

Luminesce Alliance

Ten years of impact
for kids and families



LUMINESCE
Alliance

Innovation for Children's Health



2026



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Acknowledgment of Country

Luminesce Alliance acknowledges the Traditional Custodians of Country throughout Australia and their connections to land, sea and community. We pay our respect to their elders past and present and extend that respect to all Aboriginal and Torres Strait Islander peoples today.

Our Vision

is to be Australia's most groundbreaking, innovative, and translational paediatric research alliance, dedicated to changing children's health worldwide.

Our Mission

is to serve as a leading voice on state, national, and international stages, ensuring the widespread dissemination and effective implementation of our research to directly enhance the health of children.

Our Purpose

is for Luminesce Alliance to collaboratively reshape the global landscape in paediatric research.



Supported by the



Luminesce Alliance – Innovation for Children's Health is a not-for-profit cooperative joint venture established with the support of the NSW Government to coordinate and integrate paediatric research.

Thank you to our Board Members for their constant support and wisdom. You can read more about our Board Members on our [website](#).

Cover image: Our research supports gene therapies that have the potential to change Holly's future. The challenge is ensuring children like Holly can access it. See Holly's story on page 17.



Message from the Chair

The Luminesce Alliance journey began in 2016 when the Children’s Cancer Institute, Children’s Medical Research Institute and Sydney Children’s Hospitals Network came together with a simple but powerful belief: that no single organisation could achieve for children what could be achieved together.

The vision soon attracted UNSW Sydney and the University of Sydney, creating a unique partnership that has successfully consolidated paediatric medical research in NSW, brought together clinicians and researchers, and seen discoveries quickly translated into treatments for children with rare diseases, cancer and neurodevelopmental disorders.

Work we’ve supported means that every baby born in Australia today is screened for Spinal Muscular Atrophy. There are gene therapies that can cure blinding eye diseases and other genetic conditions. Treatment of child cancer has been revolutionised by precisely targeting the cancer’s genetic code.



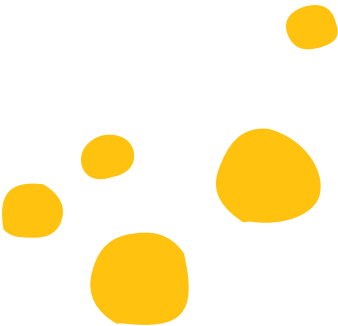
- L to R: Kathryn Greiner AO and Anastasia Ioannou.

We have changed the paediatric research ecosystem by providing researchers support that enables them to innovate and flourish, and our expertise at delivering advanced therapeutics to kids is among the best in the world.

Perhaps the thing I’m most proud of is the way we’ve created a hunger to do more for children with rare diseases and cancer. We’ve seen other organisations keen to join the collaboration, and our reach has extended far beyond NSW to impact paediatric medicine nationally and internationally.

It has been a huge privilege to chair this organisation over the last 10 years. I look forward to everything we are still to achieve.

Kathryn Greiner AO
Chair
Luminesce Alliance



Message from the Executive Director

Since I became Executive Director of Luminesce Alliance in 2019, I have been astonished at how our work has driven change for children.

Our initial focus was to pioneer paediatric precision medicine, a relatively new concept at the time. A decade later, precision medicine has become embedded in the NSW health system and we have seen outstanding results that have changed lives.

More recently, our Enabling Platforms created a precision medicine ecosystem across organisations that has enhanced access to resources for paediatric researchers and clinicians. This work has expedited discoveries, accelerated translation into the health system, and improved outcomes and experiences for children.

We’ve provided a more than tenfold return on the NSW Government’s initial investment. We’ve attracted new talent to the state, created significant new infrastructure, and positioned NSW as a leader in advanced therapeutic clinical trials.

Now it’s time to turn our attention to the next great challenge — delivering more equitable, efficient and high-impact health outcomes for all children, regardless of their background or where they live. Data, artificial intelligence and the roll-out of the Single Digital Patient Record offer us an unprecedented opportunity to make this happen.

I’d like to take this opportunity to thank every family, clinician and researcher who has been a part of this incredible journey.

Ms Anastasia Ioannou
Executive Director
Luminesce Alliance



What we've achieved

We improved health outcomes for kids

Paediatric drug discovery accelerated through Therapeutic INnovations for Kids (THINK)

Better diagnosis of childhood cancers, neurodevelopmental disorders and genetic diseases



34+

Developed analysis tools to improve genomic analysis for better diagnosis and treatment



↑93%

Advanced therapeutics clinical trials offered to kids in NSW

We made precision medicine routine

Targeted therapy offered to **every child with cancer** in Australia through ZERO

Newborn screening for Spinal Muscular Atrophy **for every baby** born in Australia

Health economics evidence for precision medicine



300+

healthcare professionals trained in supporting precision medicine delivery in clinical trials and health care settings

Developed precision medicine educational resources that empower healthcare professionals, patients, and families

