Belite Bio

Fact Sheet

BLTE

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QUICK REFERENCE

Belite Bio Inc.
NASDAQ: BLTE

Website: **www.BELITEBIO.com**

BUSINESS SUMMARY

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. Safety and efficacy of Belite's lead candidate, Tinlarebant, an oral therapy intended to reduce the accumulation of vitamin A-based toxins in the eye, is currently being evaluated in three late-stage clinical trials. In STGD1, a 2-year, Phase 3 study (DRAGON) and a 2-year, Phase 2/3 study (DRAGON II) in adolescent STGD1 subjects are currently ongoing. In GA, a 2-year, Phase 3 study (PHOENIX) is ongoing.

PIPELINE

Tinlarebant, or LBS-008, if approved, would provide a novel treatment option. Tinlarebant is a novel, once-a-day oral therapy which is intended to reduce the accumulation of toxins in the eye that cause Stargardt Disease (STGD1) and contribute to Geographic Atrophy (GA), or advanced dry AMD. These toxins 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 these toxins. In clinical trials, Tinlarebant has demonstrated its target specificity and potency that we believe could be clinically meaningful to treat (STGD1) patients and GA patients.

Tinlarebant is a potent, orally administered small molecule RBP4 antagonist that has been specifically designed to reduce the delivery of retinol to the eye as a therapeutic approach towards reducing the accumulation of cytotoxic bisretinoids, preserving the integrity of retinal tissues, and ultimately slowing or preventing loss of vision. The delivery of retinol to the RPE requires RBP4 and the RPE expresses a specific RBP4 receptor (STRA6) to regulate vitamin A uptake. Other extrahepatic tissues do not require delivery of retinol bound to RBP4 and do not express the RBP4 receptor. These tissues are able to take up vitamin A bound to non-specific carriers such as lipoproteins, triglycerides, and albumin.

Tinlarebant was selected by the National Institute of Health (NIH) Blueprint Neurotherapeutics Network, (BPN) in 2011 as a promising drug candidate for treating dry AMD. The BPN was launched in 2004 to foster small-molecule neurotherapeutic development, bringing together a unique blend of grant dollars, industry-standard scientific expertise, and contract resources under a milestone-driven cooperative agreement program.

LBS-009 is an anti-RBP4 oral therapy targeting liver disease, including non-alcoholic fatty liver disease (NAFLD), non-alcoholic steatohepatitis (NASH), and type 2 diabetes (T2D), which cumulatively impact more than 2.4 billion patients worldwide. LBS-009 is a small molecule designed to compete with retinol for binding to RBP4, the primary carrier of vitamin A (retinol) from the liver to extra-hepatic target tissues. When bound to LBS-009, RBP4 can no longer bind retinol and cannot interact with a key accessory protein (transthyretin) which would otherwise prevent elimination of RBP4 from the circulation. Consequently, the RBP4-LBS-009 complex will be removed from the circulation by renal filtration. The available data suggest that modulation of circulating RBP4 with an RBP4 antagonist, like LBS-009, has the potential to provide a therapeutic benefit for patients suffering from metabolic diseases, including NAFLD, NASH and T2D. LBS-009 is currently in preclinical development.

CONTACT INFORMATION

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Recent Press Releases *(Headline and Excerpts)*

Belite Bio Announces First Patient Dosed in Phase 2/3 DRAGON II Trial of Tinlarebant for the Treatment of Stargardt Disease

- ***Phase 2/3 of the DRAGON II trial will evaluate the efficacy, safety, and tolerability of Tinlarebant in approximately 60 adolescent STGD1 subjects across the U.S., U.K., and Japan***
- ***Phase 1b of DRAGON II trial to evaluate pharmacokinetics and pharmacodynamics of Tinlarebant in Japanese subjects was recently completed***
- ***Tinlarebant granted Orphan Drug and Sakigake (Pioneer Drug) Designation in Japan for the treatment of STGD1***
- ***Pivotal global Phase 3 trial of Tinlarebant in GA subjects ("PHOENIX") is ongoing and more than 200 subjects have been enrolled***

Sept. 10, 2024 -- Belite Bio, Inc announced that the first patient has been dosed at the Tokyo Medical Center in the Phase 2/3 portion of its DRAGON II clinical trial evaluating Tinlarebant for the treatment of STGD1. Tinlarebant is a novel oral therapy designed to reduce the accumulation of vitamin A based toxins that cause retinal disease, and this Phase 2/3 study will evaluate the efficacy, safety, and tolerability of Tinlarebant in approximately 60 adolescent STGD1 subjects across the U.S., U.K., and Japan.

The DRAGON II trial is a combination of Phase 1b open-label study in Japan to evaluate the pharmacokinetics (PK) and pharmacodynamics (PD) of Tinlarebant, administered daily for 7 days in Japanese adolescent STGD1 subjects and a Phase 2/3, multicenter (U.S., U.K., and Japan), double-masked, placebo-controlled, randomized study designed to evaluate the efficacy, safety, and tolerability of Tinlarebant, administered daily for 24 months in adolescent STGD1 subjects. Approximately 60 subjects, aged 12 to 20 years old, including approximately 10 Japanese subjects, are targeted for enrollment in the Phase 2/3 portion of the trial with a 1:1 randomization (Tinlarebant:placebo). The data from the Japanese subjects is intended to facilitate future NDA applications in Japan.

Belite Bio to Participate in Three Upcoming Investor Conferences

Sept. 03, 2024 -- Belite Bio, Inc. (NASDAQ: BLTE) announced that the executive management team will participate in three upcoming investor conferences. Details for the presentations are as follows:

- H.C. Wainwright 26th Annual Global Investment Conference (New York, New York)
  September 9, