Charles River and Genetic Cures for Kids Announce Gene Therapy Manufacturing Collaboration

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Company will perform plasmid DNA production in support of early phase trials for SPG56 Hereditary Spastic Paraplegia

WILMINGTON, Mass.--(BUSINESS WIRE)--Nov. 28, 2023-- Charles River Laboratories International, Inc. (NYSE: CRL) and Genetic Cures for Kids Inc (GC4K), an Australian non-profit foundation focused on research programs and clinical trials to find cures for rare diseases, today announced a plasmid DNA contract development and manufacturing organization (CDMO) collaboration.

Founded in 2021 by Golden and Chris Whitrod, parents of now 4 year old Tallulah Moon, GC4K's first mission, known as Our Moon's Mission, is to develop a gene therapy for the treatment of Hereditary Spastic Paraplegia Type 56 (SPG56). SPG56 is a debilitating neurodegenerative disease that robs children of the ability to walk, talk, and move independently.

What is SPG56?

SPG56 manifests as a progressive neurological disease characterised by varying degrees of spasticity and muscle weakness. This degenerative disease typically begins with motor and cognitive regression in childhood and continuously worsens through life. Additional clinical manifestations include intellectual disability, dystonia, cerebellar ataxia, subclinical peripheral neuropathy, seizures, and visual impairment.

Tragically, there is currently no treatment for this ultra-rare genetic disease. In addition, limited access to genetic testing means diagnoses are hard to achieve and as such, fewer than one in 1 million people have ever been diagnosed with SPG56 globally. GC4K is leading a gene therapy program that aim not to only cure SPG56, but to create a replicable framework that paves the way to develop treatments for some of the 7,000 other genetic diseases in the world.

Plasmid DNA Manufacturing Services

GC4K will leverage Charles River's established plasmid platform, eXpDNA™ and premier expertise in plasmid DNA production, including High Quality (HQ) plasmid which combines key features of good manufacturing practice (GMP) manufacture with a rapid turnaround to accelerate time to clinic. As a critical starting material in the development of cell and gene therapies, in 2022 Charles River opened a state-of-the-art HQ Plasmid Manufacturing Center of Excellence dedicated to addressing global supply shortages and support the growing needs of customers.

In recent years, Charles River has significantly broadened its cell and gene therapy portfolio to simplify complex supply chains and meet growing demand for plasmid DNA, viral vector, and cell therapy services. Combined with the Company's legacy testing capabilities, Charles River offers a comprehensive "concept-to-cure" advanced therapies solution.

To learn more about plasmid DNA production, watch Advancements in Manufacturing for Rapid Delivery of pDNA Starting Materials, presented by Andrew Frazer, PhD, Associate Director, Scientific Solutions, Gene Therapy CDMO Services, on-demand: https://bit.ly/3ZGgPnd

Approved Quotes

- "The opportunity to work with GC4K is exactly why we do what we do, playing a role in delivering potentially curative treatments to patients suffering with an ultra-rare disease. We are excited to support the team as they work through the next stage of their program development." - Kerstin Dolph, Corporate Senior Vice President, Biologics Solutions, Charles River
- "What was once a tragic diagnosis, has the potential to become a triumph, as we draw nearer to finding a cure for SPG56. With the advancement of this groundbreaking treatment, our partnership with Charles River is bringing us closer to aiding children like Tallulah, who, until now, have faced the daily hardships of SPG56 without any glimmer of hope. We're truly excited to have the backing of this skilled team as we gear up to produce this historic treatment. By making this announcement on Giving Tuesday, we are giving thanks to Charles River. This symbolic occasion not only commends the generosity of CRL in supporting GC4K's quest to cure SPG56, but also to recognizes the broader goal that our partnership is striving for - a brighter future for all children living with rare genetic diseases." - Golden Whitrod, Tallulah Moon's mother and Co-Founder and President, Genetic Cures for Kids, Inc.

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.

Genetic Cures for Kids, Inc., and Our Moon's Mission

GC4K is a family-driven research foundation at the forefront of genetic research, with a primary objective to not only develop a cure for SPG56 but also to establish a reproducible framework that can catalyze the development of treatments for some of the 7,000 known genetic diseases worldwide.

The foundation's campaign to cure SPG56 is "Our Moon's Mission", named in honor of the founders' daughter Tallulah Moon, who lives with SPG56.

By leveraging crowdfunding to assemble an expert international research team, which has since gained recognition and funding from the Australian Government and is guided by an Independent Scientific Advisory Board, GC4K aims to expedite the development of effective SPG56 therapies for clinical use. Additionally, the foundation is dedicated to advocating for 'rare families' and increasing awareness of rare diseases. To learn more about GC4K's commitment to advancing genetic research and to support the foundation's mission to cure SPG56, please visit www.ourmoonsmission.org

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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

Genetic Cures for Kids Inc Media Contact:
Golden Whitrod
Co-Founder, Board President, Genetic Cures for Kids (Our Moon’s Mission)
E: golden@ourmoonsmission.org
M: +61449154411

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