

24 May 2023

New funding to advance gene therapies

Researchers at the Centenary Institute and the Sydney Local Health District (SLHD) have been awarded \$500,000 from CUREator, Australia's national biotech incubator, to develop new technology to improve the safe and effective delivery of gene therapy.

The gene therapy technology will be developed by the researchers in a new start-up company called AAVec Bio, that will be dedicated to advancing adeno-associated virus (AAV) technology. This bioengineered virus technology, that serves as a vehicle to transport healthy genes to cells, will be used to potentially cure a diverse group of genetic diseases.

Led by Professor John Rasko AO, from the Centenary Institute and the Royal Prince Alfred Hospital (SLHD), the research team aims to develop a novel AAV technology that will be more effective at delivering therapeutic genes to the correct cells in the body.

The researchers say the hope is to overcome some current limitations of AAV-based gene therapy, including the need for high AAV doses to achieve therapeutic effects, dose-related toxicities and high manufacturing costs.

"The success of gene therapy depends on how effectively the therapeutic payload is delivered to target cells in the body," said Professor Rasko.

"Our novel platform could greatly improve human gene therapies, leading to more effective treatments for those suffering with unmet medical needs. It would also mean substantially reduced dosage levels are required, with less toxicities and side effects, leading to improved outcomes for patients."

Another exciting aspect of the work relates to the current high cost of gene-based drugs as the researchers anticipate that their new AAV approach could significantly decrease manufacturing costs by five- or even ten-fold, due to the reduced amount of therapeutic material required.

"This cost saving could potentially transform the gene therapy market by making these treatments far more accessible and affordable to patients," said Professor Rasko.

The researchers intend to first demonstrate the utility of their new technology for gene therapy in Pompe disease, a rare genetic disorder that leads to progressive muscle weakness and damage to the heart. Ultimately the technology could improve gene therapies for thousands of different rare diseases that affect two million Australians.

"Pompe disease affects the body's ability to breakdown glycogen, which accumulates in tissues like muscle and causes damage. It affects about 1 in 40,000 people globally," said Dr Chuck Bailey, senior scientist at the Centenary Institute and AAVec Bio.

Dr Bijay Dhungel, scientist at the Centenary Institute and AAVec Bio stated, "By using gene therapy to replace the defective gene, we have the potential to achieve a long-lasting

curative outcome for Pompe disease, in addition to demonstrating the proof-of-principle of our AAV technology and the effectiveness of our new approach.”

[ENDS]

Images:

Professor John Rasko:

<https://drive.google.com/file/d/1DRBMwl-W4XINyS2VvUCIWEEttdh42waA/view?usp=sharing>

AAVec Bio researchers: LtoR – Professor John Rasko AO, Dr Bijay Dhungel and Dr Chuck Bailey:

<https://drive.google.com/file/d/1iozM3jatDMbEOHgUkPISFSzD4J1Eai1a/view?usp=sharing>

For all media and interview enquiries, please contact

Tony Crawshaw, Media and Communications Manager, Centenary Institute on 0402 770 403 or email: t.crawshaw@centenary.org.au

About the Centenary Institute

The Centenary Institute is a world-leading independent medical research institute, closely affiliated to the University of Sydney and the Royal Prince Alfred Hospital. Our research focuses on three key areas: cancer, inflammation and cardiovascular disease. Our strength lies in uncovering disease mechanisms and applying this knowledge to improve diagnostics and treatments for patients.

For more information about the Centenary Institute, visit centenary.org.au

About CUREator

CUREator is a national biotechnology incubator run by Brandon BioCatalyst to support the development of Australian biomedical research and innovations.

CUREator provides grant funding programs targeting biomedical opportunities spanning from early-stage development through to clinical trials. Providing more than just funding, CUREator works closely with project teams to guide them through the early development phase, offering both scientific and commercial expertise and networks to support projects in meeting key commercial milestones. Funding is provided with clear milestone-driven tranches and help is provided to guide development of these assets and maximise their chance of success.

For more information about CUREator visit <https://brandonbiocatalyst.com/cureator/>