

# THINK:

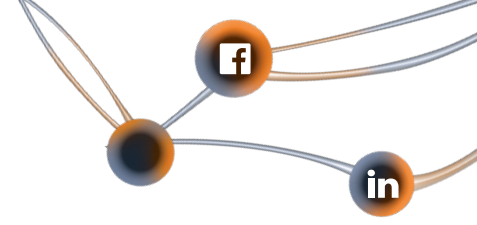
Developing a sustainable collaborative model for novel paediatric cancer drug discovery and commercialisation in Australia.



L-R Dr Jess Smith, Brandon Capital Partners; Dr Julia Warning, NSW Health; Prof David Ziegler, Sydney Children's Hospital Network and A/Prof Greg Arndt, Children's Cancer Institute, at the Luminesce Alliance Conference 2024 panel discussion







**T**here are numerous challenges associated with discovering and developing novel drugs for paediatric cancer, but Luminesce Alliance are working to overcome them by developing robust collaborative models with sustainable funding, allowing us to find new therapeutics for kids with cancer in Australia.

Around 1,000 children and adolescents are diagnosed with cancer each year in Australia.

Therapeutic INnovations for Kids (THINK) is a drug discovery and development pipeline for generating new therapies to better treat children with cancer. THINK is funded by Luminesce Alliance and delivered by Children's Cancer Institute, as part of Luminesce Alliance Enabling Platforms program.

'Drug discovery fascinates me,' says Prof Ian Street, Director of THINK.

'Even after 35 years in this field it still amazes me that we can design a 'targeted' molecule that, when given to a patient in tiny amounts, will seek out its target and halt its cancer-causing activity.

'However, for many reasons completely unrelated to our ability to produce these targeted therapies, progress in drug development for children with cancer has been slow, and many parents and families discover that the treatment options available for children today have changed little to those offered 30 years ago.'

## OVERCOMING KEY CHALLENGES

Historically, biotech and pharmaceutical companies that have successfully brought new therapies to adult cancer patients have been reluctant to engage in paediatric drug development, dissuaded by the prospect of low commercial returns and complex regulatory hurdles.

A review of FDA approvals between 2012-2021 revealed that only 39/341 (11.4%) approved oncology indications were for paediatric cancers.

This lack of commercial investment means that the true economic cost of children's cancer is assumed by the public health system (government and the taxpayer) and, because of the lack of modern and more effective and less toxic treatments, these costs can continue throughout life.

Traditionally, academia has led discovery biology. Our understanding of the genomic landscape in paediatric cancers is described by academic consortia globally that have identified the key underlying biological drivers and novel drug targets.

However, this increase in knowledge has not yet translated to an increase in new drug approvals. In response,

regulatory bodies in the United States and Europe have enacted new policies to encourage drug development for paediatric cancers.

Yet, despite these commercial incentives, it is academic, government, philanthropic and not-for-profit groups that are driving the development of novel therapeutics.

Drug discovery and development is expensive and time consuming, and only through collaboration will these non-commercial groups muster the funding and resources needed to change the therapy landscape in paediatric cancer.

Further, paediatric cancer drivers are often not addressable with conventional small molecule drugs. THINK is developing a novel approach known as Small Molecule Induced Reading Frame Shift (SMIRFS) which targets RNAs not proteins, turning 'undruggable' targets into 'druggable' targets.

## DEVELOPING A ROBUST AND COLLABORATIVE MODEL WITH LONG-TERM SUSTAINABLE FUNDING

Successful collaborative models have been formed in the past to discover, develop and fast track novel therapeutics for approval.

In Australia, one of the most successful collaborative models was the Cancer Therapeutics (CTx) consortium which generated eight licensed clinical drug candidates in adult cancer. Importantly, the commercialisation success from these collaborative models provides the financial return to fund future projects, thus creating a long-term sustainable environment for novel drug discovery and development.

THINK aims to replicate these robust and collaborative models to generate: a virtual pharmaceutical network consisting of infrastructure, academic researchers, drug discovery biologists and chemists, and a project portfolio, funded by government, philanthropy, venture capital, and pharmaceutical companies, with the goals of finding novel clinical drug candidates for *paediatric cancers first* and driving these toward clinical trials, commercialisation, and novel treatments in the clinic for kids with cancer.

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