Belite Bio Completes Enrollment in Pivotal Global Phase 3 DRAGON Trial Evaluating Oral Tinlarebant for Stargardt Disease

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- Tinlarebant (a/k/a LBS-008) is Belite Bio's **orally administered tablet** intended to slow disease progression in patients with Stargardt Disease (STGD1) and Geographic Atrophy (GA) in advanced Dry Age-related Macular Degeneration (Dry AMD)
- 90 patients completed enrollment across 11 countries worldwide
- Interim safety and efficacy data from the Phase 3 "DRAGON" trial for STGD1 is expected in mid-2024
- Tinlarebant has been granted Fast Track and Rare Pediatric Disease Designations in the U.S., and Orphan Drug Designation in both the U.S. and Europe for STGD1, for which there are no FDA approved treatments

SAN DIEGO, July 24, 2023 (GLOBE NEWSWIRE) -- Belite Bio, Inc (NASDAQ: BLTE) ("Belite" or the "Company"), a clinical stage biopharmaceutical drug development company focused on advancing novel therapeutics targeting retinal degenerative eye diseases which have significant unmet medical needs, today announced that enrollment of its pivotal global Phase 3 "DRAGON" trial for patients with Stargardt Disease (STGD1) has completed. Tinlarebant is an oral, once daily retinol binding protein 4 (RBP4) antagonist designed to lower levels of ocular vitamin-A based toxins implicated in STGD1.

"Completing enrollment of all 90 adolescent subjects across 11 countries worldwide in the Phase 3 DRAGON trial marks an important milestone for our late-stage program in STGD1. Importantly, we are pleased to see the embrace of this much needed therapeutic opportunity by the STGD1 patient community. The DRAGON trial has potential to be the first global Phase 3 clinical trial to demonstrate a treatment benefit in patients with STGD1 and we look forward to sharing the interim safety and efficacy data in mid-2024," said Dr. Tom Lin, CEO of Belite Bio.

Professor Michel Michaelides, the Principal Investigator and the Chief Investigator of the DRAGON trial from Moorfields Eye Hospital in the United Kingdom, one of the top recruiting sites globally, added that, "It has been a pleasure and privilege to be one of the recruiting sites for this promising Phase 3 DRAGON trial. The sustained slowing of disease progression demonstrated in the 18-month Phase 2 trial for patients with STGD1 is highly encouraging, and further reinforces our belief in the transformative potential of Tinlarebant to be the first FDA-approved treatment option for STGD1."

The pivotal Phase 3 DRAGON trial is a randomized, double-masked, placebo-controlled, global and multi-center study, designed to evaluate the safety and efficacy of Tinlarebant in adolescent STGD1 patients. The DRAGON trial has global sites in 11 countries, including the U.S., the United Kingdom, Germany, France, Belgium, Switzerland, Netherlands, China, Hong Kong, Taiwan, and Australia. 90 patients have been enrolled in this study with a 2:1 randomization (active:placebo). The primary efficacy endpoint is slowing of lesion growth rate, along with the assessment of safety and tolerability. 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.

Topline data for Tinlarebant in the Phase 2 STGD1 study is expected in the fourth quarter of 2023 and the interim data for the Phase 3 DRAGON trial is expected by mid-2024.

In addition, Belite plans to have the first patient enrolled in the 2-year Phase 3 study (PHOENIX) of Tinlarebant in Geographic Atrophy (GA) in the third quarter of 2023.

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. Tinlarebant has been granted Fast Track Designation and 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)

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).

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 the Company on Twitter, Instagram, LinkedIn, Facebook or visit us at www.belitebio.com.

Forward Looking Statements

This press release contains forward-looking statements, about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts. These statements include but are not limited to statements regarding the potential implications of clinical data for patients, clinical development and regulatory milestones of its product candidates, and any other statements containing the words “expect,” “will,” “target,” “plan,” and other 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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