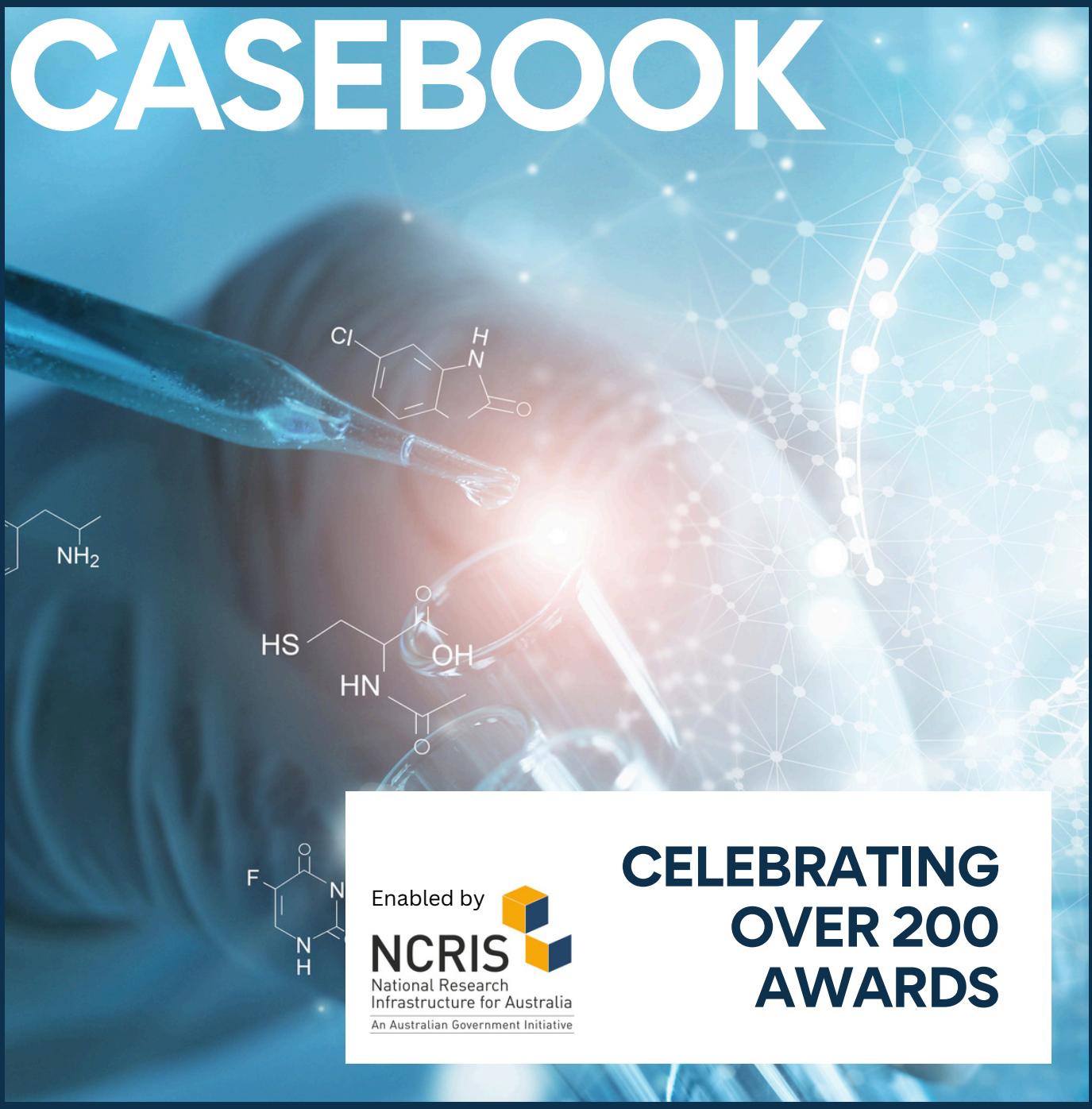
THERAPEUTIC INNOVATION AUSTRALIA

PIPELINE ACCELERATOR CASEBOOK



Enabled by NCRIS National Research Infrastructure for Australia
An Australian Government Initiative

CELEBRATING OVER 200 AWARDS

FOREWORD

DR STUART NEWMAN

Chief Executive Officer

When we opened the first round of the Pipeline Accelerator scheme in 2018, we had no idea what it would become. We had some discretionary funding, some inspiration from other schemes, and a vision.

The response was immediate. Now, seven years later, the Pipeline Accelerator is a fixture of Australia's therapeutic development landscape. From the beginning, we have always pitched the scheme as a win-win-win. In making an award:

- A researcher wins by having the cost to access excellent services significantly lowered.
- A TIA facility wins by securing a project that may not have happened but for a voucher award.
- TIA wins by supporting a project and potentially demonstrating a translational success.

This core principle has served us well, as we have expanded the scope and scale of the scheme, and also shared the framework and process with other NCRIS providers.

And now, over 200 awards and triple-wins later, we are taking the opportunity to reflect on TIA's mission and the critical role the Pipeline Accelerator scheme has played in translating Australian discoveries.

This casebook highlights the Pipeline Accelerator's achievements by hearing from the translational scientists themselves, and how the voucher scheme has helped their potential therapeutic move closer to reality.

It's also an opportunity to acknowledge and thank the researchers, facilities, and funders who have contributed to the scheme's success. And finally, we present a vision for the future of the scheme that will enable us to foster and accelerate medical breakthroughs for years to come.

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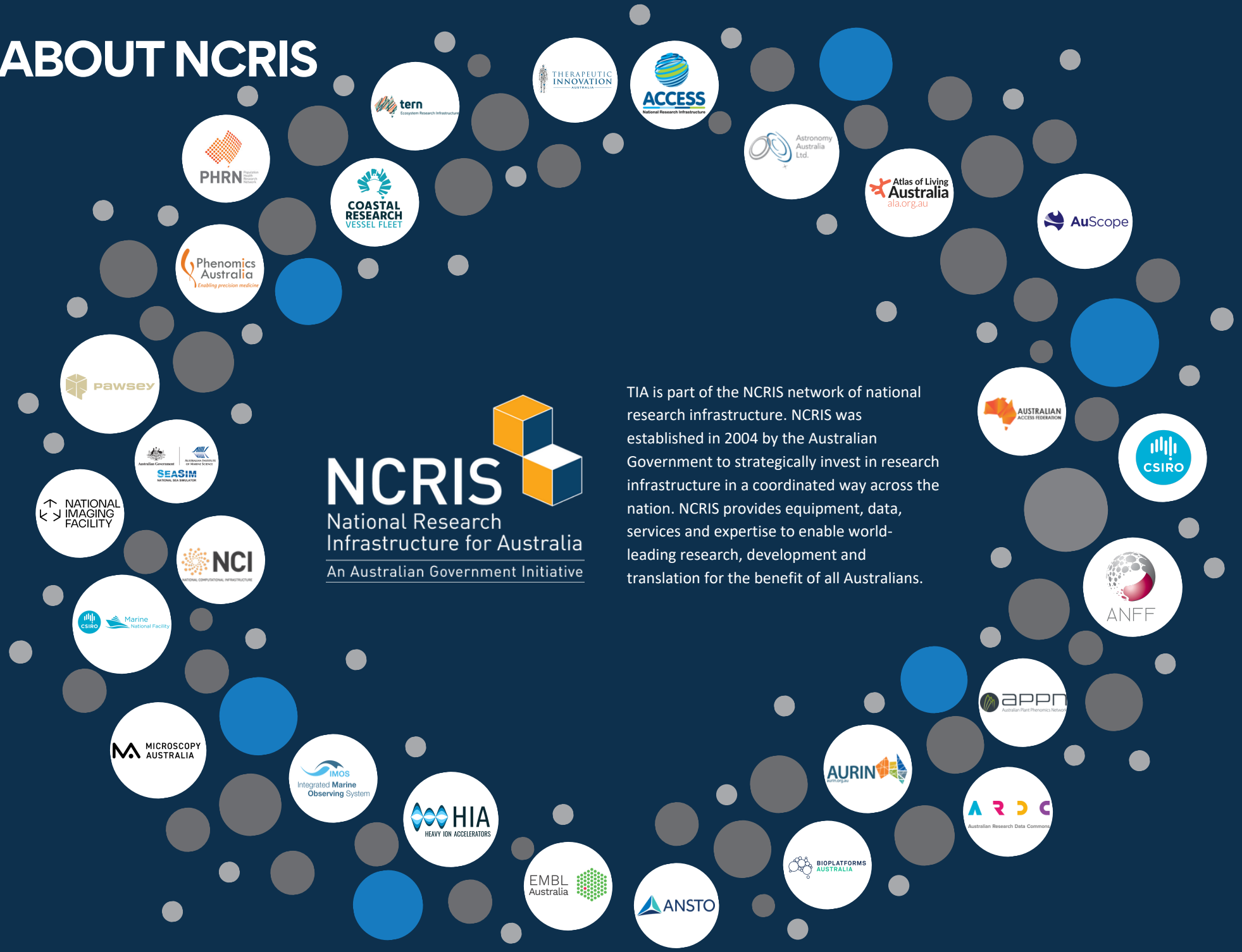
ABOUT NCRIS



