

## The INFORM2 Clinical Trial opens in Australia

The [INFORM2 Trial](#) has officially opened in Australia. This trial is an exploratory multinational phase I/II combination study of Nivolumab and Entinostat in children and adolescents with brain cancer. The trial aims to increase survival rates with the use of these two immunotherapy agents.

This is an international trial lead by the Hopp Children's Cancer Center Heidelberg, Germany and The Perth Children's Hospital is the first Australian site to open for recruitment. The Sydney Children's Hospital and the Royal Children's Hospital Melbourne will follow shortly and will join other centres in Germany, The Netherlands, Sweden, and France.

NSW Health has provided Luminesce Alliance partners, the Sydney Children's Hospitals Network (SCHN) and the Children's Cancer Institute (CCI) \$1 million over four years to support this trial and as a means of demonstrating that NSW has the capacity and capability undertake international clinical trials of this calibre and clinical potential. The Lead Investigator is Associate Professor David Ziegler.



Associate Professor  
**David Ziegler**  
Head, Clinical Trials  
Program, Kids Cancer  
Centre, SCHN Randwick and  
Group Leader, Targeted  
Therapy Group, CCI

## Our Computational Biology team joins CAVATICA to collaborate on the exchange and analysis of paediatric cancer data

[CAVATICA](#) is an international cloud-based platform that allows partners to collaborate through the sharing and analysis of paediatric cancer data.



In 2018, the Australian Bioinformatics Data Commons (AusBioCommons) project, funded by the Australian Research Data Commons (ARDC) was launched, to establish an Australian arm of the CAVATICA platform.



The Precision Medicine Program Team [Computational Biology Program \(PPM2\)](#) has been working closely with AusBioCommons by leading a flagship research project that will integrate RNAseq data from Australian patients with that from 1000 patients from the United States.

As part of the AusBioCommons collaboration, the PPM2 team hosted a series of workshops in early February at the Children's Cancer Institute, to provide training on the use of the Australian CAVATICA platform and the development of genomic analysis applications. The workshop included Dr Allison Heath, the Director of Data Technology and Innovation and two engineers from the US based CAVATICA platform. A [webinar](#) was delivered from the workshop to showcase the potential of the platform to Australian users.

This exciting partnership will see the establishment of the Australian Bioinformatics Commons Paediatric Pathfinder Project enabling data sharing between Australian and American researchers. The first project will see RNA-Seq brain-tumour data from the ZERO cancer program (Aus) merge with the Children's Brain Tumour Tissue Consortium (USA) to identify novel brain tumour subtypes.

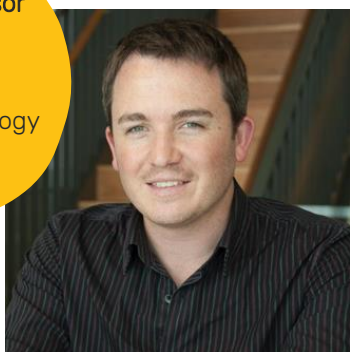
The early outcomes of this project have been substantially reduced analysis costs, international knowledge sharing and collaboration. Importantly, AusBioCommons may become the preferred way to enable sharing of large-data precision medicine data generated by projects across the Luminesce Alliance, including the Cancer Predisposition Screening Program ([PPM3](#)).

## In the Spotlight

### Associate Professor Mark Cowley PhD

Associate Professor Mark Cowley is a bioinformatician, who leads the Luminesce Alliance Computational Biology Program ([PPM2](#)), based at the Children's Cancer Institute ([CCI](#)).

Associate Professor  
Mark Cowley  
Group Leader,  
Computational Biology  
Group,  
CCI



Mark's research involves developing bioinformatics approaches to better understand the molecular basis of human disease, with a highly translational focus.

Mark leads a multidisciplinary team of 14 researchers, engineers and students who have developed scalable cloud-based technologies that allow rapid, real-time genomic analysis, integration, prioritisation and interpretation of whole genome and RNA sequencing data.

Mark's other research interests include understanding the noncoding genome through the use of whole genome sequencing, and an improved understanding of factors underlying inherited cancer predisposition.

### Computational biology group @ CCI

The Computational Biology group at CCI is a multidisciplinary team of 14 postdoctoral researchers, engineers, and students.

**Dr Mark Pinese** leads our cancer risk research while **Dr Marie Wong-Erasmus** leads our genome informatics engineering efforts to build better technologies, enabling large-scale precision medicine. **Dr Mustafa Syed** recently joined the team to focus on liquid biopsies and **Dr Irene Chen** on developing paediatric cancer knowledgebase systems to enable data-driven precision medicine recommendations and accelerate discovery.

Our team of talented research assistants, PhD and undergraduate students are critical to the success of the Paediatric Precision Medicine Computational Biology Program Team ([PPM2](#)).

