Belite Bio Initiates Pivotal Phase 3 Clinical Trial of LBS-008 in Stargardt Disease in the U.S.

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- LBS-008 (aka Tinlarebant) is Belite Bio's orally administered tablet for the treatment of Stargardt disease (STGD1) and Dry AMD
- A 2-year Phase 2 trial in adolescent STGD1 and a global Phase 3 trial in adolescent STGD1 are ongoing
- The Phase 3, Multi-center, Randomized, **D**ouble Masked, Placebo Controlled Study to Evaluate the Safety and Efficacy of Tinla**R**ebant in the Treatment of St**A**r**G**ardt Disease in Ad**O**lesce**N**t Subjects (DRAGON) trial has commenced in the U.S., the United Kingdom, Germany, Belgium, Switzerland, Hong Kong, Taiwan, and Australia, and several patients have been enrolled
- LBS-008, Belite Bio's lead asset, has been granted fast track designation and rare pediatric disease designation (RPD) in the U.S., and orphan drug designation (ODD) in both the U.S. and Europe for STGD1

SAN DIEGO, Aug. 22, 2022 (GLOBE NEWSWIRE) -- Belite Bio, Inc (NASDAQ: BLTE), a San Diego based clinical stage biopharmaceutical drug development company targeting currently untreatable eye diseases, today announced that it has commenced enrollment for the U.S. Phase 3 clinical trial of LBS-008 in patients with Stargardt Disease (STGD1), a progressively blinding disease with no approved treatment.

"STGD1 is the most common inherited retinal dystrophy causing blurring and/or loss of central vision in both adults and children," said Dr. Tom Lin, Belite Bio's Chairman and CEO. "With over 30,000 STGD1 patients alone in the U.S., we have a treatment with the potential to address a large unmet need with a clear clinical pathway to bring hope to patients afflicted with this debilitating disease."

LBS-008 is an orally-available, small molecule retinol binding protein 4 (RBP4) antagonist that selectively reduces the delivery of vitamin A (retinol) to the eye, leading to a reduction of toxic vitamin A byproducts (bisretinoids) that have been implicated in the onset and progression of STGD1. Sponsored by the NIH Blueprint program to treat non-neovascular age-related macular degeneration (Dry AMD), LBS-008 is also endorsed by NIH as a promising first-in-class oral medication to slow or halt the progression of Dry AMD, a disease which primarily affects the elderly and shares a similar pathophysiology as STGD1. There are currently no approved treatments for STGD1 or Dry AMD by the U.S. Food and Drug Administration (FDA).

Belite Bio has completed multiple randomized, double-blind, placebo-controlled, Phase 1 trials of LBS-008 in healthy adult subjects, including a single ascending dose, or SAD trial in 40 subjects in the U.S., and a SAD trial in 39 subjects and a multiple ascending dose, or MAD, trial in 32 healthy adult subjects in Australia. These trials were conducted to confirm the safety, toxicity, PK, and PD of LBS-008 on a range of SAD (10-50 mg in the U.S.; 25-400 mg in Australia) / MAD (5-25 mg in Australia) levels in healthy adult subjects in fasted / fed conditions. All dose levels were well tolerated.

Belite Bio is currently conducting a 2-year Phase 2 trial and a 2-year Phase 3 (DRAGON) trial of LBS-008 in adolescent STGD1 subjects. The Phase 2 trial has enrolled a total of 13 subjects at clinical sites in Australia and Taiwan. Preliminary data from the Phase 2 trial at the first 6-month interval shows that 8 of the 13 patients (or 61.5%) recorded a gain in best-corrected visual acuity (BCVA) in at least one eye, including 2 patients who recorded a BCVA gain in both eyes. In addition, there were no atrophic lesions in any of the 13 subjects at study start and only 1 subject showed evidence of a retinal lesion (~0.3mm^2 in size) at 6 months. Belite Bio expects the next data readout of this Phase 2 trial to occur in the last quarter of 2022 when all subjects have completed 12 months of treatment.

