Annual Report

2016
2017

THERAPEUTIC INNOVATION AUSTRALIA

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Contents

Acronyms and abbreviations

ACRF	Australian Cancer Research Foundation
ADME	absorption, distribution, metabolism and excretion
ANZCTR	Australian New Zealand Clinical Trials Registry
CEO	Chief Executive Officer
NATA	National Association of Testing Authorities, Australia
NCRIS	National Collaborative Research Infrastructure Strategy
NHMRC	National Health and Medical Research Council
Pipeline	Australian Therapeutic Pipeline
SME	small-to-medium enterprise
TGA	Therapeutic Goods Administration
THD	Translating Health Discovery into Clinical Applications
TIA	Therapeutic Innovation Australia
UQ	University of Queensland

Chairman and CEO report

Mr Terry Slater
Chairman

Dr Stuart Newman
Chief Executive Officer

Who we are

Therapeutic Innovation Australia (TIA) is a leading not-for-profit research infrastructure organisation that has managed National Collaborative Research Infrastructure Strategy (NCRIS) funding under the **Translating Health Discoveries into Clinical Applications** (THD) project since 2008.

TIA supports the hard and soft infrastructure required to accelerate the translation of laboratory discoveries into clinical applications, leading to therapeutic products and techniques. To do this, we invest in a multidisciplinary consortium of capabilities to enable access to state-of-the-art facilities, and highly qualified technical staff to operate and maintain them. TIA is broadening this consortium through its *Australian Therapeutic Pipeline*, to increase connectivity between facilities, and enable referral business between members, a higher profile and more efficient translations of discoveries.

TIA represents the only NCRIS project that supports the necessary capability areas to pursue development of new therapeutic products through the various phases of testing, from preclinical into clinical trials. Clinical trials are an inflection point for higher-level industry investment.

Our Mission

To enable and accelerate translational research leading to commercial and product outcomes.

Our Vision

Improved efficiency and effectiveness in translational health research, through better supported and better connected translational research capabilities.

TIA's leadership in therapeutic development

TIA's leadership of the THD project reinforces the Australian Government's National Innovation and Science Agenda (NISA). We take an innovative approach to develop initiatives and enabling infrastructure to support NISA, such as the following:

- **ATRAX** is TIA's national medical research project database of funded, in-progress medical research projects. ATRAX is thus a source of projects that may benefit from access to TIA-supported facilities. ATRAX has continued to develop in 2016–17, with the help of student interns who source and prepare data for input.
- **iQDOCs** is a quality assurance document database that helps researchers and facilities to establish and maintain compliance with the requirements of regulatory bodies such as the Therapeutic Goods Administration (TGA). This unique resource continued to grow in access and use, and recent improvements to the website will soon begin to benefit subscribers.
- The **Australian Therapeutic Pipeline** (the Pipeline), launched in 2014, brings facilities together, enabling a product to move quickly from the laboratory bench to clinical trials.

During 2016–17, the Pipeline became a member organisation, and all members were able to access ATRAX and iQDOCs. We are looking to increase the value proposition of Pipeline membership by developing two new initiatives: the *Pipeline Accelerator* voucher-based project support program, and the *Pipeline Navigator* interactive capability map.

2016–17 was a pivotal year

The past financial year represented a pivotal period in the history of the TIA consortium. It ended on the cusp of a period of change, as a result of our response to the *2016 National Research Infrastructure Roadmap*. The 2016

Roadmap is a comprehensive document that sets a strong foundation for the Australian Government's next steps in developing the NCRIS program. It presents an opportunity for NCRIS projects, such as TIA, to examine their current activities, and realign and redesign their investments.

During 2016–17, TIA supported the employment of 62 staff in 21 facilities, who collectively assist with more than 1100 projects. TIA facilities were accessed by 958 users – in keeping with NCRIS objectives to facilitate external access to capabilities. This number does not include the web-based Australian New Zealand Clinical Trials Registry (ANZCTR), which saw many thousands of visitors and registrants.

In keeping with government policy to support development of clinical trials, TIA consortium members supported or conducted 239 trials during 2016–17. In terms of commercial outcomes, TIA members reported working on 57 therapeutic products, leading to 10 patents and 7 licences.

The 2016 Roadmap

One of the most important external developments during 2016–17 was the publication of the 2016 Roadmap. This comprehensive document, prepared by Chief Scientist Alan Finkel and a team of expert reference groups, sketches out the future infrastructure needs of Australian science. TIA was delighted to see three of our consortium members – the National Biologics Facility, Compounds Australia and the ANZCTR – given prominent mention under the Therapeutic Development focus area.

TIA considers the 2016 Roadmap to be an excellent opportunity to take stock of our current investments in infrastructure. The Roadmap's focus on Discovery, Production, Testing and Integration already aligns strongly with TIA's strategy. We can now further realign and refocus our efforts to ensure that our nationally leading capabilities are given the support they need.

The future

The importance of translational research to both the economy and the health of our nation has never been more apparent. However, despite improvement, Australia still lags behind some other countries in the conversion of our excellent research discoveries into therapeutics with medical, social and economic value. TIA will continue to advocate for increased support of translational research. We are optimistic that this will translate into higher levels of investment in the critical infrastructure that underpins the conversion of discoveries into new therapeutics that will have positive downstream health, economic and societal impacts.

Mr Terry Slater
Chairman

Dr Stuart Newman
Chief Executive Officer

NCRIS and TIA

Since 2004, the Australian Government has invested more than $2.5 billion to deliver world-class research infrastructure under NCRIS.

NCRIS has invested in 27 active projects, comprising 222 institutions, and employing almost 1700 highly skilled technical experts, researchers and facility managers. NCRIS facilities are used by more than 35,000 researchers each year, both domestically and internationally. The program has attracted more than $1 billion in co-funding for its activities from state and territory governments, universities, research facilities and industry.

NCRIS
National Research Infrastructure for Australia
An Australian Government Initiative

