



## Belite Bio Submits IND Amendment to Initiate Phase 3 Clinical Trial of Tinarebant (LBS-008) in Advanced Dry AMD

February 1, 2023

- Tinarebant (a/k/a LBS-008) is Belite Bio's **orally administered tablet** intended to halt or slow disease progression in patients affected with Stargardt Disease (STGD1) and 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 now recruiting subjects
- 12-month interim data from the ongoing Phase 2 STGD1 study continues to show halting or slowing of lesion growth
- Tinarebant 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, January 31, 2023 - [Belite Bio](#), Inc (NASDAQ: BLTE), a San Diego based clinical stage biopharmaceutical drug development company targeting currently untreatable eye diseases, today announced the FDA submission of a Phase 3 clinical study protocol to evaluate efficacy and safety of Tinarebant (a/k/a LBS-008) in patients with geographic atrophy (GA) associated with Dry AMD.

"Dry AMD is a chronic degenerative disease of the retina that leads to blindness in the elderly. We believe that Tinarebant, being an oral once-a-day tablet, would be a non-invasive, more convenient, and well-tolerated long-term treatment for Dry AMD." said Dr. Tom Lin, CEO of Belite Bio.

### **Phase 3 Advanced Dry AMD Study Outline**

- 2-year prospective, randomized (2:1, active:placebo), double-masked, placebo-controlled study designed to assess the efficacy and safety of daily oral Tinarebant.
- Enrollment of approximately 430 GA patients across multiple centers globally is expected to begin in the first half 2023
- The primary endpoint will be the change in GA lesion size from baseline to month 24, compared to placebo.
- An interim analysis of efficacy and safety is expected to be conducted at the mid-point of the study.

### **About Tinarebant (a/k/a LBS-008)**

Tinarebant is a novel oral therapy intended 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. Tinarebant 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, Tinarebant reduces the formation of bisretinoids to preserve the health of retinal tissues.

### **About 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 protein (ABCA4) which causes an early, aberrant accumulation of cytotoxic byproducts of vitamin A in the retina leading to retinal cell death and progressive loss of vision. There are currently no approved treatments available for STGD1.

### **About Dry Age-related Macular Degeneration**

There are an estimated 20 million patients with AMD in the U.S. and 196 million patients worldwide with an estimated global direct healthcare cost of US\$255 billion. Dry AMD accounts for approximately 90% of AMD cases and is a leading cause of vision loss in the U.S. Dry AMD has a heterogeneous 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. 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. 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 Dry AMD) and STGD1, and metabolic diseases. For more information, follow us on [Twitter](#), [Instagram](#), [LinkedIn](#), [Facebook](#) or visit us at [www.belitebio.com](http://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 Tinalrebant, 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.

**Media and Investor Relations Contact:**

Jennifer Wu / [ir@belitebio.com](mailto:ir@belitebio.com)

Tim McCarthy / [tim@lifesciadvisors.com](mailto:tim@lifesciadvisors.com)