A commercial-scale viral vector manufacturing facility

How do viral vectors save and improve lives?

"This facility will produce clinical grade viral vectors in NSW, significantly cutting waiting times for patients across Australia to give them the best health outcomes."

–Brad Hazzard

NSW Minister for Health and Medical Research

The NSW Government has committed to expand and operate a world-leading viral vector manufacturing facility in the Westmead Health and Innovation District in western Sydney. The first of its kind in Australia, the facility will meet the growing demand in Australia and internationally for clinical-grade viral vectors, which are a key component of gene therapies and vaccines.

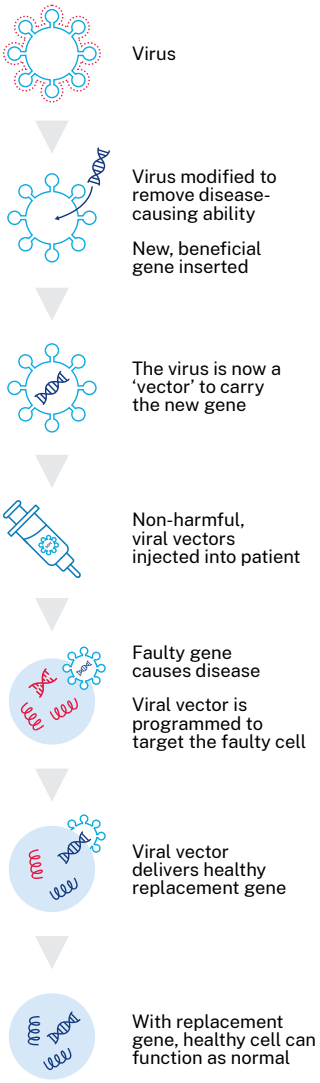
Who can viral vectors help?

Viral vectors can help a broad range of patients. They can be used to treat cancer and various genetic diseases throughout the body and play an important role in vaccine development. Patients with cancer can benefit from viral vectors, as they can deliver genes that reprogram the patient's immune system to fight cancerous cells. Viral vectors also can deliver other genes directly into cancer cells that instruct them to self-destruct. Viral vector vaccines teach the body's immune system how to respond to a foreign antigen. Recent viral vector vaccines have included the AstraZeneca COVID-19 vaccine.

What is a viral vector?

Viral vectors are an important tool used to make gene therapies. Gene therapy relies on a carrier (vector) to deliver the beneficial genes into the body's cells. Viruses, when modified to remove their disease-causing ability, can safely be used as vectors to inject cells with beneficial genes. Viruses are ideal vectors to carry information to our cells because they are naturally able to target specific cells. Viral vectors can be programmed to deliver genes to a wide range of cell types, and therefore can treat many diseases affecting a range of different organs.

What are viral vectors?



Case study

Globally recognised expertise

Genetic diseases affect eight percent of Australians, around 2 million people. There are 7,000 rare diseases that are chronically debilitating or life threatening. Many of these do not have a cure yet. Viral vectors are a way of delivering advanced therapeutic cures for such diseases. Producing viral vectors in Australia through the new facility means our researchers have faster access to the technology they need to develop these cures.

NSW has many highly regarded and experienced medical professionals and researchers that are developing viral-vector based therapeutics. The therapies span medical fields including ocular medicine, neurology, haematology, cardiology and immunology. Producing viral vectors in Westmead means NSW's experts have the technology they need to run more clinical trials and reduce the therapy wait times for patients.

Benefits to Australian patients through clinical trials

Many cell and gene therapies that use viral vectors are still in the clinical trial stages. Despite viral vectors not yet being used in products on the market, having the capability to produce enough viral vectors to run clinical trials offers a significant advantage to Australians. Participation in clinical trials is free for eligible patients and offers access to cutting edge therapies. While patients undergo clinical trials, they are given the best medical treatment and assessment by medical staff.

Demand for the viral vectors used in gene therapies is greatly outpacing supply. As technology progresses, more diseases will be treated using viral vectors, further increasing demand. Australian researchers currently wait up to two years to source the viral vectors needed for cell and gene therapy clinical trials. Depending on the disease, a single clinical trial could require a large supply of clinical grade viral vectors, which is why commercial-scale reliable production is necessary.