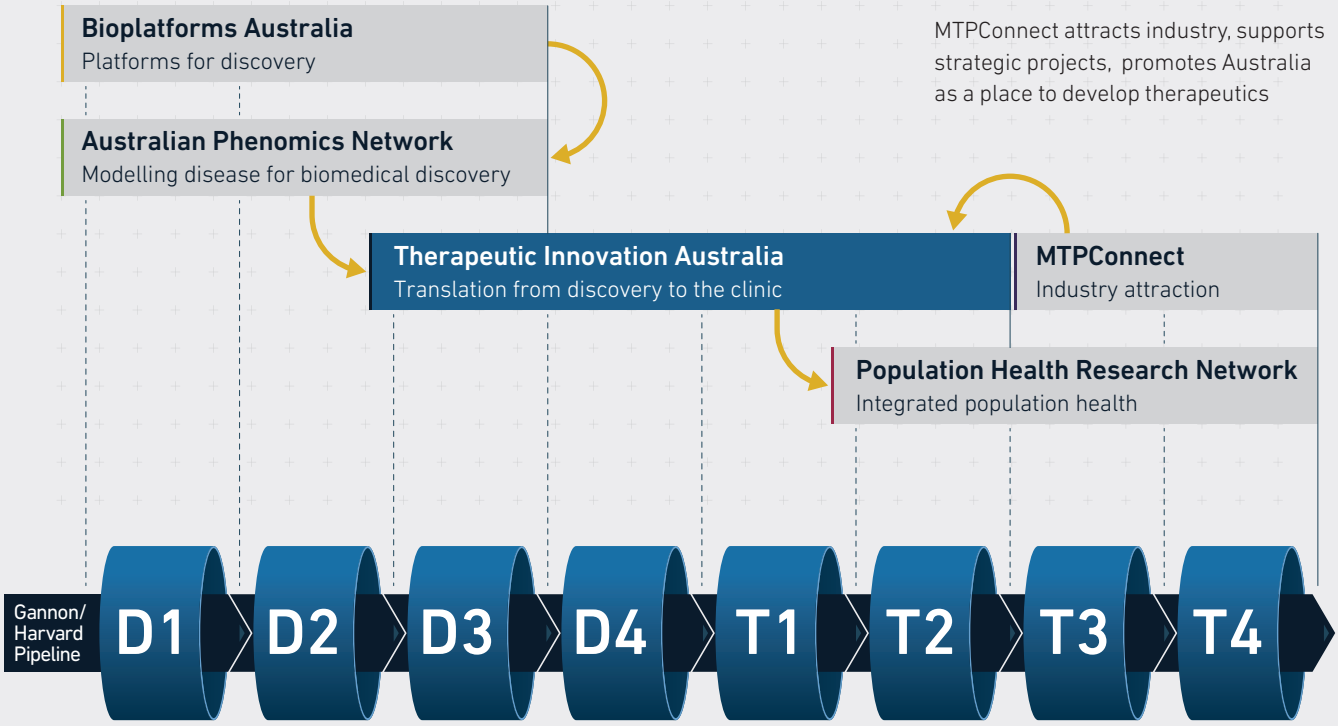
Our place in NCRIS

TIA occupies a unique niche in the NCRIS program, by supporting the human and capital capability to take discoveries and put them on the long, risky and expensive road towards clinical trials and market (see page 6). This pipeline-based approach differs from the platform-based approach seen in other projects.

TIA has the capability to source potential therapeutic development projects from other NCRIS subprojects, such as Bioplatforms Australia and the Australian Phenomics Network, and also contribute to Population Health Research Network activities.

MTPConnect is emerging as a key stakeholder for TIA, primarily as a way to attract new commercial entities to using the excellent capabilities available through our consortium. Engagement between TIA and MTPConnect is increasing, and we expect this to be a fruitful relationship.

Where TIA fits



D1 Basic research, no projected clinical or commercial outcome, no target disease e.g. basic cell biology

D2 Research with a specific disease in mind e.g. target discovery, biological pathway, biomarker discovery

D3 Research to identify an agent to modulate a disease pathway. Includes High Throughput Screening, rational drug design, antibody discovery/engineering, Hit to Lead

D4 Pre-translation, an agent identified. Includes lead optimisation, ADME-TOX, DMPK+

Adapted from Gannon, F. (2014) The steps from translatable to translational research. EMBO Reports 15(11):1107-1108

T1 Translation into humans e.g. phase I trials

T2 Translation to patients e.g. phase II/III trials

T3 Translation into practice e.g. application to clinical care

T4 Translation to populations e.g. public health applications

Adapted from Harvard Catalyst Pathfinder site (http://catalyst.harvard.edu/pathfinder)

Our consortium and its capabilities

The TIA consortium represents Australia's leading translational medical research service providers. During 2016–17, TIA managed NCRIS funding that supported critical operational salaries at public sector facilities, enabling access to these world-class capabilities. Each facility has a pricing and access policy in place that aligns with NCRIS objectives. Many have tiered pricing that offers reduced pricing for university research groups.

To make contact with any of the capabilities listed:

- contact TIA directly at info@therapeuticinnovation.com.au
- contact the facilities via www.therapeuticinnovation.com.au/facilities.

The TIA consortium is organised according to therapeutic modality and place in the therapeutic development pipeline [see page 8]:

- small molecule discovery and development
- biologics discovery and development
- preclinical studies
- cell and tissue therapies
- clinical trial planning and support.

The TIA consortium in 2016–17

Small molecule discovery and development

Australian Translational Medicinal Chemistry Facility

Advanced medicinal chemistry

HOST ORGANISATION:
Monash Institute of Pharmaceutical Sciences

Biologics discovery and manufacture

National Biologics Facility

Discovery, development and manufacture of biologic therapies

HOST ORGANISATION:
Australian Institute for Bioengineering and Nanotechnology (UQ) and CSIRO (Parkville)

Cell and tissue therapies

Cell & Tissue Therapies WA

GMP manufacture of cell and tissue therapies

HOST ORGANISATION:
Royal Perth Hospital

Cell and tissue therapies

Sydney Cell and Gene Therapy

Manufacture of cell, gene and tissue therapies

HOST ORGANISATION:
Westmead Hospital

Small molecule discovery and development

ACRF Drug Discovery Centre for Childhood Cancer

Screening for small molecule discovery

HOST ORGANISATION:
Children's Cancer Institute Australia

Preclinical studies

Centre for Integrated Preclinical Drug Development

ISO-accredited preclinical studies

HOST ORGANISATION:
UQ Centre for Clinical Research (UQ)

Clinical trial planning and support

Victorian Cancer Biobank

Biobanking tumour tissue samples

HOST ORGANISATION:
Victorian Cancer Biobank

Clinical trial planning and support

Australian Cancer Research Foundation Cancer Genomics Facility

Genomics to support clinical trials

HOST ORGANISATION:
SA Pathology

Small molecule discovery and development

Compounds Australia

National small molecule compound library for drug discovery

HOST ORGANISATION:
Griffith Institute for Drug Discovery

Preclinical studies

Translational Research Institute Preclinical Imaging Facility

Preclinical imaging studies

HOST ORGANISATION:
Translational Research Institute

Clinical trial planning and support

Molecular Diagnostics Facility

Genomics to support clinical trials

HOST ORGANISATION:
Institute for Health and Biomedical Innovation (QUT)

Clinical trial planning and support

Kinghorn Centre for Clinical Genomics

Genomics to support clinical trials

HOST ORGANISATION:
Garvan Institute of Medical Research

Small molecule discovery and development

Florey Ion Channel Analysis Facility

Screening for small molecule discovery

HOST ORGANISATION:
Florey Institute of Neuroscience and Mental Health

Preclinical studies

Centre for Drug Candidate Optimisation

Preclinical studies and small molecule development

HOST ORGANISATION:
Monash Institute of Pharmaceutical Sciences

Clinical trial planning and support

Australian New Zealand Clinical Trials Registry

National clinical trial registry

HOST ORGANISATION:
NHMRC Clinical Trials Centre

Clinical trial planning and support

Molecular Pathology Laboratory

Pathology to support clinical trials

HOST ORGANISATION:
Peter MacCallum Cancer Centre

Small molecule discovery and development

Walter and Eliza Hall Institute Screening Laboratory

Screening for small molecule discovery

HOST ORGANISATION:
Walter and Eliza Hall Institute of Medical Research

Preclinical studies

Centre for Clinical Diagnostics

Preclinical studies and diagnostic development

HOST ORGANISATION:
UQ Centre for Clinical Research

Clinical trial planning and support

Melbourne EpiCentre

Trial support and planning, database design

HOST ORGANISATION:
University of Melbourne

Clinical trial planning and support

Paediatric Trials Network Australia

Access to clinical trial planning and support software

HOST ORGANISATION:
Paediatric Trials Network Australia

Small molecule discovery and development

Discovery and development of small molecules include the screening of large compound libraries against molecular targets. 'Hit' compounds that are active in a screen are developed further to increase their drug-like characteristics.