We achieved a significant return on investment



\$476.3M

funding leveraged from **\$45M investment** (10.6x ROI)



40

R&D IPs and commercial relationships facilitated



530+

national and international collaborations

We supported world-leading science



410+

research projects



275+

peer-reviewed publications



520+

presentations nationally and internationally



\$3.1M

seed funding for innovation projects

Advanced **gene therapies**

Discovered **new therapeutic targets for cancer**

Breakthrough in **blinding genetic eye disease**

Discovered biomarkers for neurodevelopmental disorders

Developed and validated **CRISPR-Cas13**

We invested for increased capacity and capability in NSW



>380

STEM professionals supported and upskilled



40+

clinical trials facilitated

Vector and Genome Engineering facility

Stem Cell and Organoid Facility

Australia's largest computational biology team focused on children's cancer

Positioned NSW as leader in paediatric data linkage **advancing data-driven approaches** to improve health outcomes

Commitment to cultural capability through **ATSI and CALD** training workshops and support

We improved the experiences of kids and families



20+

equity and psychosocial projects

Created and implemented **solutions to address health disparities** for priority populations

Supported the **EPIC-CP social prescribing model** for children with cerebral palsy

Targeted resources for families – clinical trial resources, increasing understanding of advanced therapeutics, such as gene therapy, embedded consumer perspectives into trial design and delivery, multilingual resources and animated videos to support families

Psychosocial supports for families including siblings, grandparents



Our story

2015 – The beginning

We were originally established as Paediatrio, a unique Australia-first collaboration bringing together NSW’s three paediatric research organisations – the Children’s Cancer Institute (CCI), the Children’s Medical Research Institute (CMRI) and the Sydney Children’s Hospitals Network (SCHN, the largest provider of clinical services to children in Australia).

Paediatrio created a common mission and a united voice for paediatric research in NSW, with a focus on childhood cancer and rare diseases. Our primary aim was to bring together research and clinical care, meaning treatments could be offered more quickly for children in our hospitals.

About rare diseases

There are thousands of rare and ultrarare diseases, including cancers. Some are so rare that they don’t even have a name. For 95% of them there’s no known cure.

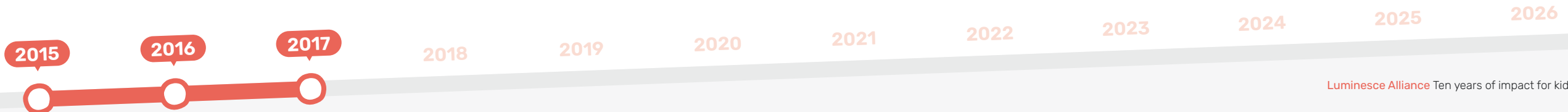
While individually rare, these diseases are collectively common. They affect around 400,000 Australian children, with many requiring lifetime care. Beyond the impacts on the health and quality of life for patients, rare diseases have a huge impact on the health system, families, communities and society.

2016-2017 Building the collaboration

Paediatrio’s strength was to be collaboration – between competitive research institutes and clinical campuses, between different research teams, clinicians and scientists, and people from a wide range of disciplines.

At first, it seemed like an impossible task. But we created multiple ways of bringing people together to discuss common issues, share resources and discover new opportunities.

Collaboration started to permeate through all levels of our member organisations. Before long, we had created something that was greater than the sum of its individual parts – Australia’s most ground-breaking, innovative and translational paediatric research hub that was set to change children’s health around the world.



2018 – The research takes off

Paediatrico received significant NSW Government funding of \$25 million to advance precision medicine research and treatment for children.

Our research community wanted the partnership to extend beyond the original trio of medical research institutes to formally include UNSW Sydney and the University of Sydney, which joined in 2018.

Data revolutionises paediatric cancer treatment



- Using the power of computers to find the genetic cause of Jack's brain tumour gave him the chance of a long and healthy life.

Jack was nine when he developed a large brain tumour. He had surgery but it came back in 2018; he was bedbound, going blind and in constant pain.

He was enrolled on the Zero Childhood Cancer Program (ZERO), established by Prof Michelle Haber AM¹ to combine medicine, technology and research to provide personalised medicine for children and young people with cancer.

Jack's tumour was analysed by the **computational biology data team** established by Luminesce Alliance. Computational biology uses massive super computers to compare data from the patient's genome and other biological measurements with that of healthy children and other children who have had similar cancers before.

Prof Mark Cowley² and his computational biology data team identified a genetic mutation called BRAF V600E that was driving Jack's cancer growth. There was a drug to target it, and Jack quickly improved. Now 19, he is happy, healthy and full of life.

The team is now Australia's largest computational biology group solely focused on paediatric cancer. ZERO has expanded and is now available to all children with cancer (0–18 years) and to young people aged 19 to 25 with paediatric type cancers.

Data analysis has been adapted to support more than 1,000 children every year to access the comprehensive precision medicine platform. The genetic basis of disease is identified in more than 90% of cases.