NCRIS
National Research Infrastructure for Australia
An Australian Government Initiative

TIA is part of the NCRIS network of national research infrastructure. NCRIS was established in 2004 by the Australian Government to strategically invest in research infrastructure in a coordinated way across the nation. NCRIS provides equipment, data, services and expertise to enable world-leading research, development and translation for the benefit of all Australians.

ABOUT THE TIA NETWORK

TIA enables open and subsidised access to a broad range of expertise across various therapeutic types. Each area is supported by a dedicated broker/facilitator equipped with relevant know-how, who can facilitate access to relevant expertise and facilities.

SMALL MOLECULE THERAPEUTICS

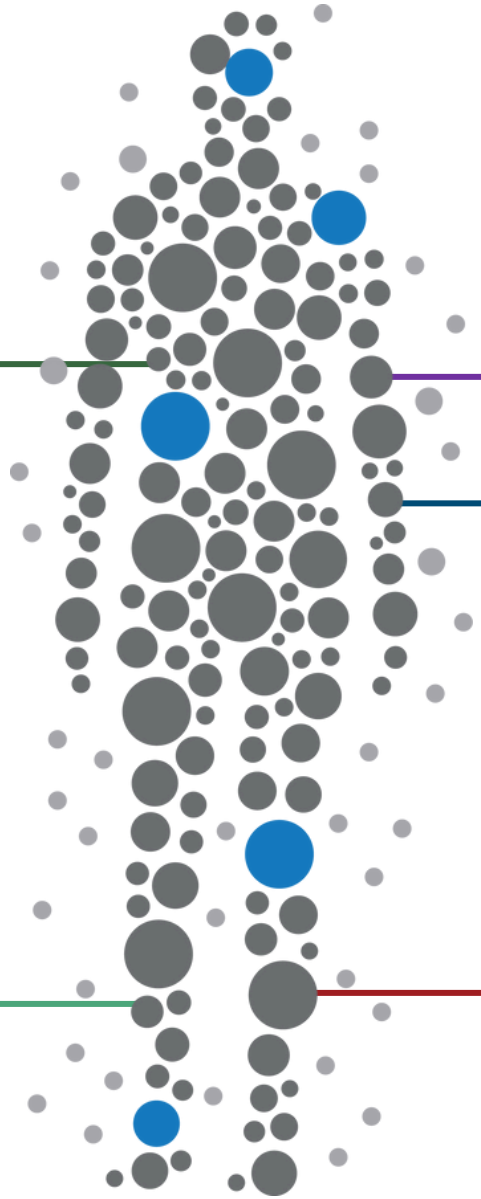
An established pipeline of complementary facilities that can translate projects from screening for hits and lead identification through to drug preclinical efficacy testing.

- Ab Initio Pharma
- Agilex Biosciences
- Aust. Translational Medicinal Chemistry Facility
- Centre for Drug Candidate Optimisation
- Centre for Integrated Preclinical Drug Development
- Compounds Australia
- CSIRO Biomedical Manufacturing
- Medicines Manufacturing Innovation Centre
- Preclinical Imaging Research Laboratory
- Qld Emory Drug Discovery Initiative

BIOLOGICS AND VACCINES

Broad expertise in development of novel biologic therapies, bridging the gap between research and clinical development.

- Biologics Innovation Facility
- CSIRO Biomedical Manufacturing
- National Biologics Facility
- Protein Expression Facility
- Recombinant Products Facility



RNA PRODUCTS

A collaborative network of RNA development and production facilities supporting researchers seeking to develop and translate novel therapeutics.

- BASE Facility
- mRNA Core
- RNA Innovation Foundry
- UNSW RNA Institute

HIGH THROUGHPUT SCREENING

A national network of compound library screening facilities focussed on discovery of small molecule pharmaceuticals.

- ACRF Drug Discovery Centre
- ANU Centre for Therapeutic Discovery
- Community for Open Antimicrobial Drug Discovery
- Griffith Discovery Biology
- IBG-Nathan Mass Spectrometry Facility
- Monash Fragment Platform
- National Drug Discovery Centre
- Stafford Fox Drug Discovery Facility
- Cell Function and Screening Facility
- Victorian Centre for Functional Genomics

CELL AND GENE THERAPIES

A network of facilities supporting development and manufacture of advanced therapies.

- Cell and Molecular Therapies
- Cell and Tissue Therapies WA
- Cell Therapies Pty Ltd
- Centre of Excellence in Cellular Immunotherapy
- Functional Genomics South Australia
- Q-Gen Cell Therapeutics
- Sydney Cell & Gene Therapy
- Vector and Genome Engineering Facility

THE PIPELINE ACCELERATOR JOURNEY

While Australia has a strong foundation in biomedical research, many promising therapeutic discoveries struggle to progress due to limited access to specialised infrastructure, expertise, and funding in the development stages between discovery and translation. This critical gap in Australia's translational research ecosystem is enduringlyknown as the "Valley of Death."

The Pipeline Accelerator was launched in 2018 to help bridge this gap by providing academic researchers and biotech companies with direct support to accelerate the development of novel therapies. This support is in the form of access vouchers of up to $50,000, which must be matched by cash co-investment. This represents a significant lowering of the initial cost to access state-of-the-art research infrastructure, ensuring that innovative therapeutic projects can advance beyond the discovery phase up to the readiness for clinical trials.

Eligibility criteria ensure that supported projects align with the scheme's goals, with a focus on advancing therapeutics within Technology Readiness Levels (TRL) 3-6 (see Figure 1, p.9). This includes preclinical and early clinical development across areas such as small molecule therapeutics, biologics and vaccines, cell and gene therapies, and more recently, RNA products. As a competitive scheme, applicants must demonstrate strong scientific merit, feasibility, and potential impact to an expert assessment committee. The requirement for matching funding demonstrates that a project has already attracted some support for its development.

By reducing financial and logistical barriers, the Pipeline Accelerator empowers academic researchers and small and medium-sized enterprises (SMEs) to generate high-quality preclinical and clinical data, de-risking their projects and enhancing their potential for commercialisation.