The Computational Biology Program (PPM2) will see the integration and interpretation of 'omic (i.e. genomic sequence, protein, metabolite, and microbiome information), biological and clinical data to accelerate translation of research findings into clinical environments and improve patients' access to new clinical trials, as well as inform new programs for prevention and earlier diagnosis of disease in the community.

## In the Spotlight

### Dr Anai Gonzalez-Cordero PhD

I am a research scientist with a background in developmental and stem cell biology.



**Dr Anai Gonzalez-Cordero**  
Paediatric Precision Medicine  
Stem Cell Medicine  
Group Leader  
Head, Stem Cell and Organoid  
Facility, CMRI

Stem cell research provides an exciting model and technology to investigate numerous areas of research.

My interests include the use of stem cells to generate mini human organs in the dish, called organoids, to understand mechanisms of disease and develop new treatments. My passion for translation research developed during my doctoral work in Prof Robin Ali laboratory, at the Institute of Ophthalmology (UCL). Prof Ali is a world-leader in gene therapy in the eye, he was the first to establish proof-of-concept for AAV ocular gene therapy consequently leading one of the world's first clinical trials of gene therapy. Together, we developed a programme of research to establish the differentiation of mouse and human pluripotent stem cells into retinal cells, specifically photoreceptors, for their use as donor cells in retinal transplantation paradigms. These studies established proof of concept for stem cell therapy as a treatment for blindness due to retinal degeneration.

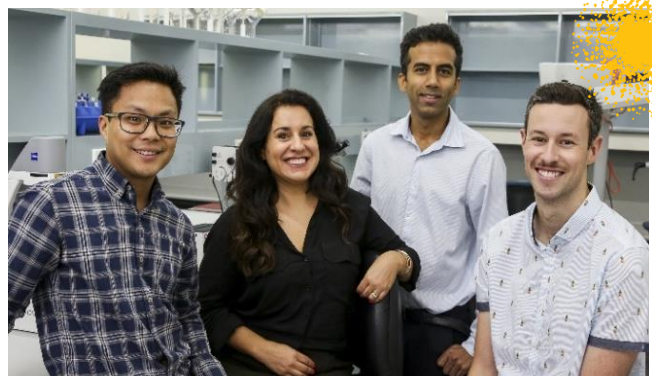
I joined the Children's Medical Research institute ([CMRI](#)) in a joint appointment with University of Sydney as a Group leader in 2019. CMRI offered the opportunity to continue my priority of combining basic and translational research focusing in stem cell-based gene and cell therapies for deafness and blindness.

My technical expertise in developmental biology and stem cells biology, including induced pluripotent stem cells and their derivatives organoids also prompt CMRI to offer me the opportunity to establish a The Stem Cell & Organoid Facility to support Australian research to have access to these tools.

### Stem Cell Medicine Group and the Stem Cell and Organoid Facility

We have assembled a team composed of senior members from the former UK group and research assistants and research students with basic, translational and clinical expertise in stem cell differentiation, cell transplantation and disease modelling, and relevant experience in the retinal medicine and stem cell biology.

It has only been a year since we started in the lab, but we have been very busy. Our team has set up a workflow to culture a number of embryonic and induced pluripotent stem cell (iPSC) lines and their derivative cells and tissues. We have engaged CMRI's state of the art Biomedical Proteomics and Single Cell Genomics facilities to perform preliminary scRNA sequencing and proteomics experiments in brain and retinal organoids. We have also partnered with [Vector and Genome Engineering Facility \(VGEF\)](#) to edit stem cell lines and the Translational Vectorology Group to test new gene therapy Adeno-associated Viruses (AAVs) in stem cell derived-organoids. [Read more.](#)



From left to right: Scott Lee, Research Assistant, Milan Fernando, Senior Research Assistant; Dr Anai Gonzalez Cordero, Principal Investigator & Facility Manager Stem Cell Medicine Lab; and, Grady Smith, Research Assistant



## Governance

Luminesce Alliance recently welcomed two new Board Directors following the recent departures of Mr David Nott who is being replaced by Ms Elizabeth Crouch AM, and Professor Nicholas Fisk, UNSW Deputy Vice-Chancellor (Research) who is being replaced by Professor Sean Emery.

A welcome also to our new pro term Audit and Risk Management Committee member, Dr Abby Bloom. The committee is chaired by Board Director [Jason Murray](#). Read more about our Directors by visiting our [website](#).

### Elizabeth Crouch AM

BEc M CyberSec

Elizabeth Crouch is Chair of the Sydney Childrens' Hospitals Network, the Customer Owned Banking Association and SGS Economics and Planning. She is also a non-Executive Director of ReadyTech Holdings and Bingo Industries.



