Belite Bio Announces Completion of DRAGON, a 2-Year, Phase 3 Trial of Oral Tinlarebant in the Treatment of Stargardt Disease

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- Tinlarebant has been granted Breakthrough Therapy, Fast Track, and Rare Pediatric Disease Designations in the U.S.; Orphan Drug Designation in the U.S., Europe, and Japan; and Pioneer Drug Designation in Japan for Stargardt disease
- Last subject visit completed in the pivotal Phase 3 DRAGON trial of Tinlarebant in Stargardt disease
- Topline data expected in Q4 2025

SAN DIEGO, Sept. 12, 2025 (GLOBE NEWSWIRE) -- Belite Bio Inc (NASDAQ: BLTE) ("Belite" or the "Company"), a clinical-stage drug development company focused on advancing novel therapeutics targeting degenerative retinal diseases that have significant unmet medical needs, today announced the completion of the last subject visit in the Phase 3 DRAGON clinical trial evaluating Tinlarebant for the treatment of Stargardt disease type 1 (STGD1).

"We are very pleased to announce the successful completion of the DRAGON trial. This is an important milestone in our mission to bring a treatment to patients living with Stargardt disease," said Dr. Tom Lin, Chairman and CEO of Belite Bio. "With no approved therapies available today, Tinlarebant has the potential to be the first treatment for this devastating inherited macular degeneration. We are deeply grateful to the patients, families, investigators, and study teams worldwide who made this clinical trial possible."

The DRAGON trial enrolled 104 adolescent subjects across 11 jurisdictions, including the United States, United Kingdom, Germany, France, Belgium, Switzerland, Netherlands, China, Hong Kong, Taiwan, and Australia, with a 2:1 randomization (Tinlarebant:placebo). A total of 94 subjects completed the study, with the last study visit conducted on September 11, 2025. The primary efficacy endpoint is the growth rate of atrophic lesions; safety and tolerability of Tinlarebant will also be assessed.

Belite Bio expects to report top-line results from the DRAGON trial in Q4 2025 and plans to file New Drug Applications in 1H 2026.

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 geographic atrophy (GA), or advanced dry age-related macular degeneration (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 (Pioneer Drug) Designation in Japan for the treatment of STGD1.

About Belite Bio

Belite Bio is a clinical-stage drug development company focused on advancing novel therapeutics targeting degenerative retinal diseases that have significant unmet medical need, such as STGD1 and GA in advanced dry AMD, in addition to specific metabolic diseases. Belite's lead candidate, Tinlarebant, an oral therapy intended to reduce the accumulation of bisretinoid toxins in the eye, is currently being evaluated in a Phase 3 study (DRAGON) and a Phase 2/3 study (DRAGON II) in adolescent STGD1 subjects and a Phase 3 study (PHOENIX) in subjects with GA. For more information, follow us on X, Instagram, LinkedIn, and 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, the ability of Tinlarebant to treat Stargardt disease and geographic atrophy, 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 timing to submit trial data to regulatory authorities for drug 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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