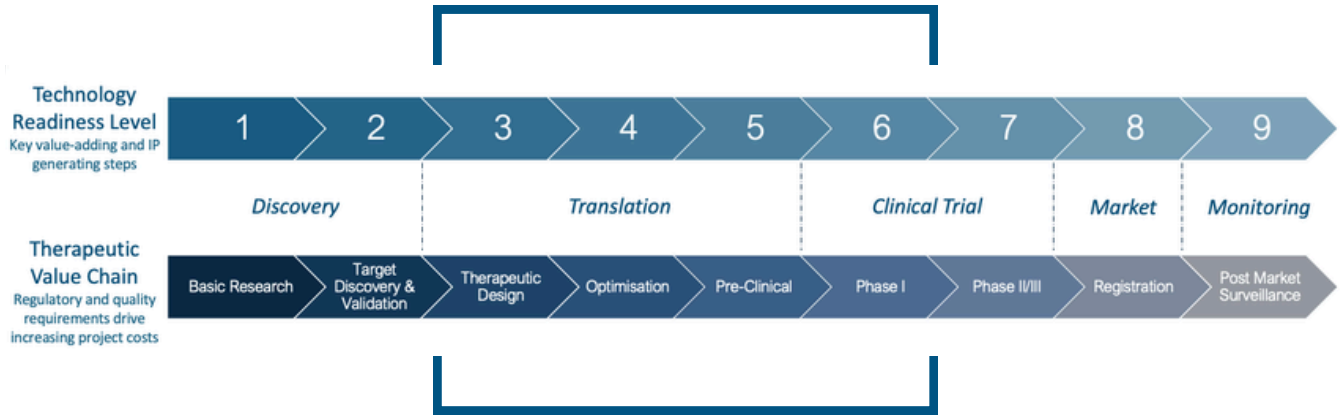


Figure 1: Technology readiness levels (TRL) as applied to a therapeutic value chain. TIA focuses on advancing therapeutic projects within TRL 3-6.

Because we assess project feasibility rather than researcher credentials, the Pipeline Accelerator is a great way to develop track records in translational research, particularly for female researchers and early/mid-career researchers (EMCRs - <10 years post-PhD excluding career breaks).

Building Momentum: The Pipeline Accelerator's Growth Over Time

Since its inception, the Pipeline Accelerator has expanded in scale and impact. TIA is now partnering with three other related NCRIS providers - Phenomics Australia, ANSTO's National Deuteration Facility (NDF) and Bioplatforms Australia (starting in 2025), to expand the range of translation expertise available for the discovery and translation of medical research. While initially offering access to a small number of facilities, the Pipeline Accelerator has evolved to support a wider range of therapeutic development stages, such as:

- Preclinical proof-of-concept studies
- Manufacturing process development
- Regulatory and clinical trial readiness

The Pipeline Accelerator has also adapted to emerging needs by integrating new technologies and expanding access to an increasingly diverse set of research facilities. It operates through a competitive funding round every six months, ensuring ongoing opportunities for researchers and companies to apply for subsidised support as they progress their therapeutic innovations.

In 2020, during the COVID-19 pandemic, TIA launched a round of the voucher-based scheme, designated to support national and international efforts to combat COVID-19. This included any aspect of the national or global response, including (but not limited to) high throughput screening (HTS), assay development, drug repurposing, diagnostic development, pre-clinical and clinical trial support, analytical validation and pilot-scale production.

In 2022, TIA partnered with the Australian Antimicrobial Resistance Network (AAMRNet), Australia's first industry-led and multi-stakeholder Antimicrobial Resistance Network, allocating an additional $200,000 specifically to support projects focused on developing novel treatments for drug-resistant infections.

In 2023, TIA introduced the Technical Feasibility Assessment (TFA) scheme for Cell & Gene Therapy (CGT) research projects. Valued at $10,000, these awards support the provision of advice on product development directly from TIA's group of CGT facilities to researchers looking to develop novel therapeutic products. The TFA awards provide researchers with much needed direction and expert advice for initiating translational research, building knowledge of the process and requirements and improving early-stage decisions.

The Pipeline Accelerator has also adapted to emerging needs in the sector, integrating new technologies and expanding access to an increasingly diverse set of research facilities.

To date, we have awarded 217 vouchers, representing a significant cumulative investment of $7.7M in Australia's therapeutic innovation pipeline.

Transforming Research: Outcomes That Matter

The impact of the Pipeline Accelerator is clear from the success stories of its recipients, including:

- Advancement to clinical trials, accelerating the development of new therapies for patients.
- New spin out companies, driving commercialisation and job creation in the biotech sector.
- Strategic partnerships, fostering collaboration between academia, industry, and government.
- A high rate of repeat voucher recipients, highlighting the scheme's ongoing value to researchers and biotech companies as they progress through the development pipeline.

Vouchers Awarded

217

Cumulative Investment

$7.7M

Success Rate

53%

2018
Total Awards - $340,000

Dipping a toe in the water.
The first pilot round of the Pipeline Accelerator scheme is launched, making 8 awards to access 6 facilities

2019
Total Awards - $330,000

The scheme begins in earnest
The Pipeline Accelerator scheme starts with separate rounds for our core facilities and also a network of high throughput screening facilities, providing a crucial early link in the drug discovery chain

2020
Total Awards - $1,535,000

Special COVID-19 Round.
TIA launches a rapid-response funding round to provide emergency support for COVID-19 projects on top of the regular rounds.

2021
Total Awards - $1,170,000

100 not out!
TIA reaches a century of voucher awards in the 7th round of awards

2022
Total Awards - $1,270,000

But is it feasible?
A new Technical Feasibility Assessment scheme is launched to provide early advice before researchers embark on costly manufacturing projects.

2023
Total Awards - $1,262,000

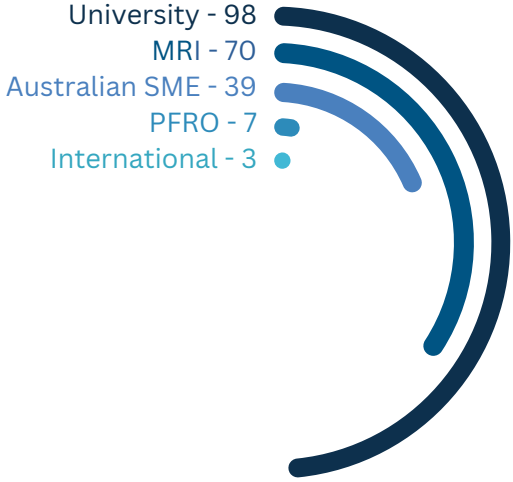
TIA partners with Phenomics Australia
Fellow NCRIS provider Phenomics Australia joins the scheme, the first step in expanding the model to other providers.

2024
Total Awards - $1,789,000

TIA partners with National Deuteration Facility
Another NCRIS provider, ANSTO's National Deuteration Facility, joins the scheme, increasing the range of accessible services.