Australian Translational Medicinal Chemistry Facility

Advanced medicinal chemistry and lead optimisation

The *Australian Translational Medicinal Chemistry Facility* (ATMCF) was established in 2012 at the Monash Institute of Pharmaceutical Sciences. It aims to support researchers during early-stage drug discovery and development using medicinal chemistry. This multidisciplinary research institute encompasses the key skills in translational aspects of drug discovery, delivery and development, including:

- advanced medicinal chemistry
- hit selection from high-throughput screening
- fragment-based drug discovery
- mechanism-based drug discovery
- structure-based drug design.

The ATMCF is accessible to academic and commercial users, with pricing depending on the type of user and the specific arrangement (e.g. fee for service or collaboration). The ATMCF collaborates with researchers from universities, medical research institutes, government research agencies and industry.

Australian Cancer Research Foundation Drug Discovery Centre for Childhood Cancer

Screening for new anticancer molecules

The Australian Cancer Research Foundation (ACRF) *Drug Discovery Centre for Childhood Cancer* is a unique facility dedicated to the development of new anticancer therapeutics. Based at Children's Cancer Institute Australia – and housed within the Lowy Cancer Research Centre at the University of New South Wales – the centre focuses on identifying new compounds for the treatment of childhood and adult cancer.

The centre uses advanced robotic automation and an array of different small molecule chemical libraries to screen for chemical hits capable of altering complex cancer cell phenotypes or biochemical pathways. The centre is available to all medical research groups and operates on a subsidised fee-for-service basis. The staff provide expertise and assistance to researchers who want to scale laboratory assays for high-throughput small molecule screening.

Compounds Australia

National small molecule compound library for drug discovery

Located within the Griffith Institute for Drug Discovery, *Compounds Australia* (formerly the Queensland Compound Library) is a unique national resource that allows chemists to deposit small molecules into a central repository. Life science research teams can access this repository, and the facility can produce microtitre plates in many formats for high-throughput screening for small molecule drug discovery. Compound integrity is assured by integrating advanced, environmentally controlled tube and plate storage with sample processing.

Based on a custom-designed suite of advanced robotics and software, Compounds Australia synergises interactions between Australasian chemistry and biomedical researchers, and their international colleagues.

Florey Ion Channel Analysis Facility

Screening for small molecules against ion channel targets

The *Florey Ion Channel Analysis Facility* (FICF) at the Florey Institute of Neuroscience and Mental Health provides contract services and collaboration opportunities in ion channel analysis and neurophysiology. Since its inception, the FICF has deployed three core technologies:

- automated medium- and high-throughput planar patch clamp recording
- automated two-electrode voltage clamp recording
- multi-electrode array recording for neuronal networks and cardiomyocytes.

In addition, the FICF has developed and accumulated a range of stable cell lines and expression vectors in various ion channel classes. This enables assay development and safety testing for a range of therapeutics.

Walter and Eliza Hall Institute Screening Laboratory

High-throughput screening for small molecule discovery

The *Walter and Eliza Hall Institute* (WEHI) *Screening Laboratory* allows screening of various libraries, including 250,000 diverse, 'lead-like' small molecule libraries, with synthetically amenable structures and high diversity. Of all compounds, 89% are Lipinski compliant and are subject to an open access intellectual property (IP) policy – that is, the IP rights are fully transferred to the customer.

The infrastructure at the WEHI Screening Laboratory is highly advanced and offers some of the highest throughput screening available in Australia.

Biologics discovery and manufacture

Biologics are an exciting and a rapidly expanding class of human therapeutics. Their discovery and development require significant expertise and specific infrastructure to ensure that complex biologic molecules can be produced in sufficient quantities to allow testing and marketing.

National Biologics Facility

Discovery, development and manufacture of biologic therapies

The *National Biologics Facility* (NBF) offers custom manufacturing solutions for the development and production of next-generation biological therapeutics. By combining extensive expertise and state-of-the-art laboratories, the NBF provides the necessary infrastructure to bridge the gap between laboratory experiments and clinical trials.
The facility staff of scientists and bioprocess engineers offer world-class expertise in molecular biology, antibody engineering (including phage display), mammalian cell culture and biopharmaceutical development.

The NBF operates two nodes:

- the Australian Institute for Bioengineering and Nanotechnology at the University of Queensland (UQ)
- CSIRO Molecular Health Technologies in Melbourne.

Each node offers unique infrastructure and services. Together, they enhance capabilities in early-stage biologics technology in Australia.

Preclinical studies

Following the discovery of a hit compound and its development into a 'lead', a molecular or cell-based therapeutic must undergo preclinical testing in animal models to ensure that it is safe for use in human clinical trials. These critical studies require the highest quality systems to ensure that results are reliable.

Centre for Integrated Preclinical Drug Development

ISO-accredited preclinical studies

The *Centre for Integrated Preclinical Drug Development* (CIPDD) at the UQ Centre for Clinical Research offers the state-of-the art infrastructure required to bring innovations to the drug development pathway. The CIPDD facilitates preclinical drug candidate selection and optimisation, which enables researchers to progress their discoveries or inventions across the 'valley of death' and attract investment for commercialisation. It also offers bioanalytical/pharmacokinetic (ADME – absorption, distribution, metabolism and excretion) support for clinical trials.

The CIPDD is a leading Good Laboratory Practice– (GLP-) and ISO17025-accredited service provider. It generates data that are accepted by the United States Food and Drug Administration, the European Medicines Agency and the TGA.

Translational Research Institute Preclinical Imaging Facility

Preclinical imaging studies

The *Translational Research Institute* (TRI) *Preclinical Imaging Facility* provides the expertise and instrumentation to image small animals in vivo and high-performance imaging of fixed samples.

The TRI houses state-of-the-art preclinical imaging equipment and high-performance computers. Siemens's IRW software and a range of open-source software are used to analyse positron emission tomography–computed tomography (PET/CT) data in 3D. The facility also provides researchers with the training and knowledge to efficiently generate high-quality, reproducible images, and to independently perform image analysis and quantification.

Centre for Drug Candidate Optimisation

Preclinical studies and small molecule development

The *Centre for Drug Candidate Optimisation* (CDCO) at the Monash Institute of Pharmaceutical Sciences was established in 2003 to fill critical gaps in drug discovery – translational expertise in ADME, and pharmacokinetic qualities of drug candidates. Identifying the pharmacokinetics of a small molecule during drug discovery is essential to guide appropriate structural modifications and to identify strategies to mitigate risks during drug development.

