sydney Children's Hospitals Network

Fontan circulation

Principle Investigator: Dr Rachael Cordina Institution: Royal Prince Alfred Hospital

congenital heart disease

Principle Investigator: Dr Jonathan Mynard Institution: The Royal Children's Hospital

correlates of CHD treatment

Principle Investigator: A/Prof Nadine Kasparian **Institution:** The University of NSW

Institution: The University of Queensland

- Project Title: Comprehensive genetic analysis in neonates with CHD and
- Project Title: Neurocognitive outcomes in children and adults with a
- **Project Title:** Optimising heart function in survivors of surgery for
- Project Title: Parents' role in buffering the emotional and neurobiological
- **Project Title:** Nanovaccines against rheumatic heart disease Principle Investigator: Dr Rachel Stephenson / Dr Waleed Hussein



2015 FUNDED PROJECTS

Project Title: Clinical genetic studies in children and families with cardiomyopathy Principle Investigator: Prof Chris Semsarian Institution: Centenary Institute

Project Title: Exercise-induced hypertension in childhood following neonatal aortic coarctation repair

Principle Investigator: A/Prof Yves d'Udekem

Institution: Murdoch Children's Research Institute

Project Title: Outcomes following a cardiac procedure in the first six years of life Principle Investigator: Dr Samantha Lain

Institution: Kolling Institute Sydney

Project Title: Functional health in adolescents who have undergone open heart surgery in infancy

Principle Investigator: Dr Christian Stocker Institution: Mater Children's Hospital

Project Title: Measuring energetic efficiency in adult congenital heart disease using 4D flow MRI

Principle Investigator: Prof Stuart Grieve

Institution: Heart Research Institute, Charles Perkins Centre (University of Sydney)

MEET THE RESEARCH ADVISORY COMMITTEE



Dr Lisa Selbie, PhD





Jemma Lawson

Dr Siiri lismaa, PhD



Claire O'Donnell

Graham Nunn







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