Belite Bio Announces Interim Analysis Results from the Pivotal Global Phase 3 DRAGON trial of Tinlarebant in Adolescent Stargardt Disease Subjects

February 27, 2025

- *An independent Data Safety Monitoring Board (DSMB) for the Phase 3 DRAGON trial recommends trial continuation without any modifications, maintaining the sample size at 104 subjects following a planned interim efficacy analysis; trial completion expected by Q4 2025 (including a three-month follow-up period)*
- *Tinlarebant has 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, for which there are no approved treatments*

SAN DIEGO, Feb. 27, 2025 (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 need today announced that following a pre-specified Interim Analysis of the pivotal global Phase 3 "DRAGON" trial data of Tinlarebant in adolescent Stargardt disease patients, the Data Safety Monitoring Board (DSMB) has recommended the trial proceed without any modifications. The Interim Analysis was performed when all subjects completed the one-year assessment.

The study design for the DRAGON trial included an adaptive sample size re-estimation that would determine the need for an increase in sample size in order to enhance power, based on a treatment effect observed at the Interim Analysis. The recommendation by the DSMB that the trial should proceed without modifications indicates that a sample size increase is not warranted. In addition, the DSMB recommended to submit the data for further regulatory review for drug approval.

According to the DSMB, Tinlarebant is well-tolerated and the safety profile remains consistent with previously observed data and the mechanism of action for Tinlarebant. In addition, visual acuity was stabilized in the majority of subjects, with mean change from baseline of less than three letter scores under both standard and low luminance, throughout the two-year study.

"We are pleased to have reached this important trial milestone and are excited by the safety profile that we continue to observe for Tinlarebant," said Dr. Hendrik Scholl, Chief Medical Officer of Belite Bio. "Following the DSMB's recommendation to continue the trial with the current sample size, we remain on track to complete the trial by Q4 2025, including a three-month follow-up period, and look forward to building on the promising efficacy results observed in our completed Phase 2 trial for Tinlarebant."

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 Stargardt disease patients. The DRAGON trial has sites in 11 jurisdictions, including the U.S., the United Kingdom, Germany, France, Belgium, Switzerland, Netherlands, China, Hong Kong, Taiwan, and Australia. The study enrolled 104 subjects with a 2:1 randomization (active:placebo). The primary efficacy endpoint is the growth rate of atrophic lesion, along with the assessment of safety and tolerability of Tinlarebant. Tinlarebant has been granted Orphan Drug Designation in the United States, Europe, and Japan, Rare Pediatric Disease (RPD) designation and Fast Track Designation in the U.S., and Sakigake (Pioneer Drug) Designation in Japan.

Webcast Information
Date: Thursday, February 27, 2025
Time: 4:30 p.m. Eastern Time (1:30 p.m. Pacific Time)
Webcast Link: https://event.summitcast.com/view/P4wkBPHuQY4sc9AqJPx7QV/9Z6J7TJewijZNsCPdTmZy7

Webcast Link Instructions
You can join the live webcast by visiting the link above or the "Presentations & Events" section of the Company's Investor Relations website at https://investors.belitebio.com/presentations-events/events. A replay will be available following the event.

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 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, 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 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.

Media and Investor Relations Contact:

Jennifer Wu /ir@belitebio.com

Julie Fallon /belite@argotpartners.com