The Phase 3 (DRAGON) trial, a randomized, double-masked, placebo-controlled, global and multi-center study, is designed to evaluate the safety and efficacy of LBS-008 in adolescent STGD1 patients. To date, the Company has commenced the Phase 3 clinical trial in the U.S., the United Kingdom, Germany, Belgium, Switzerland, Hong Kong, Taiwan, and Australia. Approximately 60 patients are targeted for enrollment in this study with a 2:1 randomization (active:placebo). (For more information, visit clinicaltrials.gov at https://www.clinicaltrials.gov/ct2/show/NCT05244304?term=belite+bio&draw=2&rank=1)

Additionally, because the accumulation of toxic bisretinoids has also been implicated in the progression of Dry AMD, Belite Bio believes that LBS-008 has the potential to be effective for the treatment of Dry AMD as well. Belite Bio plans to initiate a Phase 2/3 clinical trial for Dry AMD in the 4th quarter of 2022.

About LBS-008

LBS-008 is a novel oral therapy that prevents the buildup of toxins in the eye that cause STGD1 and contribute to dry AMD. These toxins are by-products of the visual cycle, which is dependent on the supply of vitamin A (retinol) to the eye. LBS-008 works by reducing and maintaining levels of serum retinol binding protein 4 (RBP4), a carrier protein that transports retinol to the eye. By modulating the amount of retinol entering the eye, LBS-008 reduces the formation of toxins which have been implicated in STGD1 and dry AMD in order to maintain the health of retinal tissues. LBS-008

has been granted fast track designation, rare pediatric disease designation (RPD) in the U.S., and orphan drug designation (ODD) in the U.S. and Europe for the treatment of STGD1.

Stargardt Disease

STGD1 is the most common inherited retinal dystrophy (causing blurring or loss of central vision) in both adults and children. The disease is caused by a dysfunctional retina-specific gene (ABCA4) which results in massive accumulation of toxic vitamin A byproducts (known as “bisretinoids”) in the retina leading to retinal cell death and progressive loss of central vision. The fluorescent properties of bisretinoids and the development of retinal imaging have helped ophthalmologists identify and monitor disease progression. Additionally, STGD1 and dry AMD share a similar pathophysiology characterized by excessive accumulation of cytotoxic bisretinoids, retinal cell death, and loss of vision. Vision loss occurs slowly, despite peripheral expansion of “dead retina”, until the disease reaches the center of the eye (the macula).

Dry Age-related Macular Degeneration
Dry AMD is a leading cause of vision loss in the U.S., and has zero approved treatments available. There are an estimated 11 million dry AMD patients in the U.S. and over 196 million patients worldwide with an estimated global direct healthcare cost of US$255 billion.

About Belite Bio

Belite Bio is a San Diego based clinical stage biopharmaceutical drug development company targeting currently untreatable eye diseases, such as atrophic age-related macular degeneration (commonly known as dry AMD) and Stargardt disease, and metabolic diseases. For more information, follow us on Twitter, Instagram, LinkedIn, Facebook or visit us at www.belitebio.com.

Important Cautions Regarding Forward Looking Statements
This press release contains forward-looking statements, including statements regarding the potential implications of clinical data for patients, and Belite Bio’s advancement of, and anticipated preclinical activities, clinical development, regulatory milestones, and commercialization of its product candidates. Actual results may differ materially from those indicated in the forward-looking statements as a result of various important factors, including but not limited to Belite Bio’s ability to demonstrate the safety and efficacy of its drug candidates; the clinical results for its drug candidates, which may not support further development or regulatory approval; the content and timing of decisions made by the relevant regulatory authorities regarding regulatory approval of Belite Bio’s drug candidates; the potential efficacy of LBS-008 on the treatment of Dry AMD, as well as those risks more fully discussed in the “Risk Factors” section in Belite Bio’s filings with the U.S. Securities and Exchange Commission. All forward-looking statements are based on information currently available to Belite Bio, and Belite Bio undertakes no obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.

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