Belite Bio Receives FDA Fast Track Designation For LBS-008

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- *LBS-008 (aka Tinlarebant) is the Company's orally administered tablet for the treatment of Stargardt disease (STGD1)*
- *There are currently no approved treatments for STGD1*
- *A 2-year Phase 2 trial in adolescent STGD1 and a global Phase 3 trial in adolescent STGD1 are ongoing*
- *LBS-008, the Company's lead asset, has been granted rare pediatric disease designation in the U.S. and orphan drug designation in the U.S. and Europe*
- *Approximately 30,000 patients in the U.S. suffer from STGD1*

SAN DIEGO, May 03, 2022 (GLOBE NEWSWIRE) -- Belite Bio, Inc (the "Company") (Nasdaq: BLTE), a San Diego based clinical stage biopharmaceutical drug development company targeting currently untreatable eye diseases, such as atrophic Age-related Macular Degeneration (dry AMD) and Stargardt disease (STGD1), and metabolic diseases, today announced that LBS-008, an orally administered tablet, has been granted Fast Track Designation by the U.S. Food and Drug Administration (FDA) for the treatment of STGD1. This decision was based upon FDA's review of non-clinical data and preliminary clinical data from studies of LBS-008.

"We are delighted that LBS-008 has received FDA Fast Track Designation. STGD1 is a terrible retinal disease with the potential to severely affect the vision of afflicted patients and there are currently no approved treatments," said Dr. Tom Lin, the Company's Chairman and CEO. "At present, we are conducting a Phase 3 clinical trial in order to bring to market a treatment that will halt or slow the progression of STGD1. Additionally, we are evaluating our plan to launch a Phase 2/3 trial in Dry AMD in 2022. Dry AMD is a disease which shares a similar underlying pathophysiology with STGD1 and is a leading cause of central vision loss in people over 50."

The Company expects the next near-term data readout in its STGD1 Phase 2 trial to occur in the last quarter of this year when all subjects have completed 12 months of treatment.

About Fast Track Designation
Fast Track is a process designed to facilitate the development, and expedite the review, of drugs to treat serious conditions and fill an unmet medical need. The purpose is to get important new drugs to the patient earlier. Fast Track addresses a broad range of serious conditions. Filling an unmet medical need is defined as providing a therapy where none exists or providing a therapy which may be potentially better than available therapy. Once a drug receives Fast Track designation, it is eligible for more frequent meetings with the FDA, more frequent written communication from the FDA, Accelerated Approval and Priority Review if relevant criteria are met, and Rolling Review. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

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.

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 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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