



Belite Bio Announces FDA Approval of Investigational New Drug (IND) for Phase 1 Clinical Trial of LBS-008 to Treat Macular Degeneration and Stargardt Disease

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San Diego

Belite Bio, a drug development company targeting currently untreatable conditions in ophthalmology and metabolic diseases, announced today that the U.S. Food and Drug Administration (FDA) has approved its Investigational New Drug application (IND) for LBS-008 (BPN-14967), a first-in-class oral therapy for the treatment of atrophic Age-related Macular Degeneration (commonly known as dry AMD) and Stargardt disease, an inherited juvenile form of macular degeneration. LBS-008 will be one of the first drugs to graduate from the US National Institute of Health's (NIH) Blueprint Neurotherapeutics Network (Blueprint Program), which has funded LBS-008's discovery and development, and will continue to provide support and funding until the completion of its Phase 1 single ascending dose (SAD) clinical trial.

"Initiation of the Phase 1 trial is an important milestone for LBS-008, which was funded by the NIH Blueprint Program starting from lead optimization to a preclinical proof of concept," said Dr. Konstantin Petrukhin, Professor of Ophthalmic Science at Columbia University Medical Center, and inventor of the technology. "The combined strength of the academic research conducted at Columbia University and the drug development expertise, resources, and funding provided by the NIH Blueprint Program has enabled LBS-008 to bring hope to millions of patients suffering from untreatable forms of macular degeneration."

"We are excited to announce the start of this Phase 1 trial in the US together with the NIH," said Dr. Tom Lin, Chairman of Belite Bio. "We're encouraged by the ongoing favorable regulatory view of our technology, including a recent systematic review published by the UK National Institute for Health Research identifying the reduction of serum RBP4 as the most promising treatments for dry AMD and Stargardt disease. We plan to continue to work closely with our collaborators and leverage the Blueprint Program's resources and support to move LBS-008 to the next clinical and regulatory milestone."

About LBS-008

LBS-008 is a first-in-class oral therapy that prevents the buildup of toxins (A2E) in the eye that cause Stargardt disease and contribute to atrophic Age-related Macular Degeneration (dry AMD). The toxins are by-products of the eye's visual cycle which are produced from vitamin A. LBS-008 works by reducing and modulating a carrier protein, Retinol-Binding Protein 4 (RBP4), that is to transport vitamin A to the eye. LBS-008 does not directly interfere with the visual cycle, and therefore is unlikely to affect the visual cycle rate.

Belite Bio holds the worldwide exclusive rights for LBS-008. LBS-008 received US and EU orphan drug designation (ODD) in 2017 and 2018, and rare pediatric disease designation (RPD) from the FDA in 2018 for the treatment of Stargardt disease.

Dry Age-related Macular Degeneration

Atrophic age-related macular degeneration (dry AMD) is the main cause of vision loss in the United States, and has zero approved treatments available. There are an estimated 11 million dry AMD patients in the US and over 170 million patients worldwide¹ with a global direct healthcare cost of 255 billion US dollars²

Stargardt Disease

Stargardt disease is an inherited juvenile form of macular degeneration, which affects 1 in 8,000-10,000 children³. Patients suffer from progressive vision loss starting in childhood, with most patients becoming visually impaired by the age of 20. The disease is caused by a mutation in the ABCA4 gene, which leads to the accelerated formation and accumulation of toxins from excess vitamin A by-products in the retina that cause progressive retinal cell death and permanent loss of vision. Currently, there is no treatment for Stargardt disease, which is an orphan indication.

About Belite Bio

Belite Bio, a subsidiary of Lin BioScience, is a San Diego based clinical stage biopharmaceutical drug development company targeting untreatable diseases, including age-related macular degeneration, Stargardt disease, and liver diseases. For more information, please visit www.belitebio.com.

Reference:

1. [Katie L. Pennington](#) and [Margaret M. DeAngelis](#) (2016). Epidemiology of age-related macular degeneration (AMD): associations with cardiovascular disease phenotypes and lipid factors. URL: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5178091/> (accessed 17 April 2019)

2. BrightFocus Foundation. Sources for Macular Degeneration: Facts & Figures. URL: <https://www.brightfocus.org/sources-macular-degeneration-facts-figures> (accessed 17 April 2019)
3. Facts About Stargardt Disease, National Eye Institute. URL: https://nei.nih.gov/health/stargardt/star_facts (accessed 14 September 2018)