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Andelyn Biosciences delivers gene therapy for ultra-rare NEDAMSS disease in record time using AAV Curator® platform

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In just 10 months, Andelyn manufactures and releases a life-saving IRF2BPL gene therapy for baby Elly, marking a pivotal moment in ultra-rare disease treatment and hope for the NEDAMSS community.

Andelyn Biosciences, Inc., a leading and patient-focused cell and gene therapy Contract Development and Manufacturing Organization (CDMO), has successfully manufactured a novel viral vector gene therapy with its AAV Curator® Platform to treat a baby with the ultra-rare disease Neurodevelopmental Disorder with Regression, Abnormal Movements, Loss of Speech, and Seizures (NEDAMSS). This is a major accomplishment as the therapy was produced and released only 10 months from the start of development and 14 months after the patient's initial diagnosis. This feat establishes yet another foothold for gene therapies as an effective and accessible treatment for patients.

NEDAMSS is a neurodegenerative disease affecting the central nervous system and is a regressive disorder with no known cures or treatments available. It can impact motor skills, speech, eating, and eyesight, among other functions, and often causes seizures. Diagnosed on Feb 8, 2023 at just 8 months old, Elly Krueger's form of NEDAMSS, caused by a mutation in the IRF2BPL (Interferon regulatory factor 2 binding protein-like) gene, was rapidly progressing, making every day more pressing than the one before.

The Kruegers leveraged their own expertise and network to learn everything they could about the disease in an effort to save their child. The masthead of their efforts is Elly's Team, a foundation built from the ground up with one purpose—to save Elly and children like her. And through sheer determination, after evaluating multiple possible treatment options, a potential gene therapy was chosen, and Andelyn Biosciences was selected to develop, scale up, and manufacture the drug. With time a critical factor, the team at Andelyn prioritized the program, transferred it in, and immediately got to work building a manufacturing process with their AAV Curator® Platform moving at the pace needed to try to help Elly.

Michelle Krueger, mother of Elly said, "On April 3rd, 2025, Elly became the first child to receive an IRF2BPL gene replacement therapy at Weill Cornell in New York City. This achievement marks a major milestone, not only for our family but also for the entire IRF2BPL community. Throughout this experience, the Andelyn Biosciences team became like part of our family. Everyone at the company knew the importance of speed. Many of them met Elly, held her, and prayed for her. Every step of the way, they knew there was a purpose beyond the vial – for Elly and all the other children affected by this devastating disease."

One-month post-treatment, the therapy has been safe and well tolerated, and Elly is doing very well. While it is still too early to assess efficacy, the Kruegers are filled with hope and optimism. This is an extraordinary moment for all of Elly's Team, and everyone is encouraged by the FDA's support to expand this treatment for other children.

Wade Macedone, CEO of Andelyn Biosciences, had this to say, "Supporting single-patient projects is part of the DNA of Andelyn Biosciences and the lifestyle of everyone who works at the organization. As a company we are as proud of successfully completing single-patient programs as we are scaling a manufacturing process for 10,000 or more patients. Individually, we all know that with these projects there is someone, usually a young child, whose life depends on us."

Andelyn's AAV Curator® Platform utilizes Optimization-by-DesignTM, a data-driven approach to correctly size processes for scale and speed to the clinic. The platform is highly predictable in terms of yield and quality and eliminates intellectual property concerns because the technology has all been developed internally. Furthermore, review of investigational new drug (IND) applications is streamlined as the referenced Drug Master Files (DMFs) have been reviewed and approved by the FDA previously, removing certain unnecessary hurdles or delays. The well-established platform allows for the ability to support the exacting viral vector manufacturing needs of different types of patients, including for ultra-rare diseases like NEDAMSS.