- Jack and his family returned to thank our researchers in 2026. They brought a trophy inscribed with the words: "With sincere thanks to Prof Mark Cowley and the team at Zero Childhood Cancer. For identifying the BRAF V600E mutation and helping guide the treatment that changed Jack's life."



¹ Professor Michelle Haber AM, Co-Group Leader, Children's Cancer Institute, Conjoint Professor, Faculty of Medicine and Health, UNSW Sydney.

² Professor Prof Mark Cowley, Deputy Director, Enabling Platforms and Collaboration and Computational Biology Group Leader, Children's Cancer Institute; Conjoint Professor, Faculty of Medicine and Health, UNSW Sydney.



2019 – Luminesce Alliance establishes the Centre for Paediatric Precision Medicine

In 2019, Paediatric rebranded as Luminesce Alliance. The new identity reflected our ambition to shine a brighter light on paediatric research and accelerate better health outcomes for children through a pioneering research partnership.

Research started with the Centre for Paediatric Precision Medicine program, creating a groundbreaking paediatric translational research hub delivering twenty-first century precision medicine for the families of NSW.

What is precision medicine?

Precision medicine is a personalised approach to treatment and prevention that uses genomic, lifestyle and environmental data to guide clinical decisions and improve outcomes.

It relies on analysis of a patient's genome – the 6 billion letter code that makes us human – to discover the mutation or mutations that cause disease. Identifying the genetic cause of a child's disease enables management tailored to their unique circumstances and that of their family.

Manufacturing viral vectors to use in gene therapy trials

Gene therapy treats genetic conditions by replacing or editing a faulty gene or introducing a new gene. It's shown stunning success in curing some genetic diseases, often with just one treatment.

Gene therapy is delivered using a modified virus called a viral vector. With hundreds of gene therapy clinical trials underway around the world, in 2018 global demand for clinically approved (cGMP) viral vectors far exceeded supply.

Luminesce Alliance provided seed funding to Prof Ian Alexander³ and his team to **establish a facility** to manufacture research-grade viral vectors for use in NSW.

With further \$134.5 million in NSW Government funding, this became the Viral Vector Manufacturing Facility at Westmead that opened in 2025. It was the first commercial manufacturing facility of its kind in Australia, uniquely integrated in the largest health, education, research and innovation precinct in the country.



- Our investment in viral vector manufacturing has led to a new facility that will secure supply for Australian researchers and give patients access to groundbreaking therapies.

³ Prof Ian Alexander, Head, Gene Therapy Research Unit, CMRI; Professor of Paediatrics and Molecular Medicine, Faculty of Medicine and Health, University of Sydney.



- Newborn screening for SMA has become critical for early detection of a devastating disease so that the best outcomes can be achieved.

Screening all newborn babies for Spinal Muscular Atrophy

Spinal Muscular Atrophy (SMA) is a devastating disease. It quickly paralyses babies, who lose the ability to move, feed and ultimately breathe while their brain remains intact. SMA affects one in 10,000 births and was once the leading genetic cause of infant death.

Luckily a new gene therapy revolutionised the outlook for SMA – but babies needed to be identified before they developed symptoms.

The NSW Government funded Luminesce Alliance to run a **two-year pilot** of newborn screening for SMA, using a molecular test developed from across both children's hospitals and the associated universities.

The program, led by Prof Michelle Farrar⁴ and A/Prof Veronica Wiley⁵, screened over 100,000 babies and the health economics analysis demonstrated it was effective for the early identification and treatment of SMA. This evidence led to the introduction of newborn SMA screening for all babies born in Australia.

⁴ Prof Michelle Farrar, Paediatric Neurologist, Sydney Children's Hospital and Discipline of Paediatrics, School of Clinical Medicine, UNSW Medicine and Health, UNSW Sydney.

⁵ A/Prof Veronica Wiley, Director/Principal Scientist, NSW Newborn Screening Program, The Sydney Children's Hospitals Network.

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2020 – Introducing precision medicine to the health system

We combined the diverse experience and expertise of our five powerhouse partners to focus on improving the prediction, detection and treatment of a variety of childhood diseases and conditions.

We made sure that clinicians drove the research agenda, so that researchers studied real-world problems and their discoveries could be quickly brought to the wards.

Paediatric rare disease predisposition screening

Working closely with clinicians and families, we established **Gene2Care** and integrated the GeneAdd screening platform into clinical care, helping to improve the diagnostic odyssey for children with rare genetic diseases. This work has now laid the foundation for earlier diagnosis, coordinated specialist care, stronger family support, and greater access to research opportunities across NSW.

Cystic fibrosis precision medicine

We contributed funding to the Southern Hemisphere's first **stem cell-based precision medicine platform for cystic fibrosis**, enabling clinicians to predict which therapies are most likely to benefit individual patients. This pioneering capability is now helping deliver more personalised treatment, optimise outcomes, and create new options for children with rare mutations.

