# Belite Bio Receives Approval to Initiate LBS-008 Phase 3 Clinical Trial in France

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- LBS-008 (aka Tinlarebant) 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
- A 2-year Phase 2 trial in adolescent STGD1 and a global Phase 3 trial in adolescent STGD1 are ongoing
- The Phase 3 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, Belgium, Switzerland, China, Hong Kong, Taiwan, and Australia, with several patients already enrolled
- 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, Nov 9, 2022- Belite Bio, Inc (NASDAQ: BLTE), a San Diego based clinical stage biopharmaceutical drug development company targeting currently untreatable eye diseases, today announced the approval from The National Agency for the Safety of Medicines and Health Products (L'Agence nationale de sécurité du médicament et des produits de santé, ANSM) of France to initiate the Phase 3 clinical trial of Tinlarebant in France.

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, 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)

The accumulation of toxic bisretinoids have also been implicated in the progression of Dry AMD, a disease with a huge unmet need, which primarily affects the elderly and shows a pathophysiology that is similar to that of STGD1. This finding has led to the sponsorship and endorsement of Tinlarebant by the NIH Blueprint program as a promising first-in-class oral medication to slow or halt the progression of Dry AMD. Belite believes that Tinlarebant has the potential to be an effective early intervention treatment to maintain the health of retinal tissues in Dry AMD. Belite plans to initiate a Phase 2/3 clinical trial for Dry AMD in the fourth quarter of 2022.

**About LBS-008 (aka Tinlarebant)**

Tinlarebant is a novel oral therapy intended as an early intervention to prevent the buildup of toxins in the eye that cause STGD1 and contribute to advanced Dry AMD. These toxins are by-products of vitamin A in 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 transport of retinol into the eye. By modulating the amount of retinol entering the eye, Tinlarebant reduces the formation of the formation of toxins that have been implicated in STGD1 and Dry AMD. Tinlarebant has been granted Fast Track Designation, Rare Pediatric Disease Designation in the U.S., and Orphan Drug Designation 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. 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).

**Dry Age-related Macular Degeneration**

Dry AMD is a leading cause of vision loss in the U.S. and has no 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 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. 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 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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