MEDIA RELEASE

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Research grant to make gene therapy safer

A team led by Dr Chuck Bailey at the Centenary Institute has been awarded a National Health and Medical Research Council (NHMRC) Ideas Grant to develop safer and more effective gene therapies.

The project will focus on improving the safety and efficacy of adeno-associated virus (AAV)-based gene therapies, a promising treatment approach for many genetic disorders.

AAVs are naturally occurring viruses which can be harnessed to deliver good copies of genes into humans, thus enabling the treatment of diseases caused by faulty genes.

While AAV therapies have transformed treatment options for certain conditions, significant challenges remain. One major hurdle is the need for high doses to deliver genes effectively to hard-to-treat tissues, like muscle or the central nervous system.

These higher doses, required for diseases such as spinal muscular atrophy or Duchenne muscular dystrophy, can trigger unwanted immune responses, severe side effects and in some cases, life-threatening adverse events.

Dr Bailey's team has discovered a new way to potentially improve AAV gene therapies by focusing on a specific protein named AAVR2. This protein plays a crucial role in helping the virus enter cells effectively. By boosting AAVR2 activity, the researchers aim to reduce the required dose of AAV, making the therapy safer, cheaper and more accessible to patients.

The team will apply this new method to target Pompe disease, a rare genetic condition affecting approximately 1 in 40,000 people. The condition causes severe muscle weakness, heart damage and breathing difficulties, significantly impacting mobility and quality of life.

"The support from the NHMRC enables us to address one of the biggest challenges in gene therapy today—improving safety and effectiveness," said Dr Bailey.

"We're excited to explore this innovative approach, which has the ability to transform treatment options for patients with rare and complex diseases."

Grant Details
Grant Title: Enhanced adeno-associated virus vectors for safer gene therapies.
Chief Investigators: Dr Chuck Bailey, Dr Bijay Dhungel
Grant: $1,065,723

The NHMRC Ideas Grant program supports innovative and impactful research projects in all areas of health and medical research, from discovery to implementation.

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Image:
Dr Chuck Bailey
https://drive.google.com/file/d/1MRZd2v2PREygd8m8zD__D0rMchsjeHnS/view?usp=sharing

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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 spans the critical areas of cancer, cardiovascular disease, rare diseases, inflammation, infectious diseases, healthy ageing and biomedical AI. Our strength lies in uncovering disease mechanisms and applying this knowledge to improve diagnostics and treatments for patients.

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