The expanded advanced manufacturing facility will enable large batches of viral vectors to be produced for use in clinical trials at Westmead. With more Australians potentially becoming eligible for gene therapy clinical trials, the NSW viral vector manufacturing facility will generate treatments to improve many Australian lives, and better prepare us for health and biosecurity threats in future.

"Viral vectors are essential in developing cures for genetic disorders and cancers. We've got the intellectual capital and expertise in a wide range of disciplines including ocular medicine, neurology, haematology, cardiology and immunology to be world leading."

Elizabeth Koff
Secretary of NSW Health

Case study

Advanced therapies for eye disorders

Professor Robyn Jamieson, leader of Ocular Gene and Cell Therapies Australia, is one of the experts at Sydney's Westmead Health and Innovation District. Her research is focused on creating new and innovative advanced therapies for ocular (eye) disorders. Viral vectors are critical to Professor Jamieson's research and are used by her team in developing therapies. The viral vectors are used to carry a functional gene into the affected eye tissue of the patient to enable the body to produce any proteins missing due to genetic mutation.

Professor Jamieson's research is world-leading and has resulted in direct patient benefits through tailoring their treatment options.

For example, two Sydney teenagers have received a novel gene therapy, recently approved by the Therapeutic Goods Administration, that restored their vision and may prevent them from going blind. The ocular gene therapy, LUXTURNA, treats an inherited blinding eye condition and is one of the first gene replacements for any human disease.

The life-changing therapy was part of Ocular Gene and Cell Therapies Australia (OGCTA), a new collaboration involving the Genetic Eye Clinic at Sydney Children's Hospitals Network (SCHN), the Eye Genetics Research Unit and Stem Cell Medicine Group at the Children's Medical Research Institute (CMRI), and the Save Sight Institute at Sydney Eye Hospital and the University of Sydney.

Professor Jamieson said the therapy was revolutionary and would lead to the transformation of care for patients with blinding eye

Professor Robyn Jamieson

Viral vector case study

Gene therapy clinical trial for babies with spinal muscular atrophy

The expansion of the facility to manufacture viral vectors on a commercial scale will enable more clinical trials to be conducted in NSW. This will reduce wait times and offers Australian patients access to the best health care.

The Westmead Health and Innovation District has been saving lives by leading the way in advanced therapeutics for years. In 2018, the Sydney Children's Hospitals Network was the only Australian site selected in an international clinical trial for a new spinal muscular atrophy (SMA) gene therapy.

SMA is a genetic neuromuscular disorder, similar to motor neurone disease, causing irreversible muscle weakness. Roughly one in every 10,000 babies has SMA, and most parents don't know they're carrying the gene until their child is born. Until recently, it was the leading genetic cause of infant death in Australia.

Viral vectors are an important tool in genetic therapies being developed to treat SMA. Viral vectors are used to deliver a functional version of a gene to correct or replace a faulty gene within specific cells in the body. These treatments show great promise and bring hope to families of children with SMA.

Developing a cure for SMA is only the beginning of what viral vectors can do. The number of diseases able to be treated through viral vectors is growing rapidly. Having the technology to run clinical trials is essential to developing new cures in Australia. The new NSW-based viral vector manufacturing facility will enable viral vectors to be produced locally to increase access to these tools for Australian clinical trials.

"The children receive one treatment and so far the data shows that they develop normally and lead normal lives"

–Dr Leszek Lisowski, Head of Translational Vectorology Unit, Children's Medical Research Institute.

NSW invested **$2 million** into

a newborn blood spot **screening program to detect the pre-symptomatic infants** at risk of developing SMA

offer them a chance at participating in a **clinical trial to cure SMA.**

From August 2018 to July 2020, there were **202,388 newborns screened.**

Eighteen were diagnosed with SMA, and of these, 14 newborns accessed therapy either through **innovative clinical trials or a global gene therapy managed access program.**

NSW GOVERNMENT

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