Ms Crouch Chairs the Non-Government Schools Not for Profit Advisory Committee and is on the Boards of the NSW Institute of Sport and Health Infrastructure. She Chairs Audit and Risk Committees for the City of Sydney, IPART and RailCorp and is also the Chair of CREST ANZ, an organisation that registers ethical cyber security testing companies.

Ms Crouch is Emeritus Deputy Chancellor of Macquarie University and has held previous NED roles on the Boards of Chandler Macleod Group, McGrath Estate Agents, RailCorp and the Macquarie University Hospital. She was previously on the Board of Western Sydney Local Health District and from 2016-2018 was a Trustee of the Museum of Applied Arts and Sciences, retiring in December 2018.

She spent 16 years with the Federal Government including with Federal Health and more than a decade in the private sector including as Chief Executive of the Housing Industry Association.

### Professor Sean Emery

BSc, PhD Brunel

Professor Sean Emery is the Senior Vice Dean of Research and Operations at UNSW Medicine. He has taken on incremental levels of organisational responsibility, from Departmental to Institute and more recently Faculty leadership roles and played a significant role in the development and implementation of strategic planning in each setting.



He is an internationally recognised clinical scientist and his key research outcomes have translated directly into international treatment guidelines for HIV infection. His work has contributed significantly to our understanding of the pathogenesis of HIV disease and the parallel impact of treatment toxicities. He has had considerable success with competitive domestic and international grant funding and an enviable track record of funding from the private sector (career total >AUD150m).

Professor Emery has supervised successful HDR students and mentored numerous professionals whose careers have blossomed in centres around the world. He is drawn to the challenges of a richer administrative and leadership role within a tertiary environment with a strong research commitment and focus to enhance the core remit of education.

### Dr Abby Bloom

MPH PhD FAICD

Dr Abby Bloom is an experienced non-executive company director with special expertise in non-financial risk. Trained in Epidemiology and Public Health at Yale,



Abby received her PhD in Medicine (Public Health) from Sydney University, where she is currently an Adjunct Professor. Her own research has concentrated in applied public health and chronic disease epidemiology, including the misuse of over-the-counter paediatric medication, the

physical and psychosocial impacts of social isolation among older people, and translation of health policy into programs in Jamaica West Indies and Senegal.

She is an expert in hospital Public-Private Partnerships, and has advised The World Bank, UNICEF, the IFC, AusAID and USAID. Abby has co-founded 3 medical device companies, and most recently has developed a digital social enterprise to help families manage the ageing journey.

She is currently a board member of Sydney Water, The Sydney Childrens' Hospitals Network, and The State Insurance Regulator. And she serves on several Independent Audit and Risk Committees.

## The Torch

## Luminesce Alliance Research Strategy

Luminesce Alliance held a Research Strategy Workshop in April for its long-term shared vision to identify current and emerging research priorities. The virtual workshop had 55 participants attending to hear expressions of interest registered from 35 researchers in areas of:

1. Continuing the precision medicine emphasise in cancer and inherited monogenic diseases, with expansion of disease areas beyond those covered art present including inherited immunological diseases.
2. Neurological disorders within the first 2000 days of life.

The workshop identified innovative ideas and opportunities that are now being progressed to help shape the next phase of the paediatric precision medicine program.

An exciting piece of work is also currently underway looking at a transformational integrated bio psycho-social framework for neuro developmental disorders (NDD). Over a dozen researchers are collaboratively mapping out this proposal, which is being coordinated by Professor Russell Dale, Associate Professor Michelle Farrar, Associate Professor Susan Woolfenden.



From left to right:

**Professor Russell Dale**

Professor of Paediatric Neurology  
Clinical Director Kids Neuroscience Centre  
NHMRC Investigator 2021

**Associate Professor Michelle Farrar**

Associate Professor of Paediatric Neurology  
Lead, Neuromuscular Diseases Clinical and Research Group SCH & UNSW  
Research lead (Clinical Neurology) NSW/ACT Newborn Screening Pilot for SMA  
Principal Investigator genomics clinical trials for SMA  
NHMRC Investigator 2021-6

**Associate Professor Sue Woolfenden**

NHMRC Senior Research Fellow 2019-2022, UNSW  
Adjunct Associate Professor, School of Public Health, USYD  
Senior staff specialist in the Department of Community Child Health SCHN.  
Deputy Lead Early Life Determinants of Health, Clinical Academic Group, SPHERE

To find out more about the Luminesce Alliance Research Strategy simply email us at: [info@luminesce.org.au](mailto:info@luminesce.org.au)

### Our Partners



### Contact us:

E: [info@luminesce.org.au](mailto:info@luminesce.org.au)

214 Hawkesbury Rd, WESTMEAD NSW 2145 Australia

[luminesce.org.au](http://luminesce.org.au)

**LUMINESCE**  
Alliance

Innovation for Children's Health

