Belite Bio Doses First Subject in Pivotal Phase 3 PHOENIX Trial Evaluating Oral Tinlarebant for GA

July 27, 2023

- Tinlarebant (a/k/a LBS-008) is Belite Bio's orally administered tablet intended to slow disease progression in subjects with Stargardt Disease (STGD1) and Geographic Atrophy (GA) in advanced Dry Age-related Macular Degeneration (Dry AMD)
- There are no approved oral or non-invasive treatments for GA
- Enrollment of approximately 430 GA subjects across multiple centers globally

SAN DIEGO, July 27, 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, in its pivotal Phase 3 "PHOENIX" trial for subjects with GA, the first subject has been dosed at the Retina Associates of Southern California, a clinical trial site in the United States.

"We are pleased to have successfully dosed the first subject in the Phase 3 PHOENIX trial for GA. GA is a chronic, and progressive condition that ultimately leads to blindness. Predominantly affecting an elderly population, the unmet need remains high with no currently FDA-approved oral or non-invasive treatments for GA," said Dr. Tom Lin, Belite's Chairman and CEO. "The encouraging data in our Phase 2 trial for STGD1, which shares similar pathophysiology as GA, supports the potential for Tinlarebant to change the treatment paradigm for GA, as the first oral treatment that can reduce disease progression."

Principal Investigator of the PHOENIX trial, Dr. Hani Salehi-Had, added, "The global PHOENIX trial plans to enroll GA patients with earlier-stage disease in an effort to prevent development of excessive retinal pathology and loss of vision. The continued trend of slowing expansion of autofluorescence and the reduction in incident atrophic retinal lesion growth rate with Tinlarebant in the ongoing Phase 2 STGD1 trial is encouraging and highlights Tinlarebant's potential as a treatment for GA. We are excited to be the first US site to successfully enroll the first subject into the PHOENIX trial and look forward to potentially having this oral treatment approved by FDA for our GA patients with Dry AMD."

Quan Dong Nguyen, MD, MSc, FAAO, FARVO, FASRS, Professor of Ophthalmology at the Byers Eye Institute, Stanford University School of Medicine, commented that, "Tinlarebant reduces formulation of toxic bisretinoids in the eye, but it does not interfere with the visual cycle. Its compelling safety profile seen up to 18-months in clinical trial is encouraging and supports the potential of Tinlarebant to be a much-needed and non-invasive treatment approach that can enable long-term dosing. Importantly, since Tinlarebant may be able to slow disease progression, there is also potential for Tinlarebant to treat across the disease spectrum, including intervening at earlier diseases stages such as intermediate AMD."

Phase 3 PHOENIX Trial Outline

- 2-year prospective, randomized (2:1, active:placebo, n~430 subjects), double-masked, placebo-controlled trial designed to assess the efficacy and safety of daily oral Tinlarebant.
- Enrollment of GA subjects across multiple centers globally has begun in the third quarter 2023.
- The primary endpoint will be based upon the slowing of DDAF lesion growth rate 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 trial.

For more information, visit clinicaltrials.gov at https://classic.clinicaltrials.gov/ct2/show/NCT05949593?term=Tinlarebant&draw=2&rank=3

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.

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.

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. Belite Bio is evaluating Tinlarebant in two ongoing 2-year trials in adolescent STGD1 subjects, including a Phase 2 trial and a global pivotal Phase 3 trial (the “DRAGON” trial).

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 trial (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 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, 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.

Media and Investor Relations Contact:
Jennifer Wu /ir@belitebio.com
Tim McCarthy /tim@lifesciadvisors.com