The CDCO provides expertise and infrastructure for academic and commercial users. Costs depend on the specific arrangement (fee for service, collaboration or another model); reduced rates are available for longer-term projects.

Centre for Clinical Diagnostics

Preclinical studies and diagnostic development

The *Centre for Clinical Diagnostics* (CCD) is a purpose-built facility within the UQ Centre for Clinical Research (UQCCR) in Brisbane. The CCD, together with a dedicated outpatient floor for clinical trials, establishes the UQCCR as a unique clinical research, development and evaluation facility in Australia.

The CCD was established to develop, evaluate and deliver new National Association of Testing Authorities, Australia– (NATA-) accredited in vitro diagnostics. It delivers regulator-approved data to achieve increased research and development efficiencies and return on investment, and reduced time to market for products.

Cell and tissue therapies

Cell-based therapies, including stem cells and CAR-T cells, are an emerging field of therapeutics. Like biologics, their testing and production require extensive quality-accredited infrastructure and expertise to ensure that cell-based therapies remain viable.

Cell and Tissue Therapies WA

Manufacture of cell and tissue therapies, Good Manufacturing Practice accredited

Established in 2006, *Cell & Tissue Therapies WA* (CTTWA) at Royal Perth Hospital manufactures and provides clinical products and services for the public health sector. CTTWA assists with research and development that can translate to the clinic.

CCTWA is Good Manufacturing Practice (GMP) accredited. It has five clean rooms within the manufacturing suite – of which four have International Organization for Standardization (ISO) Class 7 certification – and one large clean room with ISO Class 8 certification. CTTWA also holds a TGA product licence for the manufacture of mesenchymal stem cells.

Sydney Cell and Gene Therapy

Manufacture of cell, gene and tissue therapies

Sydney Cell and Gene Therapy (SCGT) at Westmead Hospital provides manufacturing facilities for cellular and gene therapeutics. SCGT is a consortium comprising the Westmead Research Hub, which includes two tertiary teaching hospitals (Westmead Hospital and the Children's Hospital at Westmead), and three research institutes (the Millennium Institute, the Children's Medical Research Institute and the Kid's Research Institute). The Westmead campus in Western Sydney is one of the largest healthcare and research precincts in the Southern Hemisphere.

Clinical trial planning and support

Australia is very well placed to be a global centre of quality clinical trials, and this is reflected in the high number of clinical trial sites. To accelerate this process, TIA supports centres that, in turn, provide support services to clinical trials to reduce duplication and ensure that the Australian trial community has access to consistently high-quality support.

Victorian Cancer Biobank

Biobanking tumour tissue samples

The *Victorian Cancer Biobank* (VCB) is a not-for-profit consortium of tissue banks, supported by the Victorian Government through the Victorian Cancer Agency. It provides high-quality, ethically obtained biospecimens to support research, leading to improvements in cancer diagnoses and treatments. In 2016, the VCB received NCRIS Agility Funding through TIA.

Since the biobank's establishment in 2006, more than 19,000 Victorians undergoing cancer and other surgery have donated blood and surplus tissue. More than 140 cancer research groups from Australia and overseas have applied for access to blood or tissue samples, and collectively have received more than 33,000 specimens.

Australian Cancer Research Foundation Cancer Genomics Facility

Genomics to support clinical trials

The *ACRF Cancer Genomics Facility* offers the research community the full benefit of the expertise and technology available at the Centre for Cancer Biology. It provides a full range of state-of-the-art genomics and bioinformatics services to South Australian researchers, including:

- Sanger sequencing
- next-generation sequencing from Illumina, Ion Torrent and Roche
- Sequenom MassArrays
- microarrays from Affymetrix and Illumina
- Fluidigm equipment for the study of single cells.

In addition, supercomputer infrastructure, and people skilled in analysing and interpreting the data, are available to help researchers.

Kinghorn Centre for Clinical Genomics

Genomics to support clinical trials

The Garvan Institute of Medical Research established the *Kinghorn Centre for Clinical Genomics* (KCCG) in 2012 to advance the use of genomic information in patient care. The KCCG employs world-leading DNA sequencing technology, and expertise in genetics, pathology and bioinformatics to deliver and interpret genome sequences for research and clinical use.

Services at the KCCG act as a channel to translate laboratory genomic research to the clinic. The KCCG has recently been accredited, to enable it to provide state-of-the-art interpretation of genomic data directly to clinicians. Since 1 August 2016, clinical whole-genome sequencing services to diagnose rare and genetic diseases are available through Garvan's wholly owned subsidiary company, Genome.One.

Molecular Diagnostics Facility

Genomics to support clinical trials

The Molecular Diagnostics Facility helps researchers to discover the genetic basis of common, complex human disorders. Its DNA Diagnostic Facility is NATA accredited. Clinical trials for new treatments are conducted at its Genomics Clinical Trials Centre.

The facility – located at the Institute of Health and Biomedical Innovation, Queensland University of Technology – employs more than 30 research staff, including principal researchers, postdoctoral scientists, research assistants, nurses, and postgraduate and honours students. It is well equipped, and funded by national competitive grants and industry.

Molecular Pathology Laboratory

Pathology to support clinical trials

The facility at the *Molecular Pathology Laboratory*, Peter MacCallum Cancer Centre, is fully integrated into Australia's largest cancer-specific, NATA-accredited molecular pathology laboratory.
The facility offers state-of-the-art protein, RNA and DNA molecular pathology services. It provides high-throughput diagnostic molecular services on multiple test platforms, including massive parallel sequencing, to support patient care, and basic and clinical research. The Molecular Pathology Laboratory emphasises ancillary biomarker studies in small- and large-scale clinical trials.

In partnership with collaborators, the facility can design clinical-grade tumour-specific assays for tumour profiling, and prepare large numbers of samples for molecular assays through high-throughput automation. The Molecular Pathology Laboratory is supported by 8 pathologists and 35 molecular pathology scientists.

Australian New Zealand Clinical Trials Registry

National clinical trial registry, approved by the World Health Organization (WHO)

Established in 2005, the *ANZCTR* is an online registry of clinical trials in Australia and New Zealand, and internationally. The ANZCTR includes trials related to:

- pharmaceuticals
- surgical procedures
- preventive measures
- lifestyle/public health
- medical devices
- treatment and rehabilitation strategies
- complementary therapies.

WHO recognised the ANZCTR as a Primary Registry in the WHO Registry Network. The International Committee of Medical Journal Editors also recognises the ANZCTR.

Clinical trials can be registered free of charge through the ANZCTR website. Once a trial is registered, publicly available trials information can be searched and downloaded. Specific or tailored information searches can be requested by contacting the ANZCTR directly.

Melbourne EpiCentre

Trial support and planning, database design

The *Melbourne EpiCentre* is a new collaborative research centre of the University of Melbourne and Melbourne Health. Embedded within the Royal Melbourne Hospital, it is an academic centre of excellence that focuses on research, education, healthcare quality and safety, and organisation improvement. The goal of the centre is to promote and run high-quality clinical, nonclinical and biostatistical studies, locally and internationally.

