NSW Advanced Therapeutics Ecosystem

Making opportunities happen in life sciences

NSW GOVERNMENT

Contents

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Opportunities happen in New South Wales

With Australia's largest population, strongest economy, and innovative life sciences sector, New South Wales (NSW) is open for business for companies looking to expand in Australia and the Asia-Pacific region.

An economic powerhouse in the Asia-Pacific region

Australia's best-performing economy and home to Sydney, its financial centre.

NSW accounts for more than half of Australia's economic growth.

The state's economy is larger than the individual economies of Singapore, Hong Kong and Malaysia. Our economic and political landscape is stable and secure.

A highly skilled and diverse workforce

NSW has Australia's largest population with 8.2 million residents, more than two-thirds of whom hold post-school qualifications. More than a third of Sydney's workforce holds a bachelor's degree or higher.

The state's population is also among the most diverse in the world. While English is by far the most widely spoken language, almost a third of NSW residents speak a second language. In all, there are 275 languages spoken within the state.

Australia has the fourth-highest proportion of highly educated immigrants in the OECD and is ranked 11 out of 134 countries in the 2021 Global Talent Competitiveness Index.

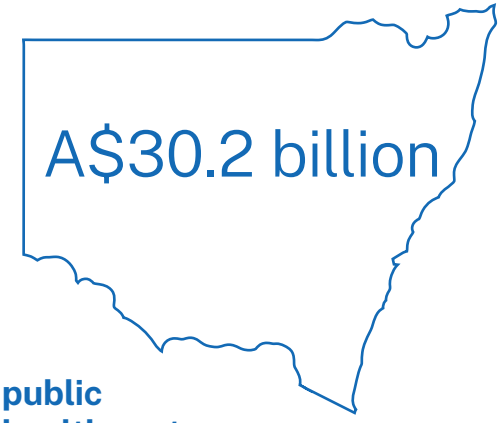
A sophisticated and highly networked health system

NSW has Australia's best-performing healthcare system, and a globally significant R&D ecosystem with strong government-backing.

NSW has an advanced and interconnected research and commercialisation ecosystem developing the next generation of therapies, supported by internationally recognised health and education sectors.

World-class healthcare is delivered to NSW's 8.2 million residents through a unique devolved structure of governance which includes 15 local health districts and 2 speciality health networks, supported by centralised policy and support pillars.

Precincts are a strength of NSW's health system. Across the state there are globally significant networked concentrations of health, research, and education facilities.



public health system annual budget

228 **public hospitals** serve over 8 million people

3rd ★★★★★ most efficient healthcare system **in the world**

(The Commonwealth Fund, 2021)

Over **127,000** **full-time equivalent** staff

Statewide public pathology service, and **Australia's first and largest Statewide Biobank**

Over **350,000** **surgeries** were performed during 2020–21

3 million **emergency department attendances** were triaged during 2020–21

World leading health and medical research ecosystem

A$10.8 billion

capital investment by the NSW Government over four years to 2024–25

NSW is home to **55%** of Australia's life sciences companies and **44%** of its start-ups

Two of the world's **top 50** universities

(QS 2022)

8th in the world for life sciences research

(Nature, 2020)

CASE STUDY

A world-class public health response to the COVID-19 pandemic

The management of COVID-19 in Australia is viewed by some international bodies as one of the best in the world. With a population of just over 25 million people, Australia recorded 10,112,229 cases and 14,421 deaths to 9 September 2022. In 2020, Australia did not experience overall excess death, and in 2021 the annual excess death rate was 3.5%, a difference of around 5,000 deaths, or around 100 deaths above the expected statistical variation in the number of deaths each year. This effective management of the pandemic was the result of strong national collaboration and the rapid implementation of policy based on available evidence and pragmatism.

As NSW Health responded to the challenges of COVID-19, it also optimised its close relationships with culturally diverse and at-risk communities at a local health district level, engaging with community leaders to increase awareness of the risks of infection, encouraging testing and highlighting the benefits of vaccination.

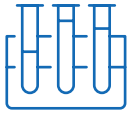
The NSW Government has committed more than A$4 billion to the NSW health system to manage the impacts of the COVID-19 pandemic since March 2020. This includes an investment of A$458.5 million by the NSW Government over 2020–21, with a further A$80 million in the 2021–22 budget, to speed up access to surgery for patients who had their surgery delayed. This was made possible through increased collaboration between public hospitals and private providers.

Support and harness health and medical research and innovation

Significant financial investment by the NSW Government ensures internationally recognised research is translated into therapies and practice both locally and globally.