The INFORM2 trial – the first of many advanced therapeutic clinical trials for NSW

The **INFORM2 Trial** is an important international trial into whether combining treatment with the drugs nivolumab and entinostat is safe and effective for children and adolescents with high-risk cancer. Nivolumab is an immunotherapy drug that stops cancer cells from hiding from the body's immune system, and entinostat alters the structure of genes in cancer cells.

With support from the NSW Government, Luminesce Alliance funding meant Australia was the first country in the world to open the trial and treat the first patient in 2020. INFORM2 is led by Prof David Ziegler⁶ and A/Prof Vanessa Tyrrell⁷.

The trial successfully showed that the treatment is safe and that both drugs can be administered together to children as young as two years of age. Exciting early data suggests that the treatment also has potent anti-cancer activity, with some children showing remarkable responses. The efficacy of the treatment, and understanding which patients are most likely to respond, is currently being evaluated.

With our support, NSW has grown to be a national leader in paediatric clinical trials. Supporting trials in Australia means more state-of-the-art treatments are available to children here, reducing expense and emotional burden for families needing to travel abroad to participate. We are currently working with our international collaborators on the next INFORM trial.

⁶ Prof David Ziegler, Paediatric Haematologist Oncologist, Kids Cancer Centre, Sydney Children's Hospital, Group Leader, Brain Tumour Group, Children's Cancer Institute, Conjoint Professor Faculty of Medicine, UNSW Sydney.

⁷ A/Prof Vanessa Tyrrell, Program Director, Zero Childhood Cancer, Theme Head Clinical Translation, Children's Cancer Institute.



One of world's first gene therapy treatments restores vision for children with inherited retinal disease

In 2020, Luxturna® became the world's first approved gene replacement therapy for an inherited blinding eye condition and one of the first gene replacements for any human disease.

Used to treat inherited retinal disease, Luxturna® is injected under the retina to carry a functioning RPE65 gene to replace the faulty one. It stops the ongoing progression of the disease and helps improve vision for children who have already developed symptoms.

⁸ Prof Robyn Jamieson, Head of Eye Genetics Research Unit CMRI, Head Eye Genetics Clinic at The Children's Hospital Westmead, Professor of Genomic Medicine, University of Sydney.

- Seeing stars for the first time: Rylee and Saman's vision was restored through the world's first gene therapy.

The first patients to receive Luxturna were 17-year-old Rylee and 15-year-old Saman, who were both diagnosed with a severe form of inherited retinal disease in their first year of life. After their treatment with Luxturna®, their vision improved and they could walk without a cane.

The therapy was delivered as part of Ocular Gene and Cell Therapies Australia, a collaboration led by Prof Robyn Jamieson⁸ involving SCHN, CMRI and Save Sight Institute, University of Sydney. Luminesce Alliance helped make the case for the treatment providing **health economic evidence** that showed significant benefits in terms of education, better employment opportunities, fewer health related costs and psychosocial impacts.

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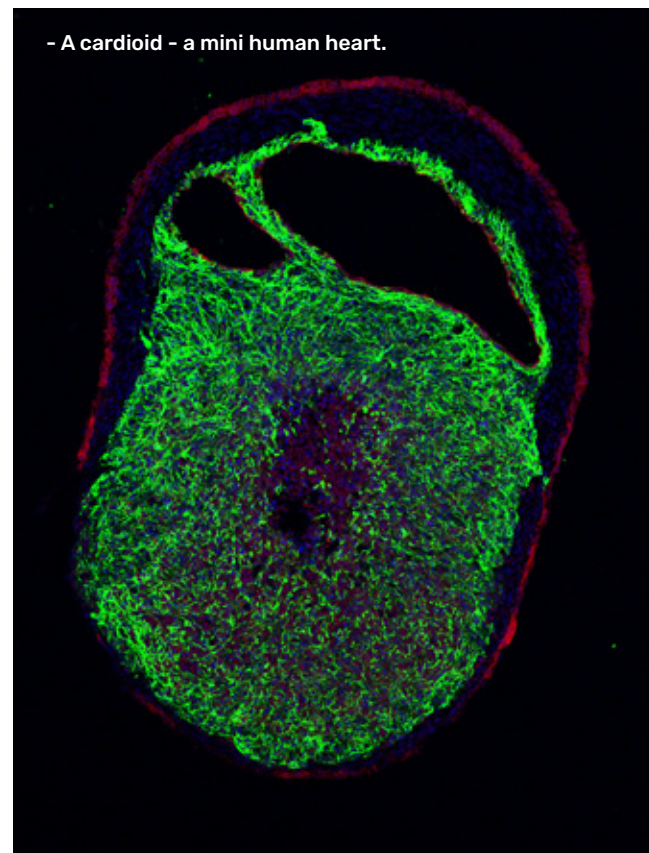
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2021 – Building NSW’s capacity and capability

By 2021, we had shown it was possible to deliver precision medicine in the health system. We had continued world-leading research and fast-tracked translation to clinical practice.