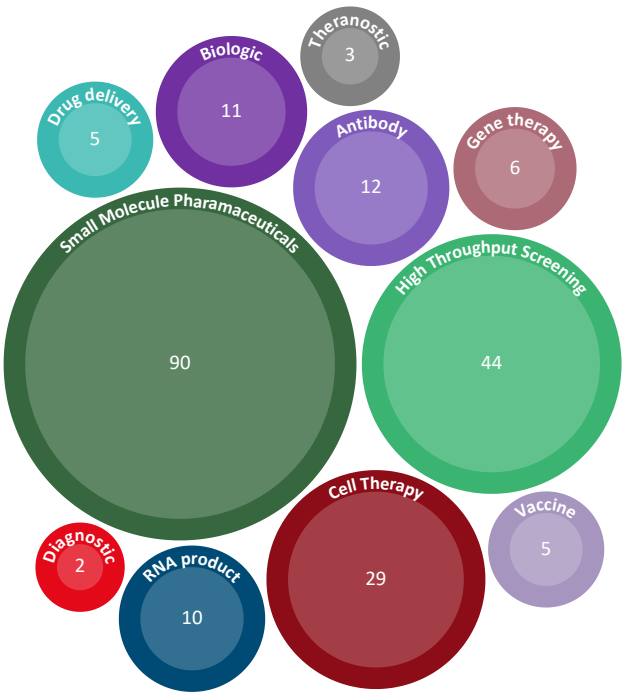
PIPELINE ACCELERATOR BY THE NUMBERS

Breakdown of recipients

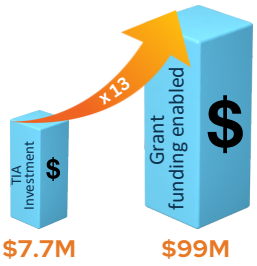


MRI - Medical Research Institute
SME - Small and Medium-sized Enterprise
PFRO - Public Funded Research Organisation

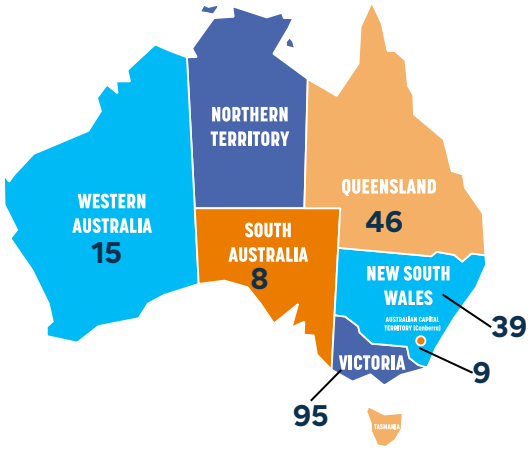
Projects by type



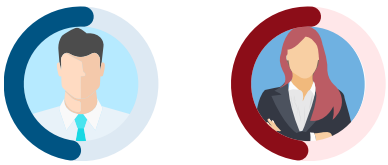
Investment leverage



Geographic distribution



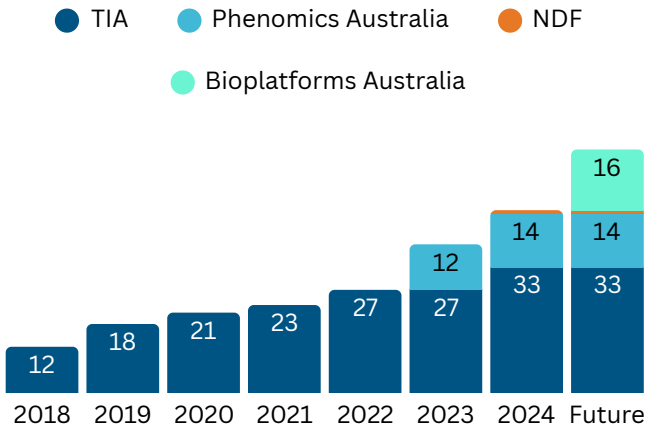
Success rate by gender



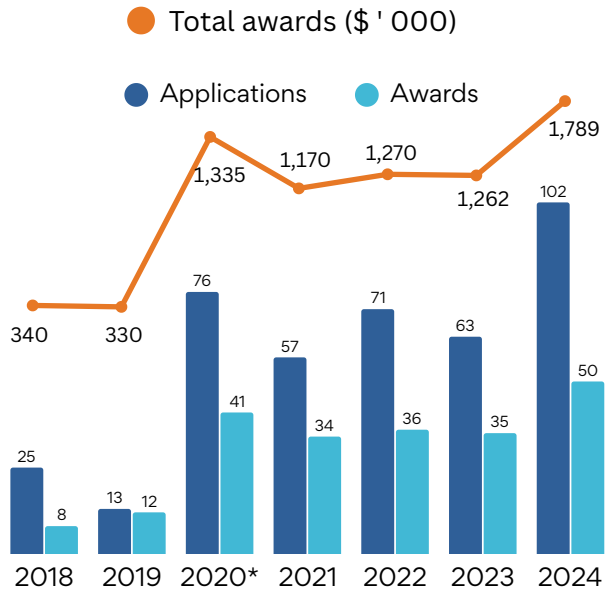
53%

54%

Number of facilities accessible



Awards and applications



*TIA operated an additional COVID-19 support round in 2020

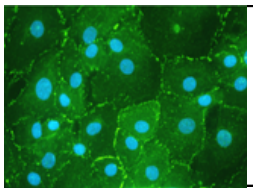
Impact in numbers

A 2025 survey of 76 voucher awardees reported the following:

- **6** new businesses created
- **15** invention disclosure or IP rights
- **15** increased Industry Engagement
- **28** new collaborations
- **11** progressed towards clinical trial
- **13** proof-of-concepts demonstrated
- **26** grants or further investments
- **13** publications

TRANSLATIONAL IMPACTS

CELL AND GENE THERAPIES

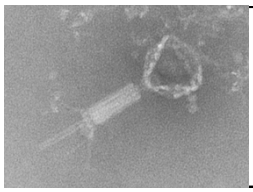


Revolutionising Corneal Transplantation: Advancing Clinical-Grade Cell Injection Therapy

Cell and Tissue Therapies WA

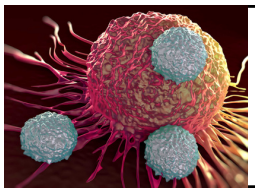
Advancing Cardiac Regenerative Medicine Through GMP-Ready Stem Cell Therapy

Q-Gen Cell Therapeutics,
Sydney Cell and Gene Therapy



A New Frontier in Fighting Superbugs: Bacteriophage Therapy in Australia

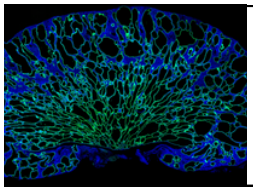
Cell and Tissue Therapies WA



Expanding Access to "Off-the-Shelf" T Cell Therapy for Cancer and Transplant Patients

Q-Gen Cell Therapeutics

SMALL MOLECULE THERAPEUTICS



Advancing Polycystic Kidney Disease Treatments: The Rise of xCystence Bio

Aust. Translational Med Chem Facility,
Cent. Drug Candidate Optimisation

A New Approach to Halting Pulmonary Hypertension Progression

Aust. Translational Med Chem Facility,
Cent. Drug Candidate Optimisation,
NDDC, Compounds Australia

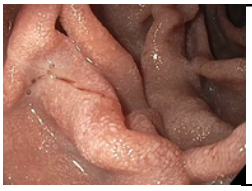
Development of METTL5 Inhibitors for the Treatment of Solid Tumours in Children and Adults

Aust. Translational Med Chem Facility,
Cent. Drug Candidate Optimisation

From Molecule to Medicine: Advancing Cancer Immunotherapy

Cent. Drug Candidate Optimisation

BIOLOGICS AND VACCINES

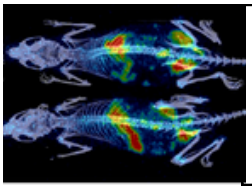


Developing Hookworm-Derived Biologics for Inflammatory Bowel Disease

National Biologics Facility

Advancing Oral Insulin with Nanotechnology

CSIRO Biomedical Manufacturing

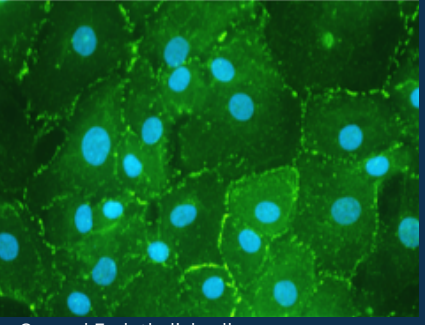


Advancing Imaging for Ovarian and Bladder Cancer

National Biologics Facility, CSIRO Biomedical Manufacturing.