A dedicated Office for Health and Medical Research, part of NSW Health, supports research, translation, and commercialisation through a range of programs:

Translational Research Grants Scheme (TRGS) which builds research capability and accelerates evidence translation within the NSW public health system. Over **A$35.8 million** has been funded over five rounds in TRGS.



Over **A$25 million** in COVID-19 research to inform the NSW response to the COVID-19 pandemic, enhance **research ecosystem and infrastructure** and translate COVID-19 research.

Medical Devices Fund, a seed funding program for new-to-world medical devices. Since 2009, the Fund has awarded **A$70 million** for 40 projects which has generated **A$870 million** in capital, 70 regulatory product approvals and over 200 completed clinical trials.

Commercialisation Training Program which develops the commercialisation skills of innovators in medical devices, diagnostics, therapeutics, and digital health. Graduates of the program have raised more than **A$77 million** in private equity and grants and founded 19 start-ups.

A$150 million over 10 years to build **cardiovascular research capacity** in NSW and make NSW a global leader in cardiovascular research.

Next-generation clinical trials

NSW is a global premier destination for high-quality, efficient, and cost-effective clinical trials.

The state's natural strengths include a large, diverse, and talented population complemented by world class research institutions, universities, and a highly networked public health system.

In addition, capabilities, policies, and services including start-up support, resource-sharing, and streamlined ethics and governance processes, have earned NSW a reputation as a globally competitive destination for clinical trials; particularly in early-stage trials.

- NSW clinical trial start-up is highly efficient, with more than 90 percent of clinical trials approved within 60 days of application, and contracts signed within 30 days.
- NSW benefits from an ethnically and culturally diverse population, with 33 percent of its residents born overseas, and up to 47 percent with at least one overseas born parent.
- Clinical trials can access the entire NSW public health system and patient population with a single ethics application which is also recognised nationally.
- A large and competitive domestic contract research organisation (CRO) sector that is highly experienced with managing trials for overseas companies.
- Access to patient populations through high-quality clinical trial sites in regional and rural NSW, capable of delivering decentralised clinical trials, supported by a A$30.6 million Commonwealth Medical Research Future Fund Grant.

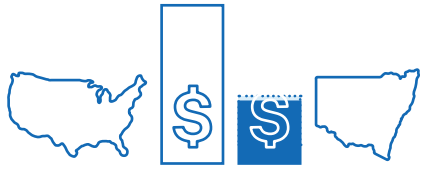
Specialist **expedited** ethics reviews of **early phase** clinical trials within

just 20 days

NSW has a large recruitable population, with

56%

of its **8.2 million residents** willing to participate in a clinical trial



Trials in NSW are

28%

less expensive than in the US

Increasing up to

60%

with federal R&D **tax incentives**

A personalised concierge service for clinical trials

clinicaltrialsNSW within NSW Health, enables capacity, capability, and collaboration across clinical trials.

Its **Clinical Trials Connect** service assists industry and researchers in establishing clinical trials in NSW in a range of ways including, by:

- finding trial investigators and engaging key opinion leaders
- identifying potential patient populations
- finding specialist trial support services including CROs, biostatistics, etc.

Contact

clinicaltrialsNSW@health.nsw.gov.au to run a high quality, fast, and cost competitive trial in NSW.

Next-generation clinical trials

CASE STUDY

NSW stands out in global trial of new gene therapy for spinal muscular atrophy

The Sydney Children's Hospitals Network (SCHN) was the only Australian site in an international clinical trial for a new gene therapy treating spinal muscular atrophy (SMA), the leading cause of infant death in Australia.

Australian participation in the trial was supported by a A$2 million investment from the NSW Government to add SMA to the NSW and the Australian Capital Territory's (ACT) newborn screening program run by SCHN. The NSW newborn screening program has near universal population coverage, screening around 100,000 newborns per year, and is an important way to identify pre-symptomatic infants before the onset of irreversible nerve death.

As a result of the early identification of newborns with SMA, NSW patients were able to join the global SPR1NT trial at the Sydney Children's Hospital, Randwick (part of the SCHN) for the new gene therapy called Zolgensma®.

NSW was the equal leading recruitment site globally, and the first international site to recruit children outside of North America. The trial led to the first market-approved adeno-associated virus-based gene therapy drug for paediatric patients.

Genomic and precision medicine

NSW is a global leader in precision medicine for cancer diagnosis and management, cardiac disease, and cystic fibrosis.

NSW has internationally significant expertise in the investigation of proteomics, microbiomics for personalised medicine approaches, and genomic medicine.

Researchers across the state are expanding the use of precision medicine across diverse clinical areas, revolutionising the understanding and treatment of disease in Australia and worldwide.

