Belite Bio Receives Approval to Initiate Tinlarebant (LBS-008) Phase 3 Clinical Trial for Stargardt Disease in South Korea

June 13, 2023

- Tinlarebant (a/k/a LBS-008) is Belite Bio's **orally administered tablet** intended to slow disease progression in patients affected with Stargardt Disease (STGD1) and Geographic Atrophy (GA) in advanced Dry Age-related Macular Degeneration (Dry AMD)
- A 2-year Phase 2 study in adolescent STGD1 patients is ongoing and a global Phase 3 study in adolescent STGD1 patients (the "DRAGON" study) is recruiting subjects
- A continued trend of slowing expansion of autofluorescence was observed and the growth rate of incident atrophic retinal lesions was reduced with Tinlarebant in the ongoing Phase 2 STGD1 study compared to a natural history study of the disease ("ProgStar")
- Tinlarebant has been granted Fast Track Designation and Rare Pediatric Disease Designation in the U.S., and Orphan Drug Designation in both the U.S. and Europe for STGD1

SAN DIEGO, June 13, 2023 - Belite Bio, Inc (NASDAQ: BLTE), a clinical stage biopharmaceutical drug development company focused on advancing novel therapeutics targeting retinal degenerative eye diseases which have significant unmet medical needs, today announces the approval of a Clinical Trial Application to the Ministry of Food and Drug Safety (MFDS) to initiate the Phase 3 clinical trial of Tinlarebant (aka LBS-008) for STGD1, or the DRAGO study, in the Republic of Korea.

The DRAGON study is a phase 3, randomized, double-masked, placebo-controlled, global and multi-center study, designed to evaluate the safety and efficacy of Tinlarebant in adolescent STGD1 patients. To date, the Company has commenced the DRAGON study in the U.S., the United Kingdom, Germany, France, Belgium, Switzerland, Netherlands, China, Hong Kong, Taiwan, and Australia. At least 90 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)

About Tinlarebant (a/k/a LBS-008)

Tinlarebant is a novel oral therapy which is intended to reduce the accumulation of toxins in the eye that cause STGD1 and contribute to GA, or advanced Dry AMD. These toxins are by-products of the visual cycle, which is dependent on the supply of vitamin A (retinol) to the eye. Tinlarebant works by reducing and maintaining levels of serum retinol binding protein 4 (RBP4), the sole carrier protein for retinol transport from the liver to the eye. By modulating the amount of retinol entering the eye, Tinlarebant reduces the formation of these toxins.

Stargardt Disease (STGD1)

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 mutations in a 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 systems have helped ophthalmologists identify and monitor disease progression. Currently, there are no FDA approved treatments for STGD1.

Importantly, STGD1 and GA, or advanced Dry AMD, share a similar pathophysiology which is characterized by the 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). Therefore, Belite Bio intends to evaluate safety and efficacy of Tinlarebant in GA patients in its Phase 3 study (PHOENIX).

GA in advanced Dry Age-related Macular Degeneration (Dry AMD)

Dry AMD is a leading cause of vision loss in older adults. Geographic Atrophy, or GA, is the advanced stage of AMD. Currently, there are no FDA approved orally administered treatments for GA and no FDA approved therapies for the other stages of Dry AMD other than GA. There are an estimated 20 million 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 clinical-stage biopharmaceutical drug development company focused on advancing novel therapeutics targeting retinal degenerative

eye diseases which have significant unmet medical needs, such as STGD1 and GA in advanced Dry AMD, in addition to specific 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, and any other statements containing the words "expect", "will", "believe" and similar expressions. 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 Tinlarebant, 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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