ALSA Ventures launches novel Gene Therapy portfolio company Axovia Therapeutics to treat Ciliopathies

- Developing the first gene therapy to treat diseases caused by cilia dysfunction
- AXV101 targets retinal dystrophy for Bardet-Biedl Syndrome (BBS) patients
- FDA Orphan Drug Designation and Rare Pediatric Disease Designation achieved
- First-in-man study planned to start in late 2024/early 2025
- Six potentially transformative therapies for ciliopathies are in its pipeline

London, UK – September 18, 2023 - ALSA Ventures, a UK-based, therapeutics-focused venture capital fund, announces the acquisition of Axovia Therapeutics Inc and the launch of a new portfolio company Axovia Therapeutics Ltd.

Axovia is developing the first gene therapies for ciliopathies and has a pipeline of products for these devastating diseases, including Bardet-Biedl Syndrome (BBS).

The ALSA Ventures' investment team has designed an accelerated development plan to take the lead program AXV101 into clinical trials in the next 18-24 months with a rapid path to clinical proof of concept and approval.

AXV101 is an AAV9-based gene therapy targeting retinal dystrophy associated with BBS in patients carrying biallelic mutations in the BBS1 gene. It is designed to halt retinal degeneration, which begins in childhood leading to blindness before 20 years of age.

Revised epidemiological analyses suggest that BBS affects between 1 in 70,000 - 1 in 100,000 in Europe and North America, and there is no treatment for the retinal degeneration.

ALSA Ventures CEO, Alek Safarian, said they were excited to take AXV101 into clinical trials, which will have access to well-characterised and motivated patients. "Especially pediatric patients who need early treatment before permanent vision damage occurs," he said.

"Our investment team was particularly attracted to Axovia Therapeutics as a valuable addition to our growing portfolio of companies because of its striking preclinical results, its Rare Pediatric Disease Designation, and the proven AAV delivery mechanism," he said.

The company is well positioned for an FDA priority review voucher (PRV) which is awarded to sponsors that develop drugs for diseases, including rare paediatric diseases.

Axovia is based on decades of work on ciliopathies at University College London by co-founders Professor Phil Beales and Dr Victor Hernandez.

Axovia Acting CEO Professor Phil Beales said the Axovia gene therapy platform gives hope to BBS patients worldwide.

“In preclinical studies, our BBS1 novel gene therapy modified the underlying disease of BBS, including rescuing vision loss by halting retinal degeneration,” stated Professor Beales.

Professor Beales is a renowned scientist and diagnostic leader in ciliopathies, having led research and patient care efforts out of University College London (UCL) Institute of Child Health for one of the most debilitating ciliopathic diseases in the world – BBS. See community resources here https://axoviatherapeutics.com/get-support-for-bbs/

“Our novel gene therapy utilises an adeno-associated virus (AAV9) to deliver a functional copy of the faulty BBS gene in key tissues,” he said.

“Since AAV is not known to cause human disease and can be tightly controlled (it does not replicate like disease-carrying viruses), it has been the gene delivery method of choice for multiple therapies, including Luxturna for retinal disease.”

Available clinical data on more than 3,000 people treated over more than 20 years indicate that AAV gene therapy is well-tolerated and efficacious.

We are grateful to Fieldfisher LLP for their assistance on the transaction.

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About Axovia Therapeutics

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).

Ciliopathies are a group of more than 40 rare inherited genetic diseases linked to more than 950 genes that impact the function of cilia. These microscopic finger-like organelles protrude from most cells in the body.

The lead program AXV101 is planned to enter clinical trials in the next 18-24 months.

AXV101 is an AAV9-based gene therapy targeting retinal dystrophy associated with BBS in patients carrying biallelic mutations in the BBS1 gene. It is designed to halt photoreceptor cell death and retinal degeneration.

BBS is a rare disease affecting between 1in 70,000 and 1 in 100,000 in Europe and North America. There is currently no cure.

For further information, please visit https://axoviatherapeutics.com/

About ALSA Ventures

ALSA Ventures is an early-stage investor in life sciences. The firm is building a portfolio of companies founded on innovative insights into disease treatment where current therapeutic options are either limited or non-existent. We work with a broad global network of preclinical and clinical experts to gain a deep understanding of translationally relevant disease biology and then leverage the team's collective knowledge and operational experience to identify opportunities for drug intervention.

Our focus is to improve patient outcomes by unlocking the full potential of clinical-stage assets. Our mission remains clear as we continue to grow our portfolio: to bring life-changing therapies to patients worldwide.

For further information, please visit **https://www.alsaventures.com/**.