A key focus was to build the capacity of the health workforce to deliver precision medicine. We expanded advanced therapeutics capability across SCHN, built Australia’s largest paediatric computational biology team, and grew expertise in health economics and implementation, functional genomics and psychosocial research. We also partnered with the NSW Department of Education to support growth of the STEM workforce.

- A cardioid - a mini human heart.



Our incredible human mini-organs

In 2019, Children’s Medical Research Institute (CMRI) opened Australia’s first Stem Cell and Organoid Facility, giving researchers across NSW access to stem cell technology for the first time at scale. Seven years on, the facility continues to enable new discoveries and to accelerate the development of gene and stem cell therapies for children.

A/Prof Anai Gonzalez Cordero⁹ at CMRI led a program to create organoids (miniaturised organs grown in the laboratory from stem cells) that researchers use to understand disease progress and test potential treatments.

The team uses children’s own blood or skin to generate induced pluripotent stem cells (iPSCs). The iPSCs are then be changed into any type of cell in the body, including eye, brain, kidney and liver tissue.

One of the remarkable organs offered by the Facility includes **cardioids**, beating heart tissue available since 2021 for researchers to study heart diseases and potential treatments.

To date, the Stem Cell & Organoid Facility has produced more than 90 iPSC lines and numerous types of stem cell-derived organoids, significantly boosting research and leading to new understanding and therapies for children with a range of rare diseases.

A/Prof Gonzalez Cordero also leads the Stem Cell Medicine Unit, which is developing pioneering next-generation organoids – complex organoids with vascular and immune systems to transform disease modelling and therapeutic testing. This work is supported by the NSW NAT-Net to reduce animal testing and accelerate discovery. The team has also established crucial collaborations such as with the NSW Organoid Innovation Centre to enhance provision of this technology using automation.

⁹ A/Prof Gonzalez Cordero, Stem Cell Medicine Unit Head and Head of Stem Cell & Organoid Facility, Children’s Medical Research Institute Faculty of Medicine and Health, University of Sydney.



- Girls with Rett syndrome like Holly will benefit from our world-first research into disease biomarkers.

New hope for children with Rett syndrome

Holly was born healthy, but gradually started to develop symptoms of autism, cerebral palsy, Parkinson’s disease, epilepsy and seizures and anxiety disorder. After years of tests, her parents were given a terrible diagnosis: Holly had Rett syndrome.

Rett syndrome is a devastating neurodevelopmental disorder mainly affecting girls. It’s caused by random mutations in the MECP2 gene. There’s no treatment, and most children with Rett syndrome do not survive to adulthood.

With funding from Luminesce Alliance, Prof Wendy Gold¹⁰ and her team carried out **work that gained global attention** when they discovered two biomarkers in patients’ blood. The biomarkers can be used to test whether a child has Rett syndrome, and provide a reliable measure of whether treatment is working.

Now 12, Holly continues to face significant health challenges. Australian and international research into gene therapy offers the only hope of a cure, says Holly’s father Brett, the founder of **Rett Research Australia**.

‘Wendy has done some truly extraordinary work. Now we’re eagerly awaiting approval to run a Rett syndrome gene therapy clinical trial in Australia so that we can finally get a treatment for Holly.’ - Brett

2022 – NSW is on the world map

The work supported by Luminesce Alliance started gaining global attention. For example, a paper on **novel RNA diagnostic testing for rare genetic diseases** became the most read paper in Genetics in Medicine, the official journal of The American College of Medical Genetics and Genomics.

Our researchers were in demand across the world to share their expertise, including through an international data sharing framework formed by ZERO and in global collaborations via our psychosocial research program.

¹⁰ Prof Wendy Gold, Head, Molecular Neurobiology Research Lab, Kids Research, The Children’s Hospital at Westmead; Professor, School of Medical Sciences, Faculty of Medicine and Health, The University of Sydney.

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- Health economics and implementation research has accelerated the uptake of precision medicine into routine clinical care across the health system.

Health economics and implementation research

Our targeted investment addressed a critical workforce gap in health systems, implementation and economics – research areas critical to scaling precision medicine and demonstrating its value.

The Health Systems Implementation and Economics Enabling Platform allowed us to answer key questions around how much precision medicine would cost the health system, how far it should be expanded into routine care, and how to translate research into practice equitably.

Embedding implementation, health economics and evaluation experts from the outset strengthens the translation of innovations into routine care, improving their uptake, impact and long-term sustainability.

For example, Luminesce Alliance funded the **evaluation of the Providing Enhanced Access to Health Services (PEACH) project**, a three-year program to improve equity in health services for children and young people from priority populations. The evaluation was led by Professors Raghu Lingam¹¹ and Karen Zwi¹² and helped secure funding to expand this vital work.

¹¹ Prof Raghu Lingam, Professor, Paediatric Population and Health Services Research, Faculty of Medicine and Health, UNSW Sydney; Consultant Paediatrician, Sydney Children's Hospitals Network.

