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

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- Tinlarebant (aka LBS-008) is Belite Bio's **orally administered tablet** for **early intervention** to maintain the health of retinal tissue in Stargardt disease (STGD1) and Dry AMD patients
- There are currently no approved treatments for 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 STGD1 trial named "DRAGON" is a 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 has commenced in the U.S., the United Kingdom, Germany, France, Belgium, Switzerland, China, Hong Kong, Taiwan, and Australia, with several patients already enrolled
- A 2-year Phase 3 trial in Dry AMD patients with geographic atrophy (GA) is expected to begin enrollment in the first half of 2023
- Tinlarebant, Belite Bio's lead asset, 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, Jan 11, 2023- Belite Bio, Inc (NASDAQ: BLTE) ("Belite" or the "Company"), a San Diego based clinical stage biopharmaceutical drug development company targeting currently untreatable eye diseases, today announces the approval from The Central Committee on Research Involving Human Subjects to initiate the Phase 3 clinical trial of Tinlarebant (aka LBS-008) for STGD1 in the Netherlands.

The DRAGON trial 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 trial in the U.S., the United Kingdom, Germany, France, Belgium, Switzerland, China, 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)

About Tinlarebant (aka LBS-008)

Tinlarebant is a novel oral therapy intended as an early intervention to prevent the accumulation of vitamin A-based toxins (bisretinoids) that cause STGD1 and contribute to pathogenesis in Dry AMD. Bisretinoids are formed as by-products of vitamin A in the visual cycle. Tinlarebant works by reducing the level of serum RBP4, the carrier protein which transports the retinol to the eye. By modulating the amount of retinol entering the eye, Tinlarebant reduces the formation of bisretinoids to preserve 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. Importantly, STGD1 and 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). There are currently no approved treatments available for STGD1.

Dry Age-related Macular Degeneration

Dry AMD is a leading cause of vision loss in the U.S. Dry AMD has a heterogenous etiology but shows a pathophysiology that is similar to that of STGD1 in which the excessive accumulation of cytotoxic bisretinoids leads to retinal cell death and loss of vision. There are an estimated 11 million patients with Dry AMD in the U.S. and over 196 million patients worldwide with an estimated global direct healthcare cost of US$255 billion. An advanced vision-threatening form of Dry AMD is Geographic Atrophy (GA), which is characterized by progressive atrophy of the retinal pigment epithelium and underlying choriocapillaris, followed by loss of photoreceptors within the retina, and ongoing visual impairment. Over 8 million people are affected worldwide with GA, which is approximately 20% of all individuals with AMD. The incidence of GA is expected to rise as the age-burden of developed countries is increasing. There are currently no approved treatments available for Dry AMD.

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 advanced 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, 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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