Charles River Joins Elly's Team to Drive Rare Disease Gene Therapy Development

July 22, 2025 at 8:00 AM EDT

Supporting fast-track production of therapeutic targeting ultra-rare neurodevelopmental disease

WILMINGTON, Mass.--(BUSINESS WIRE)--Jul. 22, 2025-- Charles River Laboratories International, Inc. (NYSE: CRL) and Elly's Team , a parent-led foundation striving to find a cure for Neurodevelopmental Disorder with Regression, Abnormal Movements, Loss of Speech, and Seizures (NEDAMSS), a rare genetic disorder, have announced a plasmid DNA contract development and manufacturing organization (CDMO) agreement. As part of Charles River's Cell and Gene Therapy (CGT) Accelerator Program (CAP), Elly's Team accessed established gene therapy CDMO capabilities and advisory services to manufacture critical starting materials for a Phase I clinical trial.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20250722374711/en/

Elly's Team is a faith-based foundation established with a singular goal of translating medical research to treatment in record time. Their path to treatment involves funding additional research to understand the disease mechanism and effects on the body, developing and manufacturing drugs, navigating the FDA approval process and clinical trials, and treating children. To learn more, visit ellysteam.org.

"The opportunity to work with Elly's Team aligns closely with our core mission: supporting the development of potentially curative treatments for Elly and other patients suffering from ultra-rare diseases with no known treatment. We are honored to work hand in hand with the team as they navigate the next stage of their program development," said Kerstin Dolph, Corporate Senior Vice President, Global Manufacturing, Charles River.

Translating Research to Treatment in Record Time

Founded in 2024 by Michelle and Dan Krueger, Elly's parents, Elly's Team is a parent-led foundation with a singular goal of translating medical research to treatment for NEDAMSS as quickly as possible.

NEDAMSS is an ultra-rare disease caused by mutations in the Interferon regulatory factor 2 binding protein-like (IRF2BPL) gene. Fewer than 150 cases, including Elly, have been diagnosed worldwide. The regressive neurodevelopmental disorder affects the central nervous system and can impact motor skills, speech, eating, and eyesight, among other functions. It can also often cause seizures.

Having quickly assembled experts in gene therapy and drug discovery, Elly's Team was able to advance multiple steps simultaneously to shorten timelines, including funding additional research to understand the disease mechanism and effects on the body, and conducting safety and toxicity studies while also manufacturing the drug. They did this while also navigating the Food and Drug Administration (FDA) approval process to get Investigational New Drug (IND) approval to dose Elly in early April 2025, only 13 months after her diagnosis.

"We want to express our deepest gratitude to all members of Elly's Team who made this incredible treatment a reality, including Charles River whose partnership and expertise has undoubtedly contributed to the advancement of our gene therapy program," stated Michelle Kruger, Elly's mother and Co-Founder, Elly's Team. "Signed off by the FDA in March 2025, on April 3, 2025, Elly became the first child to receive an IRF2BPL gene replacement therapy. This achievement marks a major milestone, not only for our family but also for the entire IRF2BPL community. In the future, another family will sit in the hospital and receive the same diagnosis, but their doctor will tell them there is a path to treatment."

Plasmid DNA CDMO Solutions

Elly's Team has leveraged Charles River's established plasmid platform, eXpDNA™, and premier expertise in plasmid DNA production, including phase-appropriate High Quality (HQ) plasmid which employs principles of good manufacturing practices (GMP) to manufacture plasmids with a rapid turnaround to accelerate time to clinic.

Additionally, the program has utilized Charles River's off-the-shelf AAV Rep/Cap plasmids, also designed to streamline adeno-associated virus (AAV)-based gene therapy programs by reducing production time and improving efficacy of the supply chain, ultimately reducing manufacturing efforts by up to 66 percent. The reliable, ready-to-use plasmid products are manufactured and released with CMC guidance and according to batch production records, with a Certificate of Analysis (COA) to support Investigational New Drug (IND) and Clinical Trial Application (CTA) filings.

In recent years, Charles River has significantly broadened its cell and gene therapy portfolio with several acquisition integrations and expansions to simplify complex supply chains and meet the growing demand for plasmid DNA, viral vector, and cell therapy services. Combined with the Company's comprehensive discovery, safety assessment, and biologics testing capabilities, Charles River offers an industry-leading "concept to cure" advanced therapies solution, because every moment matters.

In today's evolving advanced therapies ecosystem, plasmid DNA retains its common role as a critical starting material, laying the foundations for CGT production. In this bite-sized 30-minute webinar, subject matter experts cover common issues and quality control considerations from a manufacturing perspective: https://bit.ly/3RBXIrG

About Charles River

Charles River provides essential products and services to help pharmaceutical and biotechnology companies, government agencies and leading academic institutions around the globe accelerate their research and drug development efforts. Our dedicated employees are focused on providing clients with exactly what they need to improve and expedite the discovery, early-stage development and safe manufacture of new therapies for the

patients who need them. To learn more about our unique portfolio and breadth of services, visit www.criver.com.

About Elly's Team

Elly's Team is a faith-based foundation established with a singular goal of translating medical research to treatment in record time. Their path to treatment involves funding additional research to understand the disease mechanism and effects on the body, developing and manufacturing drugs, navigating the FDA approval process and clinical trials, and treating children. To learn more, visit ellysteam.org.

View source version on businesswire.com: https://www.businesswire.com/news/home/20250722374711/en/

Charles River Investor Contact:
Todd Spencer
Corporate Vice President,
Investor Relations
781.222.6455
todd.spencer@crl.com

Charles River Media Contact:
Amy Cianciaruso
Corporate Vice President,
Chief Communications Officer
781.222.6168
amy.cianciaruso@crl.com

Source: Charles River Laboratories International, Inc.