Enabling Translation of an Immunotherapy against *Porphyromonas gingivalis*

National Biologics Facility



Corneal Endothelial cells
Image: Cell and Tissue Therapies WA

REVOLUTIONISING CORNEAL TRANSPLANTATION: ADVANCING CLINICAL-GRADE CELL INJECTION THERAPY

CELL AND GENE THERAPIES

Corneal endothelial cell failure is a leading cause of blindness worldwide, yet donor corneas for transplantation remain in critically short supply. **Dr Evan Wong**, Ophthalmologist at the Lions Eye Institute, is developing a corneal cell injection therapy, offering a minimally-invasive approach to treating corneal blindness.

TIA has supported this project by awarding both a Technical Feasibility Assessment (TFA) and a Pipeline Accelerator award. The TFA provided expert guidance from Cell and Tissue Therapies WA (CTTWA) at the Royal Perth Hospital on product development requirements for Dr Wong's clinical-grade, good manufacturing practice (GMP)-compliant cultured corneal endothelial cell (CEC) therapy. The TFA enabled progression to a Pipeline Accelerator voucher, enabling CTTWA to establish a GMP-compliant culture protocol and expand corneal endothelial cells from donor tissue. The team has developed a manufacturing protocol for enough cells for approximately 130 therapeutic doses from a single donor.

This technical transfer to a GMP facility is a crucial step in clinical translation and the team is now looking to proceed to Australia's first clinical trial of cultured CEC injection therapy, positioning Australia as a pioneer amongst global leaders in the field.

Unlike traditional corneal transplants, which require a 1:1 donor-to-recipient ratio and complex surgical procedures, CEC injection therapy offers a scalable, minimally invasive alternative. It has the potential to improve access to treatment in rural hospitals, enhance donor-to-recipient efficiency, and drive future commercialization and export through advanced manufacturing and logistics.

This initiative represents a unique collaboration between Singapore Eye Research Institute, Lions Eye Institute and Eye Bank, and CTTWA, bringing together expertise in corneal donor material, transplantation, and clinical-grade cell therapy manufacture. As the team prepares for a Phase I clinical trial, efforts will focus on refining cell preservation techniques and addressing logistical challenges for broader distribution. This ground-breaking work could revolutionise the treatment of corneal endothelial failure, improving patient outcomes in Australia and beyond.

> "The results with CTTWA have shown that we can generate well over 100 treatments from a single donor – this is a game-changer. The support from TIA to get this off the ground was invaluable" **Dr Evan Wong**

Impact:

TRL 1 | TRL 2 | TRL 3 | **TRL 4** | **TRL 5** | **TRL 6** | TRL 7 | TRL 8 | TRL 9

Production of pluripotent cell derived cardiomyocytes

ADVANCING CARDIAC REGENERATIVE MEDICINE THROUGH GMP-READY STEM CELL THERAPY

CELL AND GENE THERAPIES

Heart failure remains a major global health challenge, with limited treatment options beyond heart transplantation. **Prof James Chong** (University of Sydney), **Dr Andrew Prowse** (University of Queensland), and **Dr Steve Dingwall** (Australian Institute for Bioengineering and Nanotechnology) are leading efforts to commercialise pluripotent stem cell-derived cardiomyocytes (PSC-CMs) as a scalable and safe alternative to transplantation. By refining production methods, this initiative aims to provide a reliable, high-quality cell therapy product to treat patients in heart failure.

The Pipeline Accelerator awards have been key enablers in leveraging a $4.9M MRFF grant, allowing the project team to access Q-Gen Cell Therapeutics and Sydney Cell and Gene Therapy while accelerating progress toward clinical translation.

At Q-Gen Cell Therapeutics, the team received hands-on training in GMP standards, environmental monitoring, and quality management, enabling a pilot GMP production of PSC-CMs. This collaboration refined production protocols, ensured GMP compliance, sourced materials, managed documentation, and conducted batch testing—key for Therapeutic Goods Administration compliance.

The team were then able to transfer PSC-CM batches to Sydney Cell and Gene Therapy in order to establish GMP-compliant formulation, clinical delivery protocols, and process execution under a suitable quality management system— a critical step for advancing the PSC-CM program toward Phase I clinical trials.

This project has successfully established a master cell bank, demonstrated proof-of-concept for GMP-compliant PSC-CM production, and progressed toward a pre-regulatory safety data package. Since receiving the TIA voucher, the team has secured an additional $816,000 grant from the Australian Stroke and Heart Research Accelerator. Additionally, the initiative has fostered new collaborations and positioned Australia as a leader in cardiac regenerative medicine. With clinical trials and commercialisation on the horizon, this work is set to transform heart failure treatment.

> "Translating our research to a clinical trial is a big gear change and we would not have been able to produce GMP compliant cells without our continued involvement with TIA's facilities" **Prof James Chong**

Impact:

TRL 1 | TRL 2 | TRL 3 | TRL 4 | **TRL 5** | **TRL 6** | TRL 7 | TRL 8 | TRL 9

Bacteriophage. Image: Rebecca Bamert, Lithgow Laboratory, Monash University

A NEW FRONTIER IN FIGHTING SUPERBUGS: BACTERIOPHAGE THERAPY IN AUSTRALIA

CELL AND GENE THERAPIES

A/Prof Anthony Kicic and his team at The Kids Research Institute Australia are advancing bacteriophage therapy, a new way of fighting antimicrobial resistance. In 2022, a Technical Feasibility Assessment (TFA) voucher enabled the team to engage with the Cell and Tissue Therapies Western Australia (CTTWA), to explore manufacturing pathways and regulatory considerations, laying the groundwork for future development.

Whilst the global regulatory framework for Therapeutic Goods Administration is evolving, the TFA voucher provided an opportunity to assess manufacturing scenarios and identify pathways for eventual patient treatment. The collaboration clarified the strict requirements of Good Manufacturing Practices (GMP), highlighting the challenges of transitioning from research to a regulated therapeutic.

This initial engagement has grown into a three-year partnership with CTTWA. The research team established two PC2 laboratories within the Centre for Advanced Therapies, equipped to meet GMP compliance. So far, they have successfully completed three batches of phage manufacturing, with more in progress.

The project has secured $650,000 from the Western Australian Future Health and Research Innovation Fund and a $1,800,000 donation from the Stan Perron Charitable Foundation. This will support a 3,000 bacteriophage library against many species of bacteria that develop antibiotic resistance, preclinical safety assessments, and manufacturing validation in preparation for clinical delivery. A further $1,900,000 has been awarded by the MRFF to support a clinical trial to treat *Pseudomonas aeruginosa* lung infections.

Most significantly, the project has translated into real-world clinical impact. Through Australia's Special Access Scheme, two patients in Western Australia - one adult and one child - have received phage therapy, successfully clearing their infections without adverse effects. This is a significant milestone in demonstrating the safety and therapeutic potential of bacteriophage treatments.

