Belite Bio Receives Approval to Initiate a Second Stargardt Disease Clinical Trial in the United Kingdom

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- Tinlarebant is Belite Bio's orally administered tablet intended to slow disease progression in patients affected with Stargardt Disease (STGD1) and Geographic Atrophy (GA) in advanced Dry Age-related Macular Degeneration (Dry AMD)
- Data from a 24-month Phase 2 trial in adolescent STGD1 subjects showed a sustained lower atrophic lesion growth in Tinlarebant-treated subjects compared to ProgStar participants possessing similar baseline characteristics (aged ≤18 years) ($p<0.001$)
- In the Phase 2 trial, 5 of 12 subjects (42%) with known pathogenic *ABCA*4 mutations, no incident atrophic (DDAF) lesions were formed during the 24-month treatment period and no change in autofluorescent (QDAF) lesions was observed
- Enrollment of a pivotal global Phase 3 trial of Tinlarebant in adolescent STGD1 subjects ("DRAGON") has been completed with interim data expected in 4Q 2024
- Initiated DRAGON II trial of Tinlarebant in adolescent STGD1 patients
- Tinlarebant has been granted Orphan Drug and Sakigake (Pioneer Drug) Designation in Japan for the treatment of STGD1
- A global Phase 3 trial in GA ("PHOENIX") is ongoing

SAN DIEGO, June 18, 2024 (GLOBE NEWSWIRE)- Belite Bio, Inc (NASDAQ: BLTE) ("Belite" or the "Company"), a clinical-stage biopharmaceutical drug development company focused on advancing novel therapeutics targeting degenerative retinal diseases that have significant unmet medical needs, today announces the Medicines and Healthcare products Regulatory Agency (MHRA) approval for a second clinical trial of Tinlarebant in adolescent STGD1 in the United Kingdom ("DRAGON II"). The trial will initiate in the UK after the labeling requirements are met.
The DRAGON II trial is a combination of phase 1b open-label study in Japan to evaluate the pharmacokinetics and pharmacodynamics of Tinlarebant in Japanese adolescent STGD1 subjects and a phase 2/3, multicenter, double-masked, placebo-controlled, randomized study designed to evaluate the efficacy, safety and tolerability of Tinlarebant in adolescent STGD1 subjects. Approximately 60 subjects, aged 12 to 20 years old, including approximately 10 Japanese subjects, are targeted for enrollment in the phase 2/3 portion of the trial with a 1:1 randomization (tinlarebant:placebo). The data from Japanese subjects is intended to facilitate future NDA applications in Japan.

About Tinlarebant (a/k/a LBS-008)

Tinlarebant is a novel oral therapy that is intended to reduce the accumulation of vitamin A-based toxins (known as bisretinoids) that cause retinal disease in STGD1 and also contribute to disease progression in GA, or advanced Dry AMD. Bisretinoids 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 bisretinoids. Tinlarebant has been granted Fast Track Designation and Rare Pediatric Disease designation in the U.S., Orphan Drug Designation in the U.S. Europe, and Japan, and Sakigake Designation in Japan 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 progressive accumulation of bisretinoids 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 bisretinoids, retinal cell death, and progressive 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 is evaluating safety and efficacy of Tinlarebant in GA patients in a 2-year Phase 3 study (PHOENIX).

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.

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 (i) atrophic age-related macular degeneration (AMD), commonly known as Geographic Atrophy (GA) in advanced dry AMD, and (ii) autosomal recessive Stargardt disease type 1, or STGD1, 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 and plans, as well as 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, 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", "hope" 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 timing to complete relevant clinical trials and/or to receive the interim/final data of such clinical trials; 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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