



## Charles River and INADcure Announce Gene Therapy Manufacturing Collaboration

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*CDMO will perform HQ plasmid production in support of Phase I/II clinical trials for Infantile Neuroaxonal Dystrophy*

ALDERLEY PARK, England--(BUSINESS WIRE)--Jun. 8, 2023-- Charles River Laboratories International, Inc. (NYSE: CRL) and the [INADcure Foundation](#), a nonprofit organization whose mission is to support the development of treatments for Infantile Neuroaxonal Dystrophy (INAD) and other forms of PLA2G6-related neurodegeneration (PLAN), today announced a plasmid DNA manufacturing alliance.

The collaboration will leverage Charles River's market leading contract development and manufacturing organization (CDMO) expertise in High Quality (HQ) plasmid DNA production, to manufacture its leading candidate for Phase I/II clinical trials. In INAD patients, a mutation or genetic alteration in the PLA2G6 gene causes mitochondrial and axonal membrane defects that lead to neuronal damage with axonal swellings and accumulation of pre-synaptic spheroids.

Leena and Anil Panwala founded the INADcure Foundation in 2016, following the diagnosis of their two-year-old daughter, Ariya. Upon discovering there was no dedicated organization or centralized effort driving INAD research forward, they launched the Foundation with the mission to accelerate research and drive drug discovery. After seven years of unwavering dedication and with the tremendous support of INAD-afflicted families worldwide, the Foundation is furthering the development of a gene therapy treatment specifically designed for children affected by this rare neurodegenerative disease.

### What is INAD?

INAD, otherwise known as phospholipase associated neurodegeneration (PLAN), is a rare disease with no known treatment or cure. Like other neurodegenerative diseases, INAD is progressive, and symptoms include poor motor function, involuntary eye movements, seizures, hearing loss, and difficulty swallowing and breathing. INAD affects over 150 children worldwide with a life expectancy of 10 or less years.

### Plasmid DNA Manufacturing Services

Charles River's plasmid DNA production, specifically HQ plasmid, combines key features of Good Manufacturing Practice (GMP) manufacture with rapid turnaround times to accelerate the timeline to clinic. Plasmid DNA is 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 Cobra Biologics, Vigene Biosciences, and Cognate BioServices, in 2021, has extended its comprehensive cell and gene therapy portfolio to include CDMO capabilities spanning plasmid DNA, viral vector, and cellular therapy production for clinical through to commercial supply.

*To learn more about plasmid DNA production, watch Andrew Frazer, PhD, Associate Director, Scientific Solutions, Gene Therapy CDMO Services' on-demand webinar: Advancements in Manufacturing for Rapid Delivery of pDNA Starting Materials. <https://bit.ly/3O5qYG9>*

### Approved Quotes

- "The opportunity to work with INADcure is exactly why we do the work we do. Their work is incredibly important to patients with this ultra-rare disorder. We are excited for the INADcure team to achieve this next stage of treatment development." - Kerstin Dolph, Corporate Senior Vice President, Biologics Solutions, Charles River
- "Our collaboration with Charles River brings us closer to accomplishing our goal of delivering breakthrough treatments for INAD. We are excited to begin HQ plasmid DNA manufacturing to further develop our treatment with their experienced team. Their invaluable expertise will undoubtedly contribute to the advancement of our gene therapy program." - Leena Panwala, Founder & Executive Director at INADcure.

### About INADcure

The INADcure Foundation is a 501(c)(3) nonprofit charity whose mission is to support the development of treatments, including a cure, for Infantile Neuroaxonal Dystrophy (INAD) and other forms of PLA2G6-related neurodegeneration (PLAN). Its work includes advancing our understanding of this devastating disease, educating the public about INAD and other forms of PLA2G6-related neurodegeneration, advocating for better access to treatment, and supporting the families and caregivers of children diagnosed with INAD worldwide. For more information, visit [www.INADcure.org](http://www.INADcure.org).

### 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](http://www.criver.com).

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