> "The TIA voucher enabled engagement with parties experienced in the manufacture of biologics, that then identified how phages could be produced and supplied in Australia" **A/Prof Kicic**

Impact:

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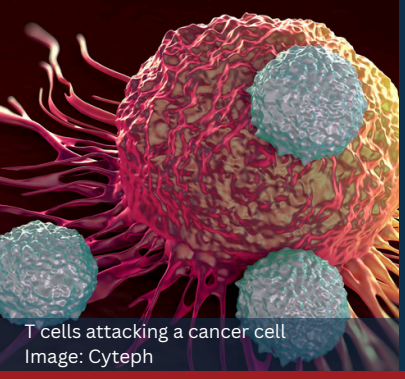


T cells attacking a cancer cell
Image: Cyteph

EXPANDING ACCESS TO "OFF-THE-SHELF" T CELL THERAPY FOR CANCER AND TRANSPLANT PATIENTS

CELL AND GENE THERAPIES

Under the leadership of **Prof Rajiv Khanna**, Distinguished Scientist at QIMR Berghofer, Co-Director of the Queensland Immunology Research Centre, and Founder of Cyteph, this project is pioneering allogeneic or 'off-the-shelf' T cell therapies for transplant patients and a dual-targeting CAR T platform to transform cancer treatment. By creating a cell bank of donor-derived virus specific T cells, many patients can be treated with a single batch, eliminating the need for costly and time-consuming individualised cell collection.

Support from TIA through the Pipeline Accelerator Scheme was instrumental in advancing this research, enabling two phase I clinical trials. TIA vouchers enabled the team to access Q-Gen Cell Therapeutics' expertise and facilities, allowing them to develop a manufacturing process and create banks of cells that have been used to treat immunocompromised transplant patients with persistent viral complications and patients with a type of brain cancer called glioblastoma multiforme. This early support enabled funding successes, including a $1.1M from the NHMRC and $1.3M from the Children's Hospital Foundation Queensland. It also enabled a partnership with the Australian Bone Marrow Donor Registry, strengthening the foundation for a national-scale program.

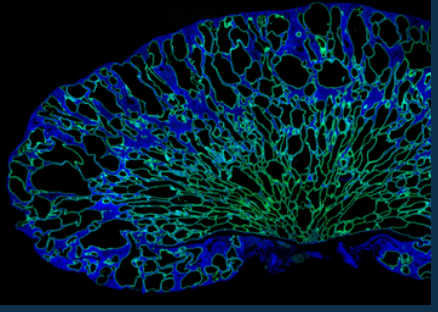
With growing evidence that donor-derived immune cells can be safely matched and used in therapy, the long-term goal is to ensure Australian patients have access to this breakthrough treatment at no cost. By collaborating with leading Australian organisations in transplantation and immunotherapy, the team aims to create a national repository of "off-the-shelf" virus-specific T cell therapies.

Encouraging results in transplant patients led to a broader vision - expanding this approach to treat brain cancer. Using CMV-specific T cells as a platform to deliver CAR T- therapies, the team secured a $1.5M MRFF CUREator grant, leading to the creation of a new company, Cyteph. With a Phase I clinical trial for patients with recurrent glioblastoma multiforme due to finish late 2025, this project is laying the groundwork for wider clinical adoption.

> "TIA's support was the catalyst that set this program in motion, enabling a small initial project to evolve into a platform with the potential to transform cancer treatment nationwide." **Prof Rajiv Khanna**

Impact:

TRL 1 | TRL 2 | TRL 3 | TRL 4 | TRL 5 | **TRL 6** | TRL 7 | TRL 8 | TRL 9



Mouse kidney with PKD
Image: Dr Allara Zylberberg

ADVANCING POLYCYSTIC KIDNEY DISEASE TREATMENTS: THE RISE OF XCYSTENCE BIO

SMALL MOLECULE THERAPEUTICS

Groundbreaking research into Polycystic Kidney Disease (PKD) —an inherited condition that often leads to renal failure and requires dialysis or transplant, has led to the creation of xCystence Bio, a new biotech spin-out from Monash University. This venture builds on discoveries made by researchers from the Monash Biomedicine Discovery Institute (BDI) and the Monash Institute of Pharmaceutical Sciences (MIPS), who identified a key cell signalling pathway driving the formation and growth of kidney cysts. Founders include BDI's **Prof Ian Smyth** and **Dr Denny Cottle**, along with **Prof Paul Stupple** and **Dr Yichao Zhao** from MIPS.

TIA's Pipeline Accelerator voucher scheme played a pivotal role by providing access to the Australian Translational Medicinal Chemistry Facility and the Centre for Drug Candidate Optimisation for efficacy testing and drug interaction studies. This support enabled the team to translate their findings into targeted therapeutic candidates with the potential to slow or halt disease progression by advancing the project toward Phase I trials and supporting the development of a diversified portfolio of compounds.

Now backed by a $500,000 CUREator grant from the Medical Research Future Fund, a $1.1M NHMRC Ideas Grant and a $891,000 NHMRC Development Grant, xCystence Bio is working to develop new treatments for PKD. The company's mission is clear: to bring safe and effective therapies to patients and improve their quality of life. TIA's early support has been instrumental in helping the team move from proof-of-concept research to establishing a viable commercial venture with promising clinical potential.

> "The TIA Voucher Program was critical in facilitating the transition from biological discovery to drug development. It allowed us to collaborate with talented scientists expert in medicinal chemistry and compound profiling who have helped us to realise our translational aspirations."
> **Prof Ian Smyth**

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A NEW APPROACH TO HALTING PULMONARY HYPERTENSION PROGRESSION

SMALL MOLECULE THERAPEUTICS

Pulmonary arterial hypertension (PAH) is a severe condition predominantly affecting women, causing progressive damage to the pulmonary vessels and leading to heart failure. Current treatments offer only symptomatic relief without addressing the underlying causes. A multidisciplinary team from Monash Institute of Pharmaceutical Sciences (MIPS), Monash University, led by **Dr Chengxue Helena Qin**, is developing a novel therapy targeting a G-protein coupled receptor involved in lung vessel inflammation.

The Pipeline Accelerator scheme facilitated access to TIA's Australian Translational Medicinal Chemistry Facility (ATMCF) and Phenomics Australia's Monash Genome Modification Platform. Early lead compounds were identified through high-throughput screening at the National Drug Discovery Centre using libraries prepared by Compounds Australia, two TIA-supported facilities. Co-funding enabled validation of hits from a 300,000-compound screen, identifying promising structures. Additional support from a NHMRC Ideas grant ($730,000), a NHMRC Development Grant ($999,000) and from BioCurate's Proof-of-Concept Fund ($499,000) allowed the team to expand on these early leads, synthesising over 50 compounds and identifying three promising chemical series with strong potential for drug development.

The project has already yielded several hits with optimisable scaffolds and promising selectivity for the target receptor. These early breakthroughs set the stage for developing potent, selective agonists, paving the way for future clinical applications. The team is now exploring collaborations with ATMCF and the Centre for Drug Candidate Optimisation, both within MIPS, to advance the project through lead optimisation and preclinical validation, with the ultimate goal of delivering a disease-modifying treatment for PAH. Should this program continue to maintain its current success over 2025, then BioCurate has indicated its willingness to consider executing its exclusive option to license the project or potentially support the formation of a spin-out company.

> "Drug discovery is a team effort, and having the right infrastructure and industry support makes all the difference. Huge thanks to Therapeutic Innovation Australia for helping us take science to the next level while also supporting the next generation of researchers. Your commitment to innovation and collaboration is truly appreciated!" **Dr Chengxue Helena Qin**

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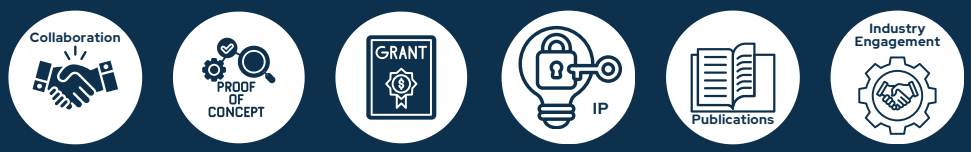

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DEVELOPMENT OF METTL5 INHIBITORS FOR THE TREATMENT OF SOLID TUMOURS IN CHILDREN AND ADULTS

SMALL MOLECULE THERAPEUTICS

At the Children's Cancer Institute, the THerapeutic INnovations for Kids (THINK) program, led by **Prof Ian Street**, Director, and **A/Prof Greg Arndt**, Head of Drug Discovery Biology, is an end-to-end drug discovery capability dedicated to generating new therapeutics for clinical application to transform the lives of children suffering from rare genetic diseases, cancer, and neurodevelopmental disorders.