The EpiCentre is leading several early- and late-phase clinical trials and clinical epidemiological projects with large primary and ambulatory care databases from the United States and the United Kingdom. The EpiCentre also provides expertise in the planning, design, data management, and statistical and clinical reporting of clinical trials, conforming to United States Food and Drug Administration requirements and industry standards.

Paediatric Trials Network Australia

Clinical trial support and planning

Paediatric Trials Network Australia (PTNA) draws together paediatric researchers from around Australia who are committed to improving child (from newborns to 18-year olds) health through paediatric clinical trials. PTNA focuses on multicentre paediatric studies developed by researchers or industry partners across all therapeutic areas. These may include randomised controlled trials studying drugs, devices or other health interventions, as well as observational and health services research.

PTNA is a not-for-profit, virtual and inclusive network that is open for membership to any paediatric research organisation. Its infrastructure, advocacy and collaboration efforts complement other paediatric research networks, and benefit all paediatric researchers and, ultimately, Australian children. PTNA welcomes enquiries from any researchers who wish to collaborate through PTNA and access its infrastructure.

The team at Compounds Australia provide compound management and logistics to the Australian research community.

Photo credit: Griffith University

Our consortium outcomes

Our brief

In 2011, the Australian Government tasked TIA with addressing four key objectives through investment via the Translating Health Discoveries project:

- to **accelerate translation** of research discoveries and enhance translational efficiency
- to **coordinate access** to integrated facilitation pathways for researchers in different areas including small molecule pharmaceuticals, biopharmaceuticals, devices, biomarkers and cell-based therapies
- to **maximise leverage** of government funding through integration into a national translational research framework (the Pipeline model)
- to address existing **infrastructure gaps**.

TIA consortium outcomes

THE BRIEF

The objective of Therapeutic Innovation Australia is to deliver the Department of Education's Translating Health Discovery (THD) subproject as part of the National Collaborative Research Infrastructure Strategy (NCRIS).
The objectives of the THD subproject are:

To **accelerate translation** of research discoveries and enhance translational efficiency

To **coordinate access** to integrated facilitation pathways for researchers in different areas including small molecule pharmaceuticals, biopharmaceuticals, devices, biomarkers and cell-based therapies

To **maximise leverage** of Australian Government funding through integration into a national translational research framework

To address existing **infrastructure gaps**

• Funded facilities achievements 2015–17

37 37 patents, 24 licences

24

67 Worked on 67 therapeutic products

500 Accessed by up to 500 researchers per year working on up to 1100 projects

1100

• **National educational seminar series on commercialisation**

• **Supported meritorious projects**
Cell therapies/researcher access
$3m, 45 projects, 26 trials

• **Funding allocation via merit-based process**

• **Launched a national Therapeutic Pipeline initiative**

• **Engaged foremost experts in Australia to provide advice through expert committees**

AUSTRALIAN THERAPEUTIC PIPELINE

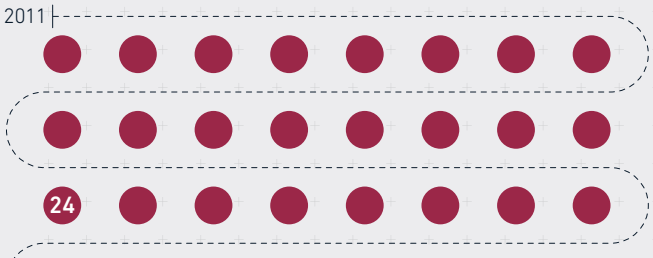
• **Co-investment of nearly**
5x NCRIS investment since 2011
($221m total co-investment on $45m)

• **Created supporting initiatives**

ATRAX
AUSTRALIAN TRANSLATIONAL RESEARCH ACCESS
An evolving snapshot of funded medical research projects in progress

iQDOCs
QUALITY DOCUMENTS
A unique collection of over 500 ready-to-use template documents for supporting quality systems

• **Developed quality systems for several cell therapy facilities**



• **Since 2011, supported 24 different facilities**

• **Collected critical new data including:**

– Mapping domestic supply chains and health and medical research sector

– Surveyed Australian translational health researchers to evaluate understanding of translational science

Accelerating translation of research

From 2015 to 2017, we have supported 500 researchers across 1000 facilities, resulting in 37 patents, 24 licences, 67 therapeutic products and 26 clinical trials. In addition, TIA ran a national educational seminar series on commercialisation.

Highlight: Enabling a clinical trial for a new cell therapy

Cell & Tissue Therapies WA (CTTWA) is enabling a clinical study of patients with myelodysplastic syndrome, a haematological disorder of bone marrow failure. The study will contribute to delivery of a cell therapy that will avoid the side effects typical of other pharmaceutical agents.

As a clinical researcher, I have been very fortunate to be able to access the Cell & Tissue Therapies facility at Royal Perth Hospital, which has facilitated my study. I have great confidence in the quality of the cell therapy since it has been manufactured by CTTWA in compliance with Australian Government regulations and under TGA licence, thus guaranteeing its safety.

Dr Melita Cirillo,
MBBS(Hons), FRACP, FRCPA
Department of Haematology
Royal Perth Hospital

Coordinating access to pathways

TIA provides researchers with access to investigative pathways that accelerate translation. This is primarily done by:

- allocation of core funding (via a merit-based process) to enable subsidised access
- developing a national Pipeline initiative to increase connectivity between Pipeline members
- engaging foremost experts in Australia to provide advice through expert committees.

Leveraging Australian Government funding

TIA aims to maximise government funding by leveraging additional funding from nongovernment sources. Since 2011, we have secured an additional $166 million – a fivefold co-investment – on the initial $45 million from NCRIS.

Highlight: Multimillion dollar deal for children's cancer research

The Australian Cancer Research Fund (ACRF) Drug Discovery Centre was able to leverage TIA funding to gain $20 million from the Australian Government to go towards the national implementation of the Zero Childhood Cancer Personalised Medicine Program. Some of the funding will be used to purchase an automated workstation that will be integrated with a pre-existing high-content imager to image childhood cancer cells.

The Zero Childhood Cancer Personalised Medicine Program is an ambitious Australian initiative that aims to see childhood cancer survival rates of 100%. Each child's unique cancer cells will be studied carefully, to help identify the drugs most likely to destroy their cancer. Scientists and doctors will then work together to identify and deliver a tailored treatment plan that offers the child the best hope of defeating the disease.

Addressing infrastructure gaps

NCRIS funding managed by TIA has helped Australia address infrastructure gaps. Our initial studies identified and mapped current translation supply chains and researcher needs in Australia. We discovered that researchers have limited access to the translational facilities they need; however, they also lack a comprehensive understanding of the commercialisation process. Thus, we have provided access to knowledge through expert committees and seminars on commercialisation.

Industry engagement

TIA's consortium members are outwardly focused and engage strongly with industry. However, the commercial-in-confidence nature of the highly competitive process of therapeutic development acts against the timely promotion of successful commercial outcomes. However, anonymised data from the consortium show more than three dozen patents and two dozen licences resulting from projects that have accessed the consortium's capabilities.