Genomic and precision medicine is supported by complementary state government investments in clinical trials infrastructure, NSW Health Pathology (Australia's largest public pathology network), and the Statewide Biobank.

The Australian Cancer Research Foundation International Centre for the Proteome of Human Cancer (ProCan®)

ProCan®, which is part of the US Cancer Moonshot and European iPC consortiums, is analysing the proteomes of tens of thousands of cancers for which clinical outcomes are already known in order to create a database that will enable cancer clinicians to enhance the accuracy of their treatment decisions. ProCan® has developed robust, high-throughput techniques that include rapid preparative methodologies and liquid Chromatography/tandem Mass Spectrometry suitable for analysis of very small cancer samples.

The multidisciplinary ProCan® team, based at Children's Medical Research Institute in Westmead, Sydney, contains proteomicists, data scientists, software engineers, project managers, histopathology technicians, and clinically-qualified medical oncologists.

ProCan® collaborates with large numbers of cancer research groups located primarily in Australia, Europe and North America who supply cohorts of tumour samples and associated clinico-pathologic data as well as other 'omic' data. The ProCan® team uses advanced computational techniques to enable prediction of clinical outcomes from proteomic data.

Precision Oncology Screening Platform Enabling Clinical Trials (PrOSPeCT)

The PrOSPeCT national project is led by the NSW-based Australian Genomic Cancer Medicine Centre (Omico) and backed by A$185 million in funding from the Australian Government, not-for-profits, and industry. The project will fast-track the development, manufacturing, and use of precision, personalised cancer treatments. This national genomic and proteomic platform that will screen more than 20,000 trial-eligible cancer patients between 2022 and 2025. It is designed to support industry-sponsored biomarker-dependent drug development and welcomes industry partners.

The Microbiome Research Centre (MRC)

The MRC at the University of New South Wales is a comprehensive world-class microbiome-focused research centre solely dedicated to studying the microbiota in health and disease; supported by grants totalling A$2.5 million from the NSW Government. Its researchers integrate basic science and translational medicine, and harness powerful multi-omic approaches such as genomics, epigenomics, transcriptomics, proteomics, metabolomics, as well as bioinformatics; to answer pertinent questions about the microbiome that are relevant to human health.

Genomic and precision medicine

> “Through PrOSPeCT, we will fast-track the development, manufacturing and use of precision, personalised cancer treatments, changing lives, creating jobs and building Australia’s sovereign capability in drug development.”

Professor David Thomas
Head of Genomic Cancer Medicine at the Garvan Institute and CEO of the Australian Genomic Cancer Medicine Centre (Omico)

CASE STUDY

NSW leads national platform strengthening precision medicine in oncology

Omico is an NSW-led national precision oncology platform which accelerates biomarker-dependent clinical trials. The platform brings together 23 of Australia’s major cancer centres, leading research institutes, Australian and state governments, industry partners and patients. It is led from The Kinghorn Cancer Centre, a joint facility of the Garvan Institute of Medical Research and St Vincent’s Hospital, in Sydney, NSW.

Omico is developing an innovative suite of clinical trials that match therapies to individuals based on the genomic information of their tumour. This is offered to patients with advanced cancers who have exhausted other treatment options. Of the more than 3000 patients who have undergone screening to date, 62 percent have had new treatment options identified.

Partnerships with industry have been critical to Omico’s success. It has connected with more than 35 partners across pharma and biotech who have provided proprietary anti-cancer therapies for trial participants. As a result, clinicians leading each trial have access to a wide range of treatment options for patients based on their tumour’s genomic information.

Genomic and precision medicine

CASE STUDY

Personalised treatment improves outcomes for children with cancer

The Zero Childhood Cancer Program (ZERO) is a world-leading personalised medicine program led by the Children's Cancer Institute and Kids Cancer Centre at Sydney Children's Hospital, Randwick. ZERO established a framework for recommending precision and personalised therapy for all Australian children diagnosed with high-risk malignancies (less than 30 percent chance of survival) or who relapse on standard therapy or have rare or undiagnosable tumours.

To date, ZERO has enrolled close to 800 children with high-risk cancers on its national clinical trial. For almost all children, the genomic basis of the disease has been identified, and personalised treatment recommendations have been made for over 70% of these children. Of the first 250 children enrolled on ZERO who received their recommended personalised treatment, 70 percent had a complete remission, a partial remission, or their tumour was stabilised. Over the next three years, ZERO will expand more than six-fold to be available to all children in Australian diagnosed with cancer, connecting them to a global network of information/data sharing and knowledge transfer.