The team has received multiple Pipeline Accelerator awards across multiple projects, one of which focuses on developing inhibitors of Methyltransferase-like protein 5 (METTL5), an RNA methyltransferase linked to neuroblastoma progression and poor survival outcomes. The goal is to create a novel, patentable METTL5 inhibitor and demonstrate its potential in preclinical cancer models to attract investment for further development.

The Pipeline Accelerator award was instrumental in accelerating METTL5 inhibitor development. The Australian Translational Medicinal Chemistry Facility (ATMCF) supported medicinal chemistry work, while the Centre for Drug Candidate Optimisation (CDCO) provided assays to assess pharmacokinetics.

With limited funds, sourced from different providers, the Pipeline Accelerator boosted the initial seed funding to enable a rapid start and provide early proof of concept data. This data was then leveraged by the team to raise an additional $3,184,000 from successful grant application and philanthropic donations. The team is now continuing medicinal chemistry optimisation, aiming to develop a first-in-class METTL5 inhibitor that offers a targeted, less toxic treatment option for children with neuroblastoma and potentially also adults with some solid tumours. The Pipeline Accelerator scheme has been critical in bridging the funding gap between early research and major grants, enabling the generation of preliminary data necessary to support long-term development. This initiative highlights how strategic early investment can drive drug discovery and the development of potentially life-saving therapies.

> "The TIA Voucher program enabled early access to medicinal chemistry and physicochemical expertise through ATMCF and CDCO, to initiate the early development of small molecule inhibitors of METTL5, and generate key proof-of-concept data to support applications for further funding."
> **Prof Ian Street**

Impact: Collaboration · Proof of Concept · Grant · IP

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Image: The Kids Research Institute Australia

FROM MOLECULE TO MEDICINE: ADVANCING CANCER IMMUNOTHERAPY

SMALL MOLECULE THERAPEUTICS

Prof Joost Lesterhuis, Head of the Cancer Program and Sarcoma Translational Research team at The Kids Research Institute, is leading an ambitious effort to develop a tablet-based cancer immunotherapy. In collaboration with **A/Prof Matthew Piggott** at the University of Western Australia, his team identified a promising molecular target and began designing small molecules to interact with it. Early work identified compounds with promising activity in biochemical and cell-based assays, but to progress towards the clinic, suitable pharmacokinetic properties must also be defined.

To address this requirement, the research team accessed the Centre for Drug Candidate Optimisation (CDCO) at Monash University through TIA's Pipeline Accelerator scheme. The CDCO supported the project through study design, data analyses and interpretation, and guidance on follow-up research. This collaboration enabled the team to investigate how their compounds are absorbed, distributed and metabolised. The pharmacological insights helped refine and prioritise multiple chemical series. By integrating CDCO feedback into the chemistry workflow, the team have discovered drug-like lead compounds. This work is now being progressed by Setonix Pharmaceuticals, a CUREator-backed company established in 2022.

The Pipeline Accelerator voucher contributed to building critical momentum for the project. The team secured close to $2 million in funding, including grants from the MRFF Medical Research Commercialisation Initiative, the WA Future Health Research and Innovation Fund, and Cancer Council WA.

Beyond these data, the collaboration with CDCO gave the team access to specialised expertise and techniques that are unique in Australia. It also laid the groundwork for a long-term partnership that continues to support the project's development.

In 2024, Setonix Pharmaceuticals was named a finalist in the WA Innovator of the Year awards. With a growing portfolio of optimised compounds and a clear clinical goal, the team is now preparing to raise venture capital to move toward a phase one clinical trial.

> "TIA vouchers have allowed us to access world-class expertise and service from the CDCO. Without this support, we would be forced to use overseas contract research organizations that do not provide the same level of collaborative support afforded by CDCO." **Prof Joost Lesterhuis**

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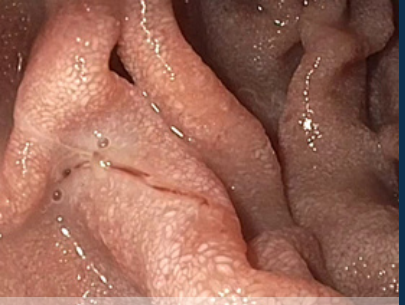

Adult hookworm in the small intestine. Image: Dr John Croese, Prince Charles Hospital

DEVELOPING HOOKWORM-DERIVED BIOLOGICS FOR INFLAMMATORY BOWEL DISEASE

BIOLOGICS AND VACCINES

Inflammatory bowel disease (IBD) affects over 80,000 Australians, with existing biologic therapies often expensive, ineffective, or causing adverse side effects. Researchers at James Cook University, led by **Prof Alex Loukas**, are exploring a novel approach using hookworm-derived proteins to suppress inflammation. With TIA's support, the team has successfully advanced the early preclinical development of three promising proteins with protective effects in a mouse model of colitis.

Through the Pipeline Accelerator scheme, the research team engaged with the National Biologics Facility (NBF) to produce the proteins of interest in an industry-relevant cell line and with a scalable bioprocess. Stability assessments provided key insights that helped refine the selection of two lead candidates for further development. This data was instrumental in supporting commercialisation efforts, giving confidence in the reproducibility of results and the feasibility of future scale-up of the candidates to support large-scale manufacturing under Good Manufacturing Practice conditions.

Macrobiome Therapeutics was spun out of James Cook University in early 2021 to develop these proteins and IP as novel anti-inflammatory biologics.

Further funding has been secured, including a $793,000 DARPA Embedded Entrepreneur Award and $1.8M from the CRC for Northern Australia, Tegmen Fund and JCU. Future collaboration with NBF may include cell line development and bioprocess optimisation, with plans to engage additional facilities for validation studies and toxicity testing. By enabling key steps in this research, TIA's support has accelerated the path toward a new class of biologic therapies for IBD, bringing potential new treatment options closer to patients.

> "The TIA voucher program was invaluable to us in securing further investment in our R&D program by providing third party validation and CMC data from a very well-regarded institution. We had a great experience with TIA, and found their work was timely, reliable, and offered huge bang for buck. We will definitely work with them again!" **Prof Alex Loukas**

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Oral insulin capsules. Image: University of Sydney, Stefanie Zingsheim

ADVANCING ORAL INSULIN WITH NANOTECHNOLOGY

BIOLOGICS AND VACCINES

Insulin therapy is essential for managing type 1 diabetes, yet current treatments rely heavily on injection-based delivery. Researchers at the University of Sydney, led by **Dr Nick Hunt**, have developed a nanotechnology-based platform using silver sulphide (Ag_2S) quantum dots to enable oral insulin delivery—a completely novel manufacturing approach.

With TIA's support the team collaborated with the CSIRO Biomedical Manufacturing team in Clayton, Victoria. The CSIRO team successfully reproduced and then optimised the synthetic manufacturing process for the insulin-conjugated quantum dots, ensuring greater reproducibility and scalability.

TIA's support facilitated collaboration between the University of Sydney and CSIRO, demonstrating that the formulation could be externally produced at scale. CSIRO successfully manufactured a gram-scale batch that met key characterisation and efficacy benchmarks, providing a crucial blueprint for transitioning from non-good manufacturing practice (GMP) to GMP production. This batch was later used for GLP validation studies at Agilex Biolabs, and Ab Initio Pharma is now working on the GMP-grade formulation. Both facilities have joined the TIA Pipeline Accelerator scheme in 2025.

