Belite Bio Announces UK's Medicines and Healthcare Products Regulatory Agency Agrees to Conditional Marketing Authorization Application Based on Interim Analysis Results for the Treatment of Stargardt Disease with Tinlarebant

November 2, 2025

- *MHRA response is based on the Phase 3 DRAGON interim analysis results*
- *Topline final data expected in Q4 2025*

SAN DIEGO, Nov. 02, 2025 (GLOBE NEWSWIRE) -- Belite Bio, Inc. (NASDAQ: BLTE), a clinical-stage drug development company focused on advancing novel therapeutics targeting degenerative retinal diseases that have significant unmet medical needs, today announced that United Kingdom's Medicines and Healthcare Products Regulatory Agency (MHRA) has agreed to accept a Conditional Marketing Authorization application for Tinlarebant for the treatment of Stargardt disease based on the interim analysis results from the Phase 3 DRAGON trial.

"We are extremely pleased with the outcome of our engagement with the MHRA. This is an incredibly exciting time for the Belite team as we see our perseverance bringing us closer to offering an effective therapy to patients living with Stargardt disease, who currently have no approved treatment options," said Dr. Tom Lin, Chairman and CEO of Belite Bio. "We look forward to continuing our work with regulatory authorities as we advance Tinlarebant through late-stage development and toward the possibility of delivering the first approved therapy for this devastating disease."

"With consistent feedback from major agencies across the world, we are encouraged that the DRAGON trial provides a strong foundation for global submissions and potential approvals," said Dr. Hendrik Scholl, Chief Medical Officer of Belite Bio.

MHRA's response is based on the interim analysis results which fulfil the criteria for a Conditional Marketing Authorization application. The Company remains on track to report final topline data from the Phase 3 DRAGON trial in the fourth quarter of 2025. These results are expected to be submitted to the MHRA for full Marketing Authorization Application.

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 U.S., 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 (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 Breakthrough Therapy Designation, 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. In addition, even if MHRA agreed that the Company may submit the Conditional Marketing Authorization application for Tinlarebant for the treatment of Stargardt disease based on the interim analysis results from Phase 3 DRAGON trial, the Company is still subject to all applicable data, document and procedural requirements of MHRA for the Company's Marketing Authorization Application. 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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