



Belite Bio Receives Sakigake (Pioneer Drug) Designation of Tinalarebant for Stargardt Disease in Japan

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- Tinalarebant is Belite Bio's orally administered tablet intended to slow disease progression in patients affected with Stargardt Disease (STGD1) and Geographic Atrophy (GA) in advanced Dry Age-related Macular Degeneration (Dry AMD)
- Data from a 24-month Phase 2 trial in adolescent STGD1 subjects showed a sustained lower atrophic lesion growth in Tinalarebant-treated subjects compared to ProgStar participants possessing similar baseline characteristics (aged ≤18 years) ($p < 0.001$)
- In the Phase 2 trial, 5 of 12 subjects (42%) with known pathogenic *ABCA4* mutations, no incident atrophic (DDAF) lesions were formed during the 24-month treatment period and no change in autofluorescent (QDAF) lesions was observed
- Enrollment of a pivotal global Phase 3 trial of Tinalarebant in adolescent STGD1 subjects ("DRAGON") has been completed with interim data expected in 4Q 2024
- Initiated DRAGON II trial of Tinalarebant in adolescent STGD1 patients
- Tinalarebant has been granted Orphan Drug Designation in Japan for the treatment of STGD1
- A global Phase 3 trial in GA ("PHOENIX") is ongoing

SAN DIEGO, June 12, 2024 (GLOBE NEWSWIRE) -- [Belite Bio](#), Inc (NASDAQ: BLTE) ("Belite Bio" or the "Company"), a clinical-stage biopharmaceutical drug development company focused on advancing novel therapeutics targeting degenerative retinal diseases that have significant unmet medical needs, today announces that its lead pipeline, Tinalarebant, has been granted Sakigake Designation by the Ministry of Health, Labour and Welfare in Japan (MHLW) for the treatment of STGD1.

Sakigake designation was established by MHLW to accelerate drug approval process in Japan for innovative drugs with prominent effectiveness targeting serious diseases, in order to make them available to patients in Japan ahead of the rest of the world, by providing (a) prioritized consultation, (b) pre-application consultation, (c) prioritized review, (d) assignment of a review partner, and (e) extension of re-examination period.

About Tinalarebant (a/k/a LBS-008)

Tinalarebant 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 GA, or advanced Dry AMD. Bisretinoids are by-products of the visual cycle, which is dependent on the supply of vitamin A (retinol) to the eye. Tinalarebant 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, Tinalarebant reduces the formation of bisretinoids. Tinalarebant 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 Designation in Japan for the treatment of STGD1.

Stargardt Disease (STGD1)

STGD1 is the most common inherited retinal dystrophy (causing blurring or loss of central vision) in both adults and children. The disease is caused by mutations in a retina-specific gene (*ABCA4*), which results in progressive accumulation of bisretinoids leading to retinal cell death and progressive loss of central vision. The fluorescent properties of bisretinoids and the development of retinal imaging systems have helped ophthalmologists identify and monitor disease progression. Currently, there are no FDA approved treatments for STGD1.

Importantly, STGD1 and GA, or advanced Dry AMD, share a similar pathophysiology, which is characterized by the excessive accumulation of bisretinoids, retinal cell death, and progressive loss of vision. Vision loss occurs slowly, despite peripheral expansion of "dead retina," until the disease reaches the center of the eye (the macula). Therefore, Belite Bio is evaluating safety and efficacy of Tinalarebant in GA patients in a 2-year Phase 3 study (PHOENIX).

GA in advanced Dry Age-related Macular Degeneration (Dry AMD)

Dry AMD is a leading cause of vision loss in older adults. Geographic Atrophy, or GA, is the advanced stage of AMD. Currently, there are no FDA approved orally administered treatments for GA and no FDA approved therapies for the other stages of Dry AMD other than GA. There are an estimated 20 million AMD patients in the U.S. and over 196 million patients worldwide with an estimated global direct healthcare cost of US\$255 billion.

About Belite Bio

Belite Bio is a clinical-stage biopharmaceutical drug development company focused on advancing novel therapeutics targeting retinal degenerative eye diseases which have significant unmet medical needs such as (i) atrophic age-related macular degeneration (AMD), commonly known as Geographic Atrophy (GA) in advanced dry AMD, and (ii) autosomal recessive Stargardt disease type 1, or STGD1, in addition to specific metabolic diseases. 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, and Belite Bio's advancement of, and anticipated preclinical activities, clinical development, regulatory milestones, and commercialization of its product candidates, 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 content and timing of decisions made by the relevant regulatory authorities regarding regulatory approval of Belite Bio's drug candidates; the potential efficacy of Tnlarebant, 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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