The project's success attracted significant investment, including $750,000 in MRFF TTRA funding, $50,000 in CSIRO Kickstart funding, and $3.2M from venture capital provided by Proto Axium for the newly established biotech spin out, Endo Axiom. With a focus on preventing autoimmune diseases and improving diabetes care, Endo Axiom is now advancing this platform toward Phase I clinical trials. By enabling critical early-stage development, TIA's support has accelerated the commercial pathway for this transformative technology, bringing the possibility of oral insulin closer to reality.

> "The TIA program enabled external manufacturing studies to show our formulation could be produced outside of our lab."
> **Dr Nick Hunt**

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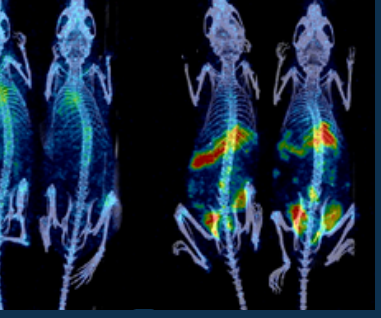

Tracer compounds in a rat cancer model

ADVANCING IMAGING FOR OVARIAN AND BLADDER CANCER

BIOLOGICS AND VACCINES

Prof John Hooper and his team at Mater Research, University of Queensland, are developing a novel radio-imaging agent (10D7 antibody) to improve ovarian cancer detection and for future applications, to guide targeted therapy. By tagging cancer cells with a tracer, this approach aims to enhance disease visibility during radiology scans, similar to prostate cancer imaging.

With TIA's support, this collaboration has leveraged expertise from the National Biologics Facility at the University of Queensland, and CSIRO Biomedical Manufacturing in Clayton, Victoria. These facilities helped optimise the 10D7 monoclonal antibody for PET-CT imaging by developing five variants to improve manufacturability and reduce immune activation. They then established large-scale production and purification methods for conjugating 10D7 with a chemical linker (DFO), ensuring clinical-grade quality.

TIA funding was pivotal in making clinical-grade production feasible. "Without it, academic researchers would struggle to afford industry manufacturing rates." said Prof John Hooper. Through this support, over 500 vials of clinical-grade antibody were produced, enabling Phase I trials.

The project has since secured $1.9M in MRFF funding for an ovarian cancer trial and $950,000 from the CUREator scheme for a bladder cancer trial.

Preclinical imaging experiments were conducted at the Translational Research Institute and facilities from the NCRIS-supported National Imaging Facility; the Centre for Advanced Imaging at the University of Queensland, with radiolabelling performed by Q-TRaCE at the Herston Royal Brisbane and Women's Hospital precinct.

With a Phase I clinical trial underway, the team credits TIA-funded research as critical in advancing this technology toward future clinical use in ovarian and bladder cancer patients.

"The TIA voucher program was absolutely essential for us to move forward confidently from a laboratory-grade reagent to a clinical grade radio-imaging agent for evaluation in ovarian cancer and bladder cancer patients. The bottom-line is that without the TIA scheme we'd still be locked out of translating our technology into something that has potential to benefit humans from both a health perspective and an economic perspective." **Prof John Hooper**

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Image: Denteric

ENABLING TRANSLATION OF AN IMMUNOTHERAPY AGAINST *PORPHYROMONAS GINGIVALIS*

BIOLOGICS AND VACCINES

Denteric is a clinical-stage biotech company developing therapies for *Porphyromonas gingivalis* (Pg) related diseases, with an initial focus on an immunotherapy to protect against periodontitis, a severe gum disease caused by Pg. This infection can lead to tooth loss and has been linked to Alzheimer's, atherosclerosis, and diabetes. Denteric's first-in-class therapeutic immunotherapy aims to provide an alternative to standard treatments like scaling and root planning by reducing inflammation and preventing tissue damage caused by Pg.

Prior to the TIA award, Denteric was already in discussions with the National Biologics Facility (NBF) to develop critical analytical methods for testing of their lead immunotherapy. This included establishment of standard physico-chemical analyses, but also developing quantitative methods for identity, purity and potency that they couldn't source from the market to meet their timelines and / or quality requirements. Once the award was made the project with NBF was able to rapidly deploy which was a key enabler for Denteric who required analytics testing as a requisite for initiating their first-in-human clinical trial.

Through this project, Denteric have successfully leveraged collaborative partnerships with both NBF and two commercial contract manufacturing organisations (CMOs). This reflects the real-world supply-chain complexity faced by many Australian researchers and SMEs in developing innovation from the lab to clinical applications. But it also showcases the considerable value that TIA-supported research infrastructure can provide to fill missing gaps along this translation pipeline.

TIA's support has been instrumental in enabling Denteric to initiate their first-in-human clinical trials. Moving forward, Denteric plans to continue the collaboration with NBF for stability studies and have already leveraged the assay package to support method-transfer to another CMO for planned clinical trials of their immunotherapy.

"Denteric is grateful to TIA for having received the NCRIS funding through the Pipeline Accelerator scheme. Being able to access the expertise of scientists at NBF has enabled our manufacturing program to advance without having to engage the necessary expertise internally. This has helped to save us both time and money and reach the clinic quicker"
Larisa Chisholm, COO Denteric

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INSIGHTS FROM THE SECTOR ON THE PIPELINE ACCELERATOR

"Voucher schemes such as the Pipeline Accelerator provide a crucial connection between research and development and encourage researchers to think translationally much earlier in their research journey. By partnering on this scheme, Phenomics Australia incentivises researchers to consider how working with PA can facilitate acceleration of their research along a pipeline of NCRIS capabilities addressing early stage through to translational outcomes."

Dr Carol Wicking
Board member and Science Advisor of **Phenomics Australia**

"I'm a strong supporter of TIA's Pipeline Accelerator Program. It is targeted at a crucial point in the discovery of novel therapeutics, supporting studies that would otherwise be too expensive to conduct. I've personally benefited from this program across multiple projects and strongly recommend it to any drug developer, in academia or biotech, to explore this program for advancing their research."

Dr Chris Burns
Chief Executive Officer and Managing Director of **Amplia Therapeutics**

FUTURE OUTLOOK

WHAT'S NEXT FOR THE PIPELINE ACCELERATOR?

TIA is immensely proud of the significant and positive impact that the Pipeline Accelerator scheme has had to date helping to drive innovative therapeutic development in this sector, but we can still go further in our mission.

We see the potential to better leverage and enable access to the fantastic and growing expertise across this pipeline of research infrastructure that is enabling therapeutic translation for Australian researchers. We will do this by **expanding scope** and **increasing scale**.

EXPANDING SCOPE

The demand for vouchers is increasing year on year. TIA will seek additional funding to accommodate this increase in demand, but also to strategically expand the scope of the network to include additional facilities that can address capability and capacity gaps.

We are also keen to share our experience with voucher-style schemes with other NCRIS providers and expand the network of facilities that can be accessed in this way. We already partner with Phenomics Australia and NDF, and have just welcomed Bioplatforms Australia in 2025.

INCREASING SCALE

The $50,000 voucher scheme was designed to assist early-stage translational projects (TRLs 3-4) to start to cross the "Valley of Death". The level of interest from researchers with later stage projects (TRLs 5-6) argues for an additional scheme to offer fewer, larger vouchers of up $200,000. These awards would be Phase I-enabling, that is, they would progress a project to the point that it is ready for a first-in-human clinical trial, which represents a major value inflection point. Awards would be accompanied by project management and business support, maximising the chance of a successful outcome.

By continuing to adapt to the evolving needs of the research and biotech communities, the Pipeline Accelerator scheme plays a pivotal role in shaping the future of Australia's biomedical sector, driving breakthroughs that improve patient outcomes and strengthen Australia's position in the global therapeutics landscape.

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