



## Charles River Announces Rare Disease Gene Therapy Collaboration with Axovia Therapeutics

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*HQ plasmid DNA production to support Phase I trials for gene therapy targeting Bardet-Biedl Syndrome*

WILMINGTON, Mass.--(BUSINESS WIRE)--Apr. 9, 2024-- Charles River Laboratories International, Inc. (NYSE: CRL) today announced a plasmid DNA contract development and manufacturing organization (CDMO) collaboration with [Axovia Therapeutics Ltd.](#) Charles River will manufacture High Quality (HQ) gene of interest plasmid to support the development of Axovia's gene therapies for ciliopathies, including Bardet-Biedl Syndrome (BBS), a condition with limited treatment options and no cure.

Ciliopathies are diseases caused by cilia dysfunction, which is a group of complex disorders caused by genetic mutations that result in defective or dysfunctional cilia in many organs of the human body. Dysfunctional cilia can cause blindness, deafness, chronic respiratory infections, kidney disease, heart disease, infertility, obesity and diabetes. Over 20 ciliopathies have been identified, collectively affecting an estimated 1 in 1,000 people.

Axovia's lead program, AXV101, is an adeno-associated virus (AAV9)-based gene therapy targeting retinal dystrophy associated with BBS. In preclinical studies for BBS, the novel gene therapy modified the underlying disease, rescuing vision loss by halting retinal degeneration, stopping BBS-induced weight gain and the development of obesity. Axovia has been granted FDA Orphan Drug and Rare Pediatric Disease Designations.

### Plasmid DNA Manufacturing Services

Axovia will leverage Charles River's established plasmid platform, [eXpDNA™](#), and premier expertise in plasmid DNA production, including HQ plasmid which combines key features of good manufacturing practice (GMP) manufacture with a rapid turnaround to accelerate time to clinic.

In recent years, Charles River has significantly broadened its cell and gene therapy portfolio to simplify complex supply chains and meet the 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.

*Available on-demand is a 30-minute case study webinar led by Dr. Andrew Frazer, Associate Director, Scientific Solutions, Gene Therapy CDMO Services, that explores the role of plasmid DNA as a critical starting material, the latest advancements in associated regulatory guidance to safeguard your therapeutic programs, and case studies demonstrating expedited development and manufacture of ATMPs: <https://bit.ly/3vbl9yS>*

### Approved Quotes

- "We are committed to supporting Axovia in the pursuit of creating treatment options for patients living with ciliopathies. The Charles River team brings decades of expertise in HQ plasmid development, and we look forward to using these capabilities to make a difference for patients." - Kerstin Dolph, Corporate Senior Vice President, Global Manufacturing, Charles River
- "This collaboration brings us closer to accomplishing our goal to develop potentially transformative therapies for ciliopathies. We are excited to leverage Charles River's expertise in plasmid DNA production to help drive our work forward." - Victor Hernandez, Co-Founder and Chief Scientific Officer, Axovia

### 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).

### About Axovia Therapeutics Ltd.

Axovia Therapeutics, an ALSA Ventures portfolio company, is developing the first novel gene therapies for ciliopathies and has a pipeline of products for these devastating diseases, including Bardet-Biedl Syndrome (BBS). For more information, visit [www.axoviatherapeutics.com](http://www.axoviatherapeutics.com).

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### Charles River Investor Contact:

Todd Spencer  
Corporate Vice President,  
Investor Relations  
781.222.6455  
[todd.spencer@crl.com](mailto:todd.spencer@crl.com)

### Charles River Media Contact:

Amy Cianciaruso  
Corporate Vice President,

Chief Communications Officer  
781.222.6168  
[amy.cianciaruso@crl.com](mailto:amy.cianciaruso@crl.com)

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