TIA's supporting Pipeline initiatives are also strongly aligned with supporting industry engagement. The Pipeline Accelerator scheme will lower the financial bar for industry and small-to-medium enterprises (SMEs) to access the high-quality capabilities provided by the Pipeline. The Pipeline Navigator online mapping tool will decode the public sector translational research landscape by providing an innovative web-based tool to identify the right capability based on a customer's specific requirements, such as therapeutic modality and quality accreditation status.

TIA is seeking to increase industry engagement, primarily via the Australian Government's $250 million Industry Growth Centre program, and specifically via MTPConnect. The mission of MTPConnect is to accelerate the rate of growth of the medical technologies, biotechnologies and pharmaceuticals sector to achieve greater commercialisation, and establish Australia as an Asia–Pacific hub for medtech and pharma (MTP) companies. MTPConnect acts as an independent voice that enables and facilitates collaboration within the sector. TIA considers MTPConnect to be an ideal partner and will continue to foster closer collaboration.

Our supporting initiatives

Engagement with key industry bodies is also critical. During the 2016–17 financial year, TIA joined AusBiotech, Australia's peak body for biotechnology companies and related organisations. This will enable increased marketing of the Pipeline and its capabilities, including promotion through AusBiotech's publications.

TIA has put in place a suite of initiatives and activities aimed at helping public sector and private sector researchers to translate their scientific discoveries to commercial therapies. TIA primarily achieves this by investing in a consortium of capabilities (see Our consortium and its capabilities) that provide translational services to research groups, SMEs, large pharmaceutical companies and international partners.

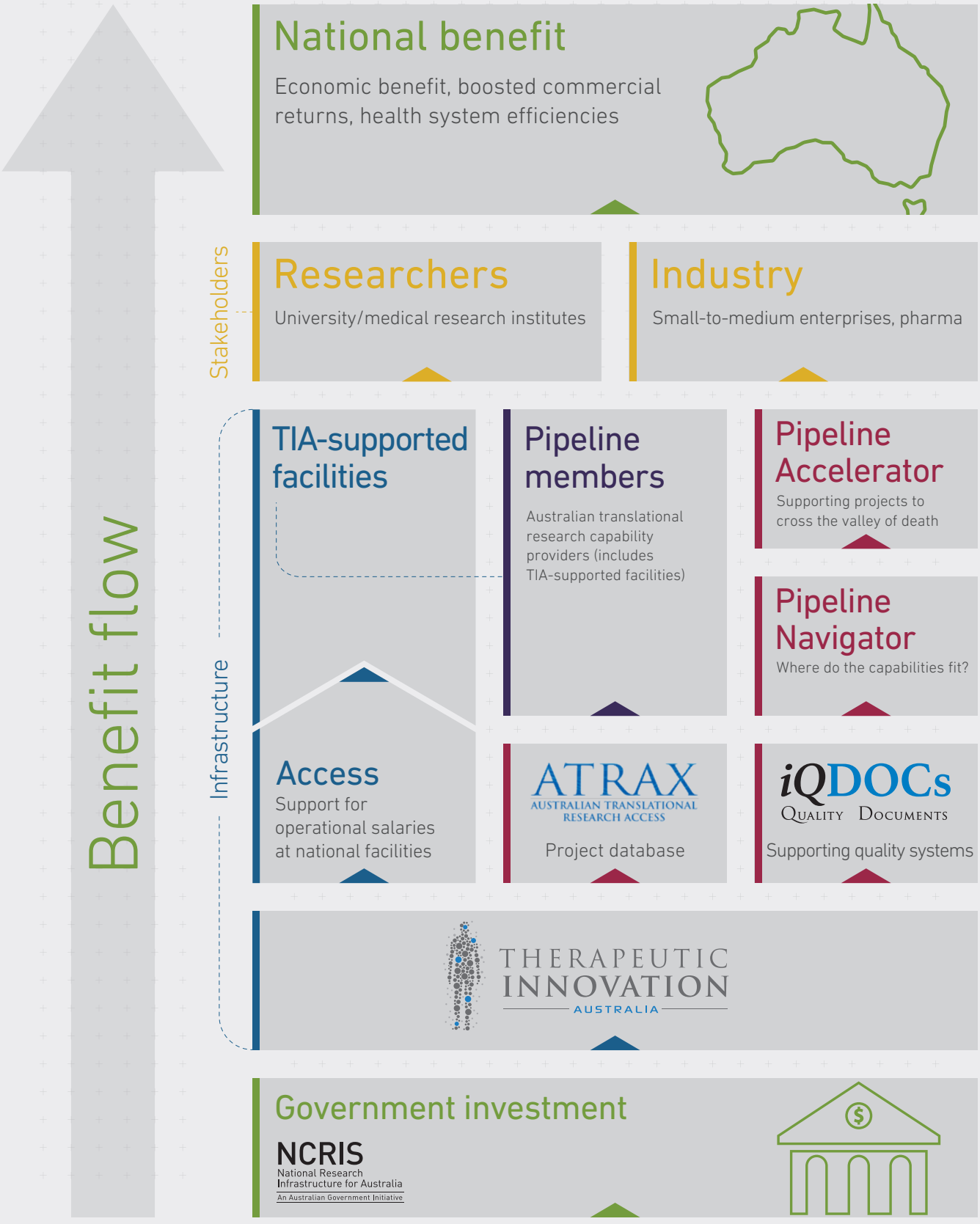
However, this is by no means the whole story. Since its inception, TIA has sought to develop innovative programs to support the translational research community. These initiatives, and their potential future development, are outlined in this chapter (also see page 17).

The National Biologics Facility is a critical part of the response to the deadly Hendra virus.

Photo credit: University of Qld

Benefit from TIA-led initiatives

Industry, researchers, consortium facilities and Pipeline members benefit from TIA-led initiatives



Australian Therapeutic Pipeline

The 2016–17 reporting period saw a renewal of the Australian Therapeutic Pipeline model to better articulate the value proposition of membership. TIA strives to use the Pipeline as a mechanism to foster collaborations among capabilities and between NCRIS projects. Through the Pipeline, TIA can refer research projects to the relevant capabilities. In addition, the Pipeline has significant potentially synergies with MTPConnect, the sector's relevant industry growth centre, as it strives to achieve its target outcomes set by the Australian Government.

The benefits of Pipeline membership are:

- inclusion in a community of practice of translational research facilities
- opportunities to raise a facility's profile within and outside the group – for example, through promotional activities
- inclusion and profiling in the Pipeline Navigator web app (in development for NCRIS 2017)
- unlimited document download from iQDOCs
- free access to ATRAX
- opportunities to benefit from the Pipeline Accelerator researcher access scheme (in development for NCRIS 2017).

Highlight: Pipeline connections

The Melbourne EpiCentre, led by Professor Sanjoy Paul, is a TIA-supported facility that provides services in clinical trial database design and statistics. The leadership of the EpiCentre is working with BioGrid Australia (a member of the Australian Therapeutic Pipeline) and other stakeholder groups in the much-needed development and integration of patient-level electronic medical records at primary and ambulatory care levels in Australia.

