



Belite Bio Initiates Phase 1 Study of LBS-008 in Australia

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San Diego

Belite Bio, a clinical-stage biotech company focused on developing first-in-class small molecule antagonists of Retinol Binding Protein 4 (RBP4), announced the initiation of a single-ascending dose phase 1 study of LBS-008 on October 3, 2018. LBS-008, an oral first-in-class small molecule RBP4 specific antagonist, is a potential treatment for atrophic Age-related Macular Degeneration (commonly known as dry AMD) and Stargardt disease, an inherited juvenile form of macular degeneration. The first-in-human phase 1 study is being conducted in healthy volunteers in Australia and is expected to be completed during the 2nd half of 2019.

LBS-008 Phase 1 Trial in Healthy Volunteers in Australia

The LBS-008 phase 1 study is a double-blind, placebo-controlled, single and multiple ascending dose study in healthy adult volunteers. Approximately 72 subjects in total are expected to be enrolled. The primary endpoint for the trial is to evaluate safety, tolerability and pharmacokinetics (PK) of LBS-008. The secondary endpoint is to assess the effects of LBS-008 on plasma levels of RBP4, the pharmacodynamics (PD) marker and potential biomarker that is proposed to correlate to clinical benefit for dry AMD. (ANZCTR registration number: ACTRN12618001823268)

LBS-008 Mechanism of Action

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 million¹ patients worldwide 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.

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)