¹² Prof Karen Zwi, Community Paediatrician, Community Child Health, Sydney Children's Hospitals Network, Conjoint Professor, Faculty of Medicine and Health, UNSW Sydney

2023 – Our Enabling Platforms

Having demonstrated the feasibility of precision medicine through the Centre for Paediatric Precision Medicine, Luminesce Alliance received \$20 million in further NSW Government funding to establish the Enabling Platforms Program 2023–2027.

The aim was to create the infrastructure NSW needed to innovate in paediatric medicine and introduce new discoveries to the health system.

What are the Enabling Platforms?

The Luminesce Alliance Enabling Platforms are functional specialisations that cross disease areas and organisations to accelerate collaborative research, clinical translation and health impact.



Functional Genomics Enabling Platform

Identifying disease-causing genes and accelerating new treatments for children.



Functional Genomics Pipeline

Translating genetic discoveries into targeted therapies for children.



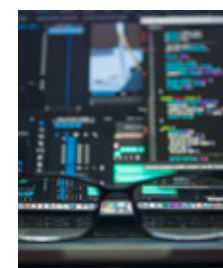
Translational Tumour Biology

Understanding cancer-driving genetic changes to develop more effective, less toxic treatment.



Data Enabling Platform

Harnessing big data to improve diagnosis, treatment and outcomes.



KidsLink

Linking health data to identify disease drivers, evaluate treatments, and guide care.



Advanced Clinical Data Analytics

Transforming complex health data into better treatments, prevention strategies, and clinical outcomes.



Genome & Transcriptome Analytics

Enabling precision medicine through the analysis of genomic, clinical and lifestyle data.



Precision Therapy Enabling Platform

Delivering new drugs and novel medical technologies that will support early phase clinical trials.



Kids Advanced Therapeutics

Advancing curative therapies for rare genetic diseases through cutting-edge genetic medicines.



THINK Drug Discovery

Taking drug research from bench to bedside for children with cancer, rare genetic diseases, and neurodevelopment diseases.



Psychosocial Enabling Platform



Developing world-leading best practice for psychological, emotional, social, and educational support of patients and their caregivers.



Health Systems Implementation and Economics Enabling Platform



Translating research discoveries into new models of care through **implementation science and health economics research**.

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- Sarah Thompson, a Registered Nurse who has become highly trained in advanced therapeutics clinical trials at Sydney Children's Hospitals Network.

Building workforce capability and capacity

A key focus of Luminesce Alliance has always been to strengthen Australia's paediatric precision medicine workforce. Since the beginning, we have created STEM jobs and established critical training pathways for researchers and clinicians.

We've done this by facilitating clinicians and researchers to drive the research agenda together. We've also provided funding for infrastructure that has attracted and retained some of the world's best and brightest to work in NSW.

We've increased workforce capacity in health economics, data computational engineering and psychosocial medicine. We've attracted statisticians, scientists, clinicians and geneticists to work in NSW. And we've supported the workforce on regulatory ethics and governance, program and trial management, and consumer engagement.

Those who have benefited include allied health practitioners, nurses, Aboriginal and Torres Strait Islander staff, early career researchers and students.

2024 – Driving innovative research

The Enabling Platforms meant we could build a unique paediatric precision medicine ecosystem for NSW.

We provided cost-effective access to scientific resources and infrastructure for researchers across disciplines and sites. This catalysed cross-sector collaboration and fostered innovative research and blue-sky thinking that would have otherwise been difficult to fund.

Our 2024 Conference united researchers, clinicians, government, industry and families to advance paediatric research. The conference highlighted the experiences of families and their critical role in shaping discoveries that improve outcomes for children. Our **2026 Conference** will showcase cutting-edge research transforming patient care and outcomes, and amplify the voices of patients, families and communities.

A cutting-edge technique to edit human RNA

CRISPR (clustered regularly interspaced short palindromic repeats) is a Nobel Prize-winning technology that enables scientists to precisely edit the genetic material of living organisms.

Building on the promise of CRISPR technology, Prof Paul Ekert's¹³ team at the Children's Cancer Institute is pioneering the use of **CRISPR-Cas13**, which edits RNA rather than DNA. This approach offers a potential way to address genetic variations identified through the ZERO Childhood Cancer Program without making permanent changes to the genome.

The team has demonstrated a way of referring promising candidates to early-stage development of therapies. This has established an accelerated pathway from research involving patient tumour samples into potential new treatments.

By expanding access to CRISPR-Cas13 technology and expertise across NSW's health and research ecosystem, this work is laying the foundation for safer, more precise therapies for children with cancer and rare diseases.

Together, these advances have the potential to enable earlier diagnoses, more effective treatments, and greater access to clinical trials – improving outcomes for children today and opening new possibilities for patients of all ages in the future.



- Luminesce Alliance Conference 2024: Prof Wendy Gold, The Children's Hospital at Westmead, The University of Sydney, and Emma Maly, Parent and Patient Advocate (L to R)

¹³ Prof Paul Ekert, Deputy Director, Research Themes; Translational Tumour Biology Group Leader, Children's Cancer Institute; Conjoint Professor, Faculty of Medicine and Health, UNSW Sydney.

