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Andelyn Biosciences Selected To Manufacture Clinical Grade AAV Gene Therapy For Rare NKH Treatment

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Collaboration with Drake Rayden Foundation advances clinical grade gene therapy manufacturing to accelerate hope for children affected by Nonketotic Hyperglycinemia.

Andelyn Biosciences, Inc., a leading and patient-focused cell and gene therapy Contract Development and Manufacturing Organization (CDMO), has been selected by Drake Rayden Foundation to apply the AAV Curator® Platform to manufacture clinical grade AAV for the Treatment of Nonketotic Hyperglycinemia (NKH), a rare, inherited metabolic genetic disorder caused by a mutation in the GLDC gene.

NKH is a debilitating disease that causes a disruption of the glycine cleavage system (GCS), leading to the inability of the body to break down the amino acid glycine. Glycine builds up to toxic levels, particularly in the brain, which leads to severe neurological problems including seizures, lethargy, poor muscle tone, breathing issues, and profound intellectual disability. Children with the severe form of NKH, comprising close to 85% of diagnosed, often live only days or weeks after birth. Those that survive live with severe seizure disorders and a very poor prognosis.

The Drake Rayden Foundation was formed in 2017 with the conviction to push research forward towards a treatment for NKH by raising funds and establishing key partnerships with research institutes and manufacturing organizations. This initiative reflects Andelyn's commitment to accelerate gene therapies to patients universally by working with not only biotech and pharma companies, but also individual families and non-profit organizations. Andelyn will work closely with the Drake Rayden Foundation and scientists from Dr. Steven Gray's laboratory at the University of Texas Southwestern (UTSW) to establish the manufacturing process in Andelyn's AAV Curator® Platform, a methodology that offers reliable speed and quality.

Matt Niloff, Chief Commercial Officer at Andelyn Biosciences said, “We are elated that our established and rapidly growing work on clinical and commercial gene therapy programs for the biotech industry allows us to also support family foundations seeking treatments for loved ones-often children with rare diseases. The power of gene therapy continues to offer the much-needed hope for the many patients with genetic diseases, and we are honored to play a pivotal role in delivering these therapies.”

Tarah O’Sullivan, founder and mother of Drake and Vivian said, “We have clung to the verse in Galatians that brought such encouragement when we faced the exhaustion that comes from trying to create treatment in a rare disease. Chapter 6 verse 9 reminds us, “Let us not become weary in doing good, for at the proper time we will reap a harvest if we do not give up.” This gene therapy is bringing such a season of hope for the future.”

With industry-leading process development and clinical and commercial manufacturing capabilities, state-of-the-art facilities, and a patient-first approach, Andelyn continues to serve as the CDMO partner of choice for organizations advancing life-changing gene therapies.