Belite Bio Submits Investigational New Drug (IND) Application to FDA for Approval to Proceed with LBS-008 Phase 3 Clinical Trial for the Treatment of Stargardt Disease

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- LBS-008 (a/k/a Tinlarebant) is the Company's orally administered tablet for the treatment of Stargardt disease (STGD1)
- There are currently no approved treatments for STGD1
- Approximately 30,000 patients in the U.S. suffer from STGD1
- A 2-year Phase 2 trial in adolescent STGD1 and a global Phase 3 trial in adolescent STGD1 are ongoing
- The Phase 3, Multicenter, 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 been approved for patient enrollment in the United Kingdom, Switzerland, Hong Kong, Taiwan, and Australia, and enrollment has commenced
- LBS-008, the Company's lead asset, 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

SAN DIEGO, July 19, 2022 (GLOBE NEWSWIRE) -- Belite Bio, Inc (NASDAQ: BLTE) ("Belite" or the "Company"), a San Diego based clinical stage biopharmaceutical drug development company targeting untreatable eye diseases, today announced the submission of an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) to support Phase 3 development of LBS-008, the Company's orally administered treatment for Stargardt Disease (STGD1), a rare genetic eye disease that causes progressive vision loss in children and adults.

LBS-008 is a 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 intended 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 FDA approved treatments for STGD1 or dry AMD.

Dr. Tom Lin, Belite's Chairman and CEO commented, "We are very pleased with LBS-008's progress in the clinic. With the promising results from the Phase 2 trial in early-onset STGD1 subjects, we have initiated the global Phase 3 trial for LBS-008 and believe that we are on a clear clinical development pathway to accelerate and bring forward a promising treatment for STGD1 and dry AMD."

Belite Bio is currently conducting a 2-year Phase 2 trial and a 2-year Phase 3 trial of LBS-008 in adolescent STGD1 subjects. The Phase 2 trial, which has enrolled 13 subjects at clinical sites in Australia and Taiwan, was initiated immediately following the successful completion of a Phase 1b dose-finding trial in the same adolescent STGD1 patients. The 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 BCVA in at least one eye, including 2 patients who recorded a gain in BCVA in both eyes. And 12 of the 13 patients had no DDAF lesion measured at the start of Phase 2 and at 6-months, only 1 of the 13 patients had a DDAF lesion growth of 0.3mm^2 in both eyes during the 6-month period.

Dr. Rando Allikmets, the William and Donna Acquavella Professor and Research Director at the Edward S. Harkness Eye Institute, Columbia University, who identified the gene responsible for STGD1, ABCA4, noted that "vision loss in patients with STGD1 and dry AMD is irreversible and currently there are no FDA-approved treatments available. Therapeutic applications, which can slow the rate of disease progression, measured by atrophic lesion growth, which is an FDA accepted primary endpoint in both STGD1 and advanced dry AMD, should help to preserve functional vision, greatly improving the quality of life for patients. Dr. Allikmets further commented that "the data from Belite Bio's pre-clinical and clinical studies fully support the hypothesis that LBS-008 can achieve this effect and, therefore, warrants further investigation as a promising treatment to halt or slow disease progression in STGD1 and maybe also dry AMD."

"Ultimately the optimal treatment pathway for STGD1 and dry AMD would be early detection and treatment," said Dr. Quan Dong Nguyen, MD, MSc, FARVO, FASRS, Professor of Ophthalmology, Pediatrics, and Medicine (Immunology/Rheumatology) at the Byers Eye Institute, Stanford University School of Medicine. "Belite Bio's compelling clinical data to date has shown trends for stabilization or improvement of vision in adolescent STGD1 subjects. The disease is otherwise known for its rapid or progressive loss of vision. The positive results support the trial hypothesis that reduced delivery of vitamin A may be effective to slow the progression of STGD1. Because the accumulation of toxic bisretinoids has also been implicated in the progression of dry AMD, LBS-008 has the potential to be effective in this disease as well."

The global Phase 3, or the DRAGON, trial has been approved for patient enrollment in the United Kingdom, Switzerland, Hong Kong, Taiwan, and Australia, and enrollment has commenced. The Company has additional plans to submit clinical trial application across other jurisdictions.

About LBS-008

LBS-008 is a novel oral therapy that prevents the accumulation of toxic byproducts of vitamin A which are known to cause STGD1 and contribute to dry AMD. These toxins are byproducts of the visual cycle, which is dependent on the supply of vitamin A 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. 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.

Stargardt Disease

STGD1 is the most common inherited retinal dystrophy (causing blurring or loss of central vision) in both children and adults. 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 no approved treatments. The cause of dry AMD is not known although it is believed to have diverse causes. 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 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 certain "forward-looking statements" within the meaning of federal securities laws. All statements, other than statements of historical facts, included herein are "forward-looking statements" including, among other things, statements about Belite’s beliefs and expectations. The expectations reflected in these forward-looking statements involve significant assumptions, risks and uncertainties, and these expectations may prove to be incorrect. Investors should not place undue reliance on these forward-looking statements, which speak only as of the date of this press release. Potential risks and uncertainties include, but are not limited to, risks discussed in Belite’s filings with the U.S. Securities and Exchange Commission at www.sec.gov. Other than as required under the securities laws, the Company does not assume a duty to update these forward-looking statements.

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