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2025 – Achieving impact

By 2025, the impact of Luminesce Alliance could be measured not only in research milestones and growing collaborations, but in the lives of children and families. Across Australia and beyond, discoveries were moving from the laboratory to the clinic, opening new possibilities for diagnosis, treatment and care.

Our work earned recognition through national and state awards, yet our greatest success was seeing families benefit from world-leading research and innovative therapies that were changing lives.

Our awards

The Transcriptome Analytics Project Team (part of Luminesce Alliance Data Enabling Platform) were Highly Commended in the prestigious Research Australia Health & Medical Research Awards - Digital and Data Health Innovation category.

Providing Enhanced Access to Health Services (PEACH) won the Sydney Children's Hospitals Network Quality and Innovation Award for partnering with consumers 2024.

Prof Wendy Gold was nominated for the 2025 AccessCR Consumer Partnerships in Research Awards, recognising her leadership in embedding lived experience into research. Her outstanding contribution to working with patients, families and consumers was recognised through her selection as a People's Choice Finalist.

Kids Advanced Therapeutics team at The Sydney Children's Hospitals Network were Finalists in the 2025 NSW Health Awards, Health Research Category.

Building an advanced therapeutics pipeline

The THERapeutic INnovations for Kids (THINK) program was set up as the only group in Australia focused on developing new drugs specially for children with cancer.

Established by Prof Ian Street¹⁴ and now led by A/Prof Antoine De Weck¹⁵, **THINK** brings together a group of data analysts, computational experts, biologists, medicinal chemists and pharmacologists to create an end-to-end drug discovery pipeline.

First it conducts genomic sequencing to identifying possible drug targets using computational biology, understands their biological function, tests them with hundreds of thousands of molecules to see if any will stop the process, and then develops successful compounds into pharmaceuticals to be tested on patients in clinical trials.

With support from Luminesce Alliance, THINK identified a possible treatment target for neuroblastoma, a protein called METTL5 that is linked strongly to poor survival.

¹⁴ Prof Ian Street, Director, THERapeutic INnovations for Kids, Children's Cancer Institute; Conjoint Professor, Faculty of Medicine and Health, UNSW Sydney.

¹⁵ A/Prof Antoine de Weck, Group Leader, Computational Drug Discovery Biology Group, Children's Cancer Institute; Conjoint Associate Professor, Faculty of Medicine and Health, UNSW Sydney.



- Children's Cancer Institute's Prof Mark Cowley and his team at the Research Australia Health & Medical Research Awards

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Bringing advanced therapeutics clinical trials to NSW

Taking part in an advanced therapeutic clinical trial is often the only way children with rare diseases can access potential treatment. Advanced therapeutics are treatments such as gene therapies, biologics and cell-based treatments.

But until recently, NSW was not as effective as some other countries in attracting clinical trials.

Luminesce Alliance initially funded teams across SCHN to identify and coordinate clinical trials. This initiative grew into the Kids Advanced Therapeutics (KAT) Enabling Platform, aiming to provide access to advanced therapeutics through early phase clinical trials and other treatment pathways.

Led by Dr Michelle Lorentzos¹⁶ and Ms Lani Attwood¹⁷, KAT supported researchers and health professionals from laboratory discovery through to delivery in the hospital, for example by providing start up services for trials, regulatory guidance, patient advocacy, and nurturing partnerships and collaboration.

The KAT team’s work has seen a 93% increase in the number of paediatric advanced therapeutics trials conducted in NSW and led to SCHN becoming the first Australian site accredited to deliver approved gene therapy for Duchenne muscular dystrophy. John was one of the first patients, and the treatment helped slow his disease.



- John is enrolled in a clinical trial made possible through the Kids Advanced Therapeutics program at The Sydney Children’s Hospitals Network, proudly supported by Luminesce Alliance and Sydney Children’s Hospitals Foundation.

Our innovation projects

To date we have allocated \$3.1 million to 11 innovation research projects. This funding helped researchers get the proof-of-concept they needed to apply for further funding, or gather evidence and data to translate precision medicine into clinical practice.

The amazing projects we’ve supported have led to innovation in cancer, Rett Syndrome, inherited retinal dystrophies and blindness, metabolic liver disease, accelerating a drug discovery pipeline to treat difficult childhood cancers, and establishing a RNA diagnostic service to provide answers for affected families.

Our current innovation projects include a **Phase 1 clinical trial** using targeted CAR T-cells to treat children with sarcoma, a project looking at reactivating the function of **genes to suppress tumours**, and evaluating novel **gene editing strategies** to treat genetic liver disease in children.

¹⁶ Prof Dr Michelle Lorentzos, Advanced Therapeutics Medical Lead, Sydney Children’s Hospitals Network; Clinical Lead, Neurogenetics Service, The Children’s Hospital at Westmead; Senior Clinical Lecturer, Faculty of Medicine and Health, The University of Sydney.

