



## PRESS RELEASE

### Promising results shown in the treatment of growth hormone resistant dwarfism

***With gene therapy, a cure for growth-hormone resistant dwarfism may be on the horizon***

**Singapore, 25 April 2022** – Researchers from the NUS Yong Loo Lin School of Medicine (NUS Medicine) have shown that gene therapy using a single-dose injection of a virus carrying the ‘good’ gene can potentially be used to cure growth-hormone resistant dwarfism, also known as ‘Laron Syndrome’.

People with Laron syndrome are very short as their bodies are unable to use the growth hormone, a substance that helps the body to grow. The team led by Prof Lee K O, from Department of Medicine at NUS Medicine, attempted to increase a hormone called insulin-like-growth factor 1 (IGF1) in a laboratory model by replacing the defective growth hormone receptor gene, using a virus containing instructions specifically targeted at the liver. The liver is the main organ producing the IGF1 hormone. Previously, the only treatment available was injection of genetically-engineered recombinant insulin-like-growth factor 1 (rhIGF1), administered daily or even twice daily. Yet, the results from this original treatment have not been ideal.

The team previously generated a specific gene delivery tool, called the AAV8 carrier that expressed the human insulin gene in the liver. When this showed potential to be used by diabetic patients as a long-term basal insulin gene therapy, the team of researchers which included Dr Sia Kian Chuan and Dr Gan Shu Uin, from the Department of Surgery, attempted to further explore the potential of the AAV8 gene delivery tool to understand the extent of its efficacy. The results showed the significant size and weight which increased steadily throughout the course of 26 weeks, in comparison to the untreated laboratory model.

Other researchers have shown that there is persistent gene expression for up to 10 years to produce proteins in the liver necessary for growth, following a single dose of AAV administration. This makes AAV gene therapy an attractive treatment with potentially significantly good therapy outcomes.

Using an AAV8 gene delivery tool, gene therapy has emerged as a safe and efficient treatment with the potential to treat a variety of inherited or rare mutation disorders such as hemophilia and spinal muscular atrophy. There were no treatments available for these disorders previously. With these optimistic results, the AAV vector can be potentially used to treat people with Laron syndrome.

Prof Lee said, “Gene therapy, has given us a promising start in managing dwarfism. This is far more favourable compared to rhIGF1 injections, once or twice daily for many years, which causes side effects of pain and discomfort to patients and comes as a huge financial burden to those involved.”

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Our multidisciplinary and real-world approach to education, research and entrepreneurship enables us to work closely with industry, governments and academia to address crucial and complex issues relevant to Asia and the world. Researchers in our faculties, 30 university-level research institutes, research centres of excellence and corporate labs focus on themes that include energy; environmental and urban sustainability; treatment and prevention of diseases; active ageing; advanced materials; risk management and resilience of financial systems; Asian studies; and Smart Nation capabilities such as artificial intelligence, data science, operations research and cybersecurity.

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Through a dynamic and future-oriented five-year curriculum that is inter-disciplinary and inter-professional in nature, our students undergo a holistic learning experience that exposes them to multiple facets of healthcare and prepares them to become visionary leaders and compassionate doctors and nurses of tomorrow. Since the School's founding in 1905, more than 12,000 graduates have passed through our doors.

In our pursuit of health for all, our strategic research programmes focus on innovative, cutting-edge biomedical research with collaborators around the world to deliver high impact solutions to benefit human lives.

The School is the oldest institution of higher learning in the National University of Singapore and a founding institutional member of the National University Health System. It is one of the leading medical schools in Asia and ranks among the best in the world (Times Higher Education World University Rankings 2022 by subject and the Quacquarelli Symonds (QS) World University Rankings by subject 2021).

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