Belite Bio Receives Orphan Drug Status for Tinlarebant in Stargardt Disease in Switzerland

May 18, 2026

- *Tinlarebant is the first therapeutic candidate to demonstrate clinical efficacy in Stargardt disease, having met the primary endpoint, reductions in lesion growth rate, in the pivotal, global Phase 3 DRAGON trial*
- *Company recently initiated a rolling submission of a New Drug Application (NDA) with the U.S. Food and Drug Administration (FDA), which it expects to complete in 2Q 2026*
- *Tinlarebant has previously 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 the treatment of Stargardt disease*

SAN DIEGO, May 18, 2026 (GLOBE NEWSWIRE) -- Belite Bio, Inc (NASDAQ: BLTE) ("Belite Bio ®" 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 that the Swiss Agency for Therapeutic Products (Swissmedic) has granted tinlarebant, the Company's lead product candidate, orphan drug status (ODS) for the treatment of Stargardt disease (STGD1), a rare, inherited retinal disorder caused by mutations in the ABCA4 gene.

"As the first therapy to demonstrate a clinically meaningful treatment effect in Stargardt disease in a registration trial, tinlarebant provides a potential option for people living with this debilitative disease where there are no currently approved treatments. The importance of bringing this therapy to market is further underscored by the granting of ODS from Swissmedic," said Dr. Hendrik Scholl, Chief Medical Officer of Belite Bio. "Following the successful Phase 3 DRAGON trial, where tinlarebant demonstrated an unprecedented 35.7% reduction in the growth rate of retinal lesions, as measured by retinal imaging, we look forward to bringing this therapy to patients affected by Stargardt disease."

In December 2025, Belite Bio reported positive results from the Phase 3 DRAGON trial, which was 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 worldwide, including Switzerland, with a 2:1 randomization (tinlarebant:placebo). The primary efficacy endpoint was the growth rate of atrophic lesions, alongside the assessment of safety and tolerability. The trial met its primary efficacy endpoint, demonstrating a statistically significant and clinically meaningful 35.7% reduction in the growth rate of retinal lesions, measured as definitely decreased autofluorescence (DDAF) by fundus autofluorescence imaging, compared with placebo.

About Swissmedics' Orphan Drug Status
Swissmedic grants ODS to encourage the development of treatments for rare, life-threatening, or chronic debilitating diseases that effect less than 5 in 10,000 people in Switzerland through regulatory and financial incentives. Incentives include eligibility for 15 years of document protection (compared to the standard 10 years), qualification for accelerated review timelines, fee reductions, potentially including waiving of the new marketing authorization application flat-rate fee, and potential initiation of an Early Access Pathway, which allows patients to access critical treatments before full marketing authorization is completed.

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 Stargardt disease 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, Japan, and Switzerland, and Sakigake Designation in Japan for the treatment of Stargardt disease.

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 Stargardt disease type 1 and geographic atrophy, in addition to specific metabolic diseases. Belite Bio's lead candidate, tinlarebant, is an oral therapy intended to reduce the accumulation of bisretinoid toxins in the eye. The Company has completed a Phase 3 trial (DRAGON) in adolescent and adult Stargardt disease subjects, and the drug is currently being evaluated in a Phase 2/3 trial (DRAGON II) in adolescent and adult Stargardt disease subjects and a Phase 3 trial (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 regarding future expectations, plans and prospectus, 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 STGD1 and GA, 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, as well as any other statements regarding matters that are not historical facts, and any other statements containing the words "expect", "believe", "target", "plan", "hope", "potential" and other similar expressions. Actual results may differ materially from those indicated in the forward-looking statements as a result of various important factors related to Belite Bio's business, 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; expectations for the timing of initiation, enrollment and completion of, and data relating to, its clinical trials; the timing to complete any ancillary clinical trials and/or to receive the interim/final data of such clinical trials; the timing to communicate with and submit trial data to regulatory authorities for drug approval in various jurisdictions; the content and timing of decisions made by the relevant regulatory authorities regarding regulatory approval of Belite Bio's drug candidates; timing for Belite Bio to share additional data at upcoming medical meetings; the potential efficacy of tinlarebant to set a new benchmark for future research in inherited retinal disorders, 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
Argot Partners / belite@argotpartners.com