¹⁷ Ms Lani Attwood, Acting Director Research, Sydney Children’s Hospitals Network.

2026 – A powerhouse for research

In just three years, the Luminesce Alliance Enabling Platform Program has significantly expanded the reach and impact of precision medicine and delivered a strong return on the NSW Government’s investment.

We’ve leveraged an additional \$180.6 million in co-investment and competitive research funding – more than 9 times the 2023 NSW Government’s \$20 million investment.

We’ve continued to deliver high-impact translational research and positioned NSW as a global leader in paediatric precision medicine, generating enduring economic and health benefits for the state.

Advanced Clinical Data Analytics

With our support, the Advanced Clinical Data Analytics team has developed a platform that is unlocking the full value of health data to deliver better quality care. The platform is being used to predict how many emergency department admissions to expect on a given day, how many staff members need to be on duty, and the most promising clinical course of action for individual patients. This is laying the foundation for a learning health system that uses routine care data to improve outcomes, accelerate clinical trials, and drive innovation across NSW Health.

Advanced Genome and Transcriptome Analytics

We have supported the development of NSW’s first and Australia’s largest paediatric computational biology team, harnessing AI and machine learning to unlock the power of genomic data and deliver precision treatments tailored to each child. Through collaborations with partners including Microsoft, Equinix, NetApp, NCI, Australian BioCommons and We Discover, the team has established a nationally significant capability that is advancing precision medicine and improving outcomes for children.

Ensuring equity for all children

All our partners are focusing their attention on ensuring access and equity for children:

- Providing Enhanced Access to Health Services (PEACH) is expanding to embed equity principles across other local health districts.
- KAT researchers have highlighted the urgent need for equitable access to emerging therapies and clinical trials for all Australian children and adolescents.
- The Psychosocial Enabling Platform team is supporting 21 research projects with expertise in consumer engagement, equity, culturally and linguistically diverse populations and Aboriginal communities.
- Aboriginal health research training is being delivered to strengthen partnerships with communities, support Aboriginal leadership, and foster culturally responsive research.



- Research supported by Luminesce-Alliance has led the world in taking a psychosocial approach to precision medicine.

A psychosocial approach to precision medicine

With the rapid expansion of precision medicine, we needed to better understand the implications of genetic testing and advanced therapies for children, families and the health professionals who treat them.

As a first step, we funded one of the world's first studies exploring patient, parent and healthcare professionals' experiences of precision medicine trials and genetic testing for childhood cancer. Led by Dr Kate Hetherington¹⁸ and Prof Claire Wakefield¹⁹, the study generated new knowledge about hopes and challenges, led to the development of patient resources, and informed improvements to trial clinical processes.

The project helped establish NSW hospitals and research centres as world leaders in collaborative psychosocial research in childhood genetics, supporting new jobs and training for behavioural

scientists, psychologists and researchers. It enabled collaborations across the United States, Canada, the Netherlands, Belgium, Norway, Sweden, Finland and Germany, and a research network across five countries to improve psychosocial outcomes for children with cancer and rare diseases.

We have continued this work by establishing the Psychosocial Enabling Platform to support the embedding of psychosocial wellbeing and equity across paediatric research. The platform strengthens research quality, inclusivity and translation by providing expertise and resources in equity, cultural competence and bilingual support. This ensures research is responsive to families from culturally and linguistically diverse backgrounds, Aboriginal and Torres Strait Islander communities, people living with disability, and those in rural and remote areas.

¹⁸ Dr Kate Hetherington, Senior Lecturer and Clinical Psychologist, Behavioural Sciences Unit, UNSW School of Clinical Medicine, Discipline of Paediatrics and Child Health and Minderoo Children's Comprehensive Cancer Centre, Sydney Children's Hospital, Randwick.

¹⁹ Prof Claire Wakefield, Director of Research, Division of Quality of Life and Pediatric Palliative Care, Taube Faculty Scholar in Pediatric Palliative Care, Stanford University, School of Medicine.

Our next challenge

Despite the incredible medical advances of the last decade, children's access to new therapies remains limited, fragmented and inequitable in NSW.

Our clinicians and researchers have told us the key to improving outcomes for children and families is to better address equity.

That's why we're proposing IMPACT for Kids, a statewide infrastructure that will transform how clinical trials are delivered in NSW.

Our idea is to leverage NSW's investment in the Single Digital Patient Record, using the power of data and digital technology to embed clinical trials into routine care.

It's an evolution of everything we've done so far, and builds on our expertise in collaboration, working with consumers, data, psychosocial support, and clinical trials management.

Our aim is that NSW will be a leader in running trials that are more efficient, more effective and more inclusive of all children and families, no matter where they live or their social background.



2015

2016

2017

2018

2019

2020

2021

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2025

2026



Thank you

With the support of NSW Health, we have spent the past decade improving outcomes for children through the collaborative efforts of researchers, clinicians and families. Thank you for making this progress possible.

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LUMINESCE Alliance

Innovation for Children's Health



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