BioGrid links real-time de-identified health data across institutions, jurisdictions and diseases to help researchers and clinicians improve their research and clinical outcomes. The web-based infrastructure provides ethical access, while protecting both privacy and intellectual property.

In early 2017, under the leadership of new Chief Executive Officer Dr Stuart Newman, work began on two new initiatives: **Pipeline Accelerator** and **Pipeline Navigator**. These are designed to:

- address the connectivity gap in translational research
- promote the capabilities within the TIA and Pipeline consortia
- facilitate access to capabilities by SMEs and researcher groups.

Both initiatives are expected to be made available to stakeholders at the end of 2017.

Pipeline Accelerator

TIA is developing a voucher-based infrastructure-access scheme that makes it more affordable for SMEs and research groups to access world-class infrastructure. An open call for projects would identify those who would gain subsidised access to at least one TIA-supported facility. This scheme will be able to respond quickly to the needs of researchers and industry by facilitating access to, and enabling a support mechanism for, the Australian Therapeutic Pipeline.

We expect this exciting support scheme to be launched during the 2017–18 project period.

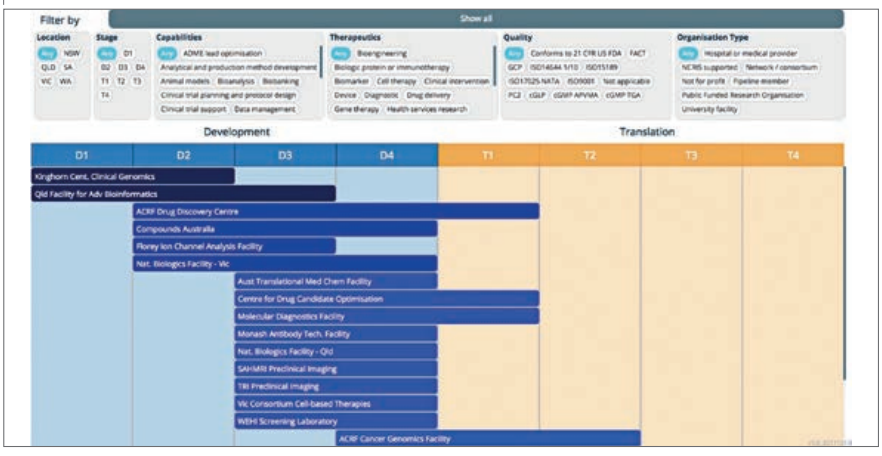
Pipeline Navigator

TIA is developing an interactive online map of the therapeutic development pathway, represented graphically as a pipeline, encompassing discrete and widely acknowledged definitions of stages of translational research (i.e. discovery, T1, T2, T3 and T4). Each Pipeline member facility will be placed in the appropriate position on the pipeline.

To make sense of what will be a complex picture, facilities will be able to be filtered out according to user preferences, such as therapeutic modality, location or quality accreditation. This will produce a focused list of potential capability providers that is sent to the user, to help to plan a development strategy.

We expect this unique mapping tool to be launched during the 2017–18 project period.

Screenshot of the Pipeline Navigator tool

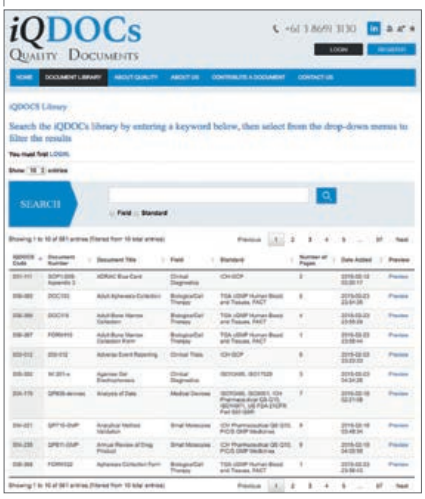


iQDOCs – International Quality Documents

For novel therapeutics to reach the clinic, the investigator must satisfy the safety and efficacy requirements of regulators. Much of the documentation used to meet these regulatory standards, particularly those that relate to facility or process compliance, is reproducible. Furthermore, the process of compliance is both time consuming and expensive, with creation of a full system often taking more than 12 months and $300,000.

To reduce the cost and time burden of creating documents from scratch to meet regulators' requirements, TIA established iQDOCs, a world-first open-access library of generic template documents. This library helps to ensure reliability and reproducibility of results, and supports laboratories to prepare for achieving relevant licensing and/or accreditations.

iQDOCs search page with new document preview link



Registrants can apply for access via the website, then select and download documents independently. The library comprises more than 560 generic documents, including templates for:

- calibration procedures
- contingency plans
- training modules
- process control
- material specifications
- position descriptions
- reporting forms.

All documents are in Microsoft Word or Excel formats (.doc, .docx, .xls, .xlsx). The documents relate to codes of Good Practice (GxP) and ISO/IEC codes. The documents in the library are sourced from a range of commercially and publicly funded organisations. During 2016–17, website functionality was upgraded to enhance the utility of, and access to, this unique resource:

- A 'preview' function was added to allow users to 'quick view' a document before committing to downloading it.
- The 10-document cap on downloads was removed for Pipeline members.

ATRAX – Australian Translational Research Access

ATRAX is a cloud-based relational database of active research projects. It aggregates public-domain information on research funding outcomes or commercialisation projects. ATRAX can identify new or in-progress projects that could access TIA facilities, as well as projects for industry partnerships. The data, which go beyond therapeutic development to include public health and health service research projects, represent a high-level overview of medical research in Australia.

TIA has continually updated ATRAX, which currently contains more than 3800 active Australian medical research projects. ATRAX reinforces and enables the Pipeline as a project identification and facilitation tool. ATRAX thus has the potential to make a strong contribution to the management of translational research in Australia.

ATRAX dashboard

During 2016¬–17, the following ATRAX-related development activities began:

- A subcontractor updated the database to include all 2016 calendar-year National Health and Medical Research Council (NHMRC) outcomes.
- A customer portal was added to the database, enabling controlled access by Pipeline members (for whom access is free).
- A student placement from Monash University was organised (starting in July 2017) to source and collate research funding outcomes for January–June 2017, to later enter into ATRAX.
- We started liaising with other NCRIS projects (**NeCTAR, Research Data Services**) on linking ATRAX records to persistent identifiers of researchers (ORCID) and their projects (Research Activity ID – RAID).
- We explored using ATRAX as a data source for the proposed Pipeline Navigator data visualisation web app.

ATRAX will continue to form an important part of the value proposition for membership and development of the Australian Therapeutic Pipeline.

Governance

TIA's structure

TIA is a not-for-profit limited liability company that manages NCRIS funding. TIA is overseen by a diverse and skills-based Board that has considerable experience and expertise in therapeutic discovery, development, commercialisation and regulation. The management team comprises a Chief Executive Officer (CEO), an office manager for administrative and financial support, and a part-time adviser for the Australian Therapeutic Pipeline. Additional expertise is provided by a series of expert committees that meet to provide advice on developments in a variety of fields.

