Belite Bio Announces FDA Granting of Breakthrough Therapy Designation for Tinlarebant for the Treatment of Stargardt Disease

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- *Designation is based on the pivotal Phase 3 DRAGON trial interim analysis results demonstrating Tinlarebant's efficacy and favorable safety profile*
- *Trial completion expected by Q4 2025 (including a three-month follow-up period)*
- *Tinlarebant has previously been granted Fast Track and Rare Pediatric Disease Designations in the U.S., Orphan Drug Designation in the U.S., Europe, and Japan, and the Pioneer Drug Designation in Japan for Stargardt disease*

SAN DIEGO, May 21, 2025 (GLOBE NEWSWIRE) -- Belite Bio, Inc. (NASDAQ: BLTE), a clinical-stage biopharmaceutical drug development company focused on advancing novel therapeutics targeting degenerative retinal diseases that have significant unmet medical needs, today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for Tinlarebant for the treatment of Stargardt disease (STGD1) based on the previously reported interim data from the ongoing Phase 3 DRAGON trial. There are currently no approved treatments for STGD1.

"Breakthrough Therapy Designation is an exciting milestone that underscores Tinlarebant's potential to address a serious unmet need for patients with STGD1 — a condition where there are currently no approved therapies," said Dr. Tom Lin, Chairman and CEO of Belite Bio. "We remain deeply committed to the Stargardt community and to advancing Tinlarebant as we prepare for the DRAGON study readout expected by the end of this year."

The Breakthrough Therapy Designation is supported by a pre-specified interim analysis of the pivotal, global Phase 3 DRAGON trial data of Tinlarebant in adolescent STGD1 patients. The designation is based on preliminary clinical evidence indicating that a drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. In addition, according to the Data Safety and Monitoring Board (DSMB), Tinlarebant continued to demonstrate a favorable safety profile consistent with prior findings and its mechanism of action. Importantly, visual acuity remained stable in the majority of participants, with mean changes from baseline of less than three letters over the course of the two-year study.

"This granting represents a significant achievement for Belite Bio and serves as validation of a therapeutic approach that directly targets the underlying pathophysiology of STGD1 in order to slow or halt the disease process," stated Dr. Nathan L. Mata, Belite Bio's Chief Scientific Officer. Dr. Mata noted further, "Although it has been more than 26 years since the development of the first STGD1 mouse model, it is encouraging to know that the learnings from this model, and the many years spent evaluating the therapeutic benefit of targeting retinol binding protein 4 (RBP4) to reduce the accumulation of cytotoxic byproducts of vitamin A, have advanced our understanding of the disease and have led us closer than ever to the realization of an approved treatment for patients living with STGD1."

"We are very encouraged by the FDA's decision. STGD1 is a progressive condition that typically begins in adolescence and inevitably leads to legal blindness. People living with STGD1 experience a severe loss of quality of life even though they typically have decades of their lives ahead of them," said Dr. Hendrik Scholl, Chief Medical Officer of Belite Bio. "Breakthrough Therapy Designation reinforces the importance of our work to develop a potential therapy for people who currently have limited options. We look forward to continuing to evaluate Tinlarebant's safety and efficacy as the DRAGON trial progresses."

About FDA Breakthrough Therapy Designation
The FDA grants Breakthrough Therapy Designation to expedite the development and regulatory review of drugs that are intended for serious or life-threatening conditions. The designation is based on preliminary clinical evidence indicating that a drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. Breakthrough Therapy Designation affords all of the benefits of the fast-track program, including eligibility for rolling review and priority review, as well as additional regulatory engagement to facilitate expedited development, with the aim to bring therapies to patients more quickly.

About Phase 3 DRAGON
The pivotal Phase 3 DRAGON trial is a randomized, double-masked, placebo-controlled, global study designed to evaluate the safety and efficacy of Tinlarebant in adolescent patients with Stargardt disease. The trial enrolled 104 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). The primary efficacy endpoint is the growth rate of atrophic lesions, alongside the assessment of safety and tolerability.

About Tinlarebant
Tinlarebant is an orally administered, once-a-day tablet intended as an early intervention for maintaining the health and integrity of retinal tissues in

Stargardt disease type 1 (STGD1) and Geographic Atrophy (GA) patients. Currently, there are no FDA approved treatments for STGD1 and no approved orally administered treatments for GA. Therefore, if approved, Tinlarebant would be a novel oral therapeutic addressing an unmet medical need in both STGD1 and GA.

About Belite Bio

Belite Bio is a clinical-stage biopharmaceutical drug development company focused on advancing novel therapeutics targeting degenerative retinal diseases that have significant unmet medical needs, such as Stargardt disease type 1 (STGD1) and Geographic Atrophy (GA) in advanced dry age-related macular degeneration (AMD), in addition to specific metabolic diseases. Belite's lead candidate, Tinlarebant, an oral therapy intended to reduce the accumulation of 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, interim analysis and recommendation from DSMB; Belite Bio's advancement of, and anticipated future activities on preclinical studies, clinical development, regulatory milestones, and commercialization of its product candidates; and any other statements containing the words "expect", "hope", "indicate", "look forward to", 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 of potential submission with FDA; 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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