ZERO is generating comprehensive, internationally unique datasets and tumour tissue resources that may hold the key to improving long-term health outcomes for children with cancer. The program has developed cutting-edge advanced capabilities in child cancer preclinical modelling, liquid biopsy, computational biology, and drug discovery and development. Industry partnership is welcomed at many stages to effectively and efficiently find new drug targets, develop new therapeutics and repurpose existing medications, test efficacy of new agents in paediatric cancer models and improve disease detection and monitoring.

Gene and gene-modified cell therapy

NSW is a global centre of excellence for viral vector development and for gene and cell therapy research and clinical delivery.

The state has a complete gene therapy pipeline spanning design, construction, and testing of gene therapies through to commercial-scale production and patient treatment. Research teams work collaboratively to exploit synergies across different technologies, including gene editing technology that combines viral vector and RNA capabilities. There is also shared translational support, such as the statewide NSW Health Pathology genomics service which is Australia's largest public pathology provider.

NSW has sponsored research and licensing agreements with European and US leading biopharma companies. NSW is also driving the use of new therapeutic tools by taking advantage of existing advanced manufacturing infrastructure and health system readiness.

The NSW Government has established a research and clinical delivery program in cell therapies, including CAR T-cell therapies and antigen-specific immune effector cells for pathogens and cancer.

CASE STUDY

Multi-million-dollar government investment in the region's first Viral Vector Manufacturing Facility

This project is the first GMP-grade clinical and commercial viral vector manufacturing facility in the Southeast Asia–Pacific region. The new facility at the Westmead Health and Innovation District will ensure a reliable and reasonably priced domestic viral vector supply for use in cell and gene therapy development and delivery. This will remove the bottleneck in taking these therapies from bench to bedside due to a global shortage in manufacturing capacity and exponentially increasing demand.

The Stage 1 'pilot' facility is manufacturing clinical-grade viral vectors on site and working towards GMP accreditation in early 2023. It currently supports a small number of local clinical trials for advanced therapeutics. Stage 2 of the project, currently in planning, will see the 25L viral vector pilot facility expanded to a commercial-scale viral vector manufacturing facility with a 550L capacity.

The viral vector manufacturing facility is a unique opportunity for industry to co-invest with the NSW Government in world-leading technology. There are partnership opportunities at all stages of the viral vector manufacturing process, including logistics, raw material supplies and manufacturing, process development, health data sciences and software engineering.

Gene and gene-modified cell therapy

> The rapidly advancing gene therapy revolution offers tremendous opportunities for Australia and the world. NSW is at the global forefront in some areas and is ready to use its expertise to bring health benefits to society."

Professor Ian Alexander
Head of the Gene Therapy Research Unit, Sydney Children's Hospitals Network and Children's Medical Research Institute

CASE STUDY

NSW Ocular Gene and Cell Therapies Australia team among first in the world to administer revolutionary gene replacement therapy

LUXTERNA® is the world's first approved gene replacement therapy for an inherited blinding eye condition, and one of the first gene replacements for any human disease. Two Sydney siblings diagnosed with *Leber congenital amaurosis*, a severe form of retinal dystrophy, were among the first in the world to receive the gene therapy in late 2020 and early 2021.

LUXTERNA® is injected under the retina and carries a functioning RPE65 gene to replace the patient's faulty one (which causes a range of symptoms including total blindness), relieving some of the devastating symptoms. Since receiving this gene replacement therapy, the siblings had profound improvements in their vision which mirrored clinical trial results.

The therapy was delivered through Ocular Gene and Cell Therapies Australia; a collaboration involving the Genetic Eye Clinic and other teams at Sydney Children's Hospitals Network, the Eye Genetics Research Unit and Stem Cell Medicine Group at CMRI, and the Save Sight Institute at Sydney Eye Hospital and University of Sydney.

RNA therapeutics and diagnostics

NSW is a global centre of excellence for RNA therapeutics and diagnostics.

As a result of the NSW Government's multi-million-dollar investments, NSW is an international leader in RNA therapeutics and diagnostics, with capability to research, develop, and manufacture RNA-based treatments locally.

The state's world-class RNA ecosystem consists of specialised research facilities and a collaborative network across universities and medical research institutes. The NSW Government, in partnership with the NSW RNA Bioscience Alliance, is investing A$96 million to develop an RNA pilot manufacturing facility.

The NSW Government will accelerate the translation of local RNA research into new products, services and jobs through a $119 million investment in R&D over 10 years in the 2022-23 NSW Budget.

With capabilities in RNA therapeutics including siRNA, circRNA, CRISPR gene editing and other mRNA – and strengths in cancer treatment, immunotherapy, genetic disorders, diagnostics, and drug delivery systems – NSW is at the forefront of the RNA revolution.

NSW RNA Bioscience Alliance

A collaborative alliance of all universities in NSW and the ACT working with the NSW Government to boost the development of RNA research. The Alliance coordinates and leverages science, engineering and medical research, creating a central point for expertise and training.

The University of New South Wales RNA Institute

Australia's leading RNA-focussed research, development and translational Institute was established with a A$25 million investment to grow Australia's RNA-based therapeutics industry. Leveraging world class research in Chemistry, Engineering, Biology and Medicine, combined with a certified pre-clinical production facility and leading capabilities in targeted delivery and analytics, the Institute aims to overcome key challenges in the field of RNA therapeutics from fundamental science to new manufacturing methods.

NSW RNA Production Research Network

Launched with a A$15 million investment by the NSW Government, this collaborative network of five universities and seven medical research institutes is conducting three pilot research projects to develop RNA-based therapeutic solutions that will assist the COVID-19 response and treatment of other diseases:

1. Nasal & lung delivery
2. siRNA for viral infections
3. mRNA and viral vectors.

RNA therapeutics and diagnostics

> Through a combination of talented researchers, world-class research institutions, and significant state government capital investment, NSW's domestic RNA therapeutics pipeline, from bench to bedside, is truly world-class."

Professor Pall Thordarson
Director of the UNSW RNA Institute

CASE STUDY

RNA pilot manufacturing facility

This GMP-grade facility will be a significant foundational milestone for the strong RNA R&D ecosystem in NSW that supports the translation of fundamental research through clinical trials to commercial outcomes.

The NSW Government has invested A$96 million to build this first-of-its-kind facility that will allow industry and researchers to develop RNA diagnostics, therapeutics and vaccines, supported by an $119 million investment in R&D to support the translation of local RNA research. Whilst initially facilitating the development of sRNA and mRNA, the facility will have adaptability as a central tenant, allowing it to pivot to new RNA-based therapeutic advances.

The pilot facility will include laboratories and pre-clinical trial spaces available for industry and researchers to translate successful pre-clinical projects into therapeutic formulations for clinical trials. The activities of the NSW RNA pilot manufacturing facility will be supported by the robust clinical trial framework in NSW and world-leading universities and medical research institutes.

Phage therapy

Phages are viruses that kill bacteria and, unlike antibiotics, are unaffected by resistance. Phage therapy has potential applications across health, agriculture, and biodefence.

The recently established Phage Australia network, of which NSW Health is a partner, is focused on the rapid translation of phage therapeutics into clinical practice including being first in the world to establish a national open label clinical trial with standardised treatment protocols and data collection.

This network of phage researchers and clinician scientists led out of NSW will establish phage therapy as the third major intervention for infectious diseases, after vaccines and antibiotics. This collaboration will build a national industry ecosystem of genomics and informatics, diagnostics, clinical trials, manufacturing, and internationally networked biobanks.

> “Phage therapy is both our last resort for dealing with antibiotic resistance now, and our best hope for the future. Phage Australia, with the support of NSW Health as a valuable partner, is playing a leading international role in developing safe phage treatments for serious infections.”

Professor Jon Iredell
Director, Phage Australia

CASE STUDY

NSW Hospital successfully treats girl using intravenous phage therapy

In an Australian-first, clinical teams across The Children’s Hospital at Westmead, in collaboration with colleagues from the Westmead Institute for Medical Research (WIMR), successfully treated a seven-year-old girl using intravenous phage therapy for a longstanding bone and joint infection. The patient suffered from a severe bone infection in the leg and foot, and the initial treatment with antibiotics failed to treat the aggressive and highly resistant bacterial infection. With limited antibiotic options left, amputation was the only remaining treatment option if the infection did not resolve.

NSW researchers acquired a suitable phage on compassionate grounds and administered the therapy to the patient. After a two-week dosing regime in conjunction with long-term antibiotic treatment, the young girl’s infection resolved. She did not require limb amputation, and long-term follow-up has demonstrated radiological and mobility improvement.

Phage therapy

CASE STUDY

NSW researchers demonstrate safety of phage therapy in patient

An NSW research collaboration between WIMR, and the University of Sydney led by Professor Jon Iredell administered adjunctive phage therapy to 13 patients with severe *Staphylococcus aureus* infections. The phages were produced under GMP conditions to ensure their quality as therapeutic products. The patients tolerated the therapy well and did not show any signs of adverse reaction from the phage therapy. This was the first time that research had demonstrated the safety and tolerability of GMP-quality IV-administered phage therapy in people with severe *Staphylococcus aureus* infections.

For more information

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