During 2016–17, we said goodbye to CEO Dr Stewart Hay. Dr Hay departed TIA in September 2016 to pursue new commercial opportunities. TIA wishes him the very best in his future endeavours. We also said goodbye to Ellie Mohammadi, who left in early 2017. Ellie's contributions to TIA over the years have been significant, and we wish her the best. As noted earlier, Dr Stuart Newman re-joined TIA as CEO in April 2017.

At the very end of 2016–17, we welcomed Professor Ross McKinnon, the original architect of the THD project, to the Board. Ross returns to TIA at a pivotal moment in the project's history. His insight and knowledge will make a major contribution to the strategic development of the THD project.

Our Board

Mr Terry Slater
AM, BSc, BEc, MPH, MAICD, FAIM
Chair

Mr Slater was the CEO of Donate, the peak body of the organ and tissue transplant organisations in Australia. Terry was National Manager of the TGA for nine years and has consulted to many organisations in health services and semi-government bodies.

Ms Liz Furler
BA (Social Work), Member of the Public Health Association of Australia

Ms Furler has extensive experience in the health and education sectors. Previous positions include CEO of Principals Australia, Executive Director with the Department of Education and Children's Services in South Australia, Executive Director of TRACsa (South Australian Centre for Trauma and Injury Recovery), General Manager of the Australian Natural Therapists Association (Melbourne), CEO of the Royal Australian College of General Practitioners, First Assistant Secretary of the Health Services and Public Health divisions in the Australian Government Department of Health, and Principal Adviser to the Australian Government Department of Human Services and Health. Earlier positions included senior roles with state and Australian government health sectors.

Professor Ross McKinnon
BPharm (SAIT), BSc (Hons) (Flinders), PhD (Flinders)

Professor McKinnon is an academic pharmacist and pharmacologist with contributions spanning pharmaceutical research, policy and education. His research has attracted more than $26 million in funding, and produced more than 150 original papers, multiple patent families and spin-off companies. He was Inaugural Director of both the Sansom Institute (University of South Australia) and the Flinders Centre for Innovation in Cancer (Flinders University). He is currently Dean of Research in the School of Medicine at Flinders University.

Ross provided the original vision for TIA as National Facilitator for the THD project. His international roles include Vice-President of the International Pharmaceutical Federation and Chair of the 5th Pharmaceutical Sciences World Congress held in Melbourne. He is a Director of the Australian Institute of Policy and Science, and was recently elected as a Fellow of the Australian Academy of Health and Medical Sciences.

Professor Judith Whitworth
AC, MBBS, MD, PhD, DSc(Melb), HonDSc(Glasgow), FRACP, FAICD, FTSE, FAHMS(hon), MD(hon), Sydney, MD(hon) UNSW, D Lett(hon) CDU, LLD(hon) Melb

Professor Whitworth is an Emeritus Professor at the Australian National University, where she is the past Director of the John Curtin School of Medical Research. She is a Fellow of the Australian Academy of Technological Sciences and Engineering, the Royal Australasian College of Physicians, and the Australian Institute of Company Directors.

Judith has chaired the Medical Research Committee of the NHMRC, and is a Past-President of the Australian Society for Medical Research and the High Blood Pressure Research Council of Australia. She is also an Honorary Life Member of the Australian and New Zealand Society of Nephrology.

Mr Rob Anderson
FCA, FAICD
Director and Company Secretary

Mr Anderson was the Managing Director of Orthogen Australia, which operated in the cell therapies sector. He is also principal of Anderson Business Consultants. Rob has significant experience consulting to government and industry. He has extensive commercial and financial experience in a diverse range of industries and business structures, including as Partner (Audit and Advisory) at Deloitte Touche Tohmatsu.

Our management

TIA's management includes skilled and qualified individuals with a range of skills and experience. The CEO splits his time between TIA's Melbourne office and a Brisbane office – kindly provided by the Queensland Department of Science, Information Technology & Innovation – at the EcoSciences Precinct at the site of the old Boggo Road Jail.

Dr Stuart Newman
Chief Executive Officer

Stuart brings with him a diverse range of skills and experience. Since completing a PhD in Antarctic biology at the University of Tasmania, he has built up considerable experience in science policy, pharmaceutical research and development, grant funding, intellectual property management, business development and commercialisation.

Stuart previously worked with TIA as Queensland Development Manager, where he helped to develop TIA's Queensland Node, and assisted in establishing the iQDOCs resource and the ATRAX database.

Associate Professor Stella Clark
Scientific Adviser to the Board and Chair, Australian Therapeutic Pipeline

Stella Clark joined TIA as Chair of the Australian Therapeutic Pipeline in 2016. She has unique expertise and genuine interest in facilitating scientific endeavours across organisations both public and private. Stella has worked at various executive levels and is currently working with TIA to develop the Pipeline.

TIA expert committees

TIA draws from a group of expert committees to provide advice on the latest developments in a variety of fields. The primary role of the expert committees was to meet on an ad hoc basis to advise the TIA Board about program investment priorities. Once these priorities were set in place and aligned with the original investment plan, the THD project was implemented.

These committees are listed right, with membership listed as of the most recent meeting.

Clinical Trials Infrastructure Committee

- Emeritus Professor Judith Whitworth (Chair)
- John Chalmers
- Rohan Hammett
- Omar Ali Khan
- John Simes
- Professor John Zalcberg

Cell & Gene Therapies Committee

- Professor Ian Gust (Chair)
- David De Kretser
- Rita McLachlan
- Roland Scollay
- Peter Turner
- Graeme Woodrow

Biomaterials, Biopharmaceuticals and Medical Devices Committee

- Professor Bryan Williams (Chair)
- Rob Crombie
- Rita Maclachlan
- Guy Maddern
- Andrew Maxwell
- Keith McLean
- Greg Roger

Virtual Pharma Network Committee

- Professor Bill Charman
- Joshua Funder
- Phil Kearney
- Jenny Marty
- Nick Nicola
- Michael Panaccio
- Craig Rogers
- Professor Maree Smith
- Dr Graeme Stevenson

Note: Previous Chair George Morstyn resigned from the TIA Board and chairmanship of this committee in 2016, with no interim chair named.

NCRIS Expert Advisory Committee

This committee advises the Board on the allocation of NCRIS soft infrastructure funding. Membership and composition of this committee are currently under review.

Queensland Node Strategic Committee

- Peter Andrews (Chair)
- Max Aitken
- Carrie Hillyard
- Mario Pennisi
- Maree Smith
- Zee Upton
- Liz Wickham

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NCRIS
National Research Infrastructure for Australia
An Australian Government Initiative

About TIA:

Therapeutic Innovation Australia (TIA) is the lead agent for the NCRIS Translating Health Discoveries project. We support access to research facilities that can accelerate translation of discoveries from the lab to the clinic. Our members provide a wide range of quality translational services in the fields of biologics, cell therapies, small molecules, preclinical testing and clinical trials. TIA works to encourage quality accreditation in Australian research labs through its www.iqdocs.org resource and connect these capabilities through its Australian Therapeutic Pipeline initiative.