



Charles River and Cure AP-4 Announce Gene Therapy Manufacturing Collaboration

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Company will perform HQ plasmid production in support of Phase I/II clinical trials for AP-4 Hereditary Spastic Paraplegia

ALDERLEY PARK, England--(BUSINESS WIRE)--Sep. 6, 2022-- Charles River Laboratories International, Inc. (NYSE: CRL) and [Cure AP-4](#), a non-profit foundation dedicated to raising funds and awareness about Adapter-Protein 4 Hereditary Spastic Paraplegia (AP-4 HSP), today announced a manufacturing collaboration. Charles River, a contract research and development manufacturing organization (CRO/CDMO), will provide High Quality (HQ) plasmid DNA for Cure AP-4's Phase I/II gene therapy trials against AP-4 HSP.

Founded in 2016 by the families of two newly diagnosed AP-4 HSP (SPG47) patients, Molly Duffy and Robbie Edwards, Cure AP-4's gene therapy treatment will look to address the root cause of AP-4 HSP, a rare neurodegenerative disorder, and is intended as a one-time, curative treatment for the patient.

What is AP-4 HSP?

AP-4 HSP, also known as AP-4 Deficiency Syndrome, includes four sub-types of HSP: SPG47, SPG50, SPG51 and SPG52. Each of these HSP sub-types is associated with a defective autosomal recessive gene which causes a failure in the AP-4 Adaptor Complex. The phenotype and prognosis for each sub-type is extremely similar. Patients afflicted with any of the AP-4 HSP genetic disorders generally present with symptoms including global developmental delay, microcephaly, seizures, brain malformation, and hypotonia (low-muscle tone). The few patients who learn to walk independently tend to lose that ability a few months or few years later as they develop hypertonia (high-muscle tone) and muscle spasticity. Of the 249 currently confirmed global AP-4 HSP cases, most patients experience mobility in some or all extremities as the disorder progresses and are severely intellectually challenged.

Plasmid DNA Manufacturing Services

The collaboration will leverage Charles River's market leading expertise in plasmid DNA production, specifically HQ plasmid, which combines key features of GMP manufacture with rapid turnaround times to accelerate the timeline to clinic. DNA plasmids are a critical starting material for many cell and gene therapy therapeutics and demand continues to outstrip supply. In response to this, Charles River recently announced the opening of a [state-of-the-art HQ plasmid manufacturing center of excellence](#) to address these supply shortages and support the growing needs of the cell and gene therapy field.

Charles River, with the acquisitions of Cognate BioServices, Cobra Biologics, and Vigene Biosciences in 2021, has extended its comprehensive cell and gene therapy portfolio to include CDMO capabilities spanning viral vector, plasmid DNA and cellular therapy production for clinical through to commercial supply.

Approved Quotes

- "The opportunity to work with a group as driven as Cure AP-4 is exactly why we do the work we do. Playing a small but critical role in delivering a potentially transformative therapy to a severely underserved patient population gives us an incredible sense of pride, and we are excited for the Cure AP-4 team to achieve this next stage." –Birgit Girshick, Corporate Vice President and Chief Operating Officer, Charles River
- "We refuse to accept the bleak prognosis which our children face and have decided to fight. Our aim is to improve the quality of life for children impacted by AP-4 HSP by accelerating the research for cures and treatments and providing support for patient therapies critical to their well-being and rehabilitation. Our partnership with Charles River brings us closer to accomplishing that goal and we are excited to begin manufacturing our treatment with their experienced team." –Chris Edwards, Robbie Edwards' father and President, Cure AP-4

About Cure AP-4

Cure AP-4, originally known as Cure SPG47, was founded in 2016 by the families of two newly diagnosed SPG47 patients, Molly Duffy and Robbie Edwards. At the time there were only nine other documented cases worldwide, and due to the extreme rarity of the disorder there are no known treatments or cures.

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.

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