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Andelyn Biosciences partners Queenâ??s University on AAV9-GM2 gene therapy manufacturing

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The collaboration will use Andelynâ??s AAV Curator platform to develop and manufacture AAV9-GM2 for GM2 gangliosidoses, including Tay-Sachs and Sandhoff diseases.

Andelyn Biosciences has partnered with Queen's University to develop and manufacture AAV9-GM2, an investigational gene therapy for GM2 gangliosidoses, including Tay-Sachs and Sandhoff diseases.

GM2 gangliosidoses are rare inherited lysosomal storage disorders caused by genetic mutations that disrupt normal lysosomal function. The resulting accumulation of harmful substances in cells leads to progressive damage to nerve cells in the brain and spinal cord.

The condition most commonly presents during infancy, although later-onset forms can emerge during childhood, adolescence or adulthood.

Under the partnership, Andelyn Biosciences will use its AAV Curator Platform in a multi-phase programme intended to advance AAV9-GM2 towards patient dosing.

The AAV Curator Platform is Andelyn's viral vector process for adeno-associated virus development and manufacturing. It incorporates a proprietary cell line and modular approach to adapting manufacturing unit operations according to individual programme requirements.

Andelyn said the platform is designed to support both yield and product quality during viral vector development and manufacturing.

"We are proud to partner with Queen's University to advance this gene therapy candidate for the benefit of patients and families facing the challenges of GM2 gangliosidoses," said Matt Niloff, Chief Commercial Officer at Andelyn Biosciences.

"Our deep expertise in AAV development and production allows us to support Queen's University with the scale and quality rigor necessary to bring this life-changing therapy one step closer to reality."

Dr Jagdeep Walia, medical geneticist and Professor in the Department of Pediatrics at Queen's University, said Andelyn's experience in viral vector manufacturing was an important consideration in the collaboration.

"This partnership with Andelyn is very strategic as they have the established expertise of producing vectors for many gene therapy programs. Their track record and ability to manufacture the viral vector for the upcoming GM2 program gives us confidence that the participants in the clinical trial will be receiving the highest quality, safe product."

The partnership expands Andelyn's work supporting gene therapy programmes targeting rare and ultra-rare diseases, where manufacturing scale, product quality and development timelines are important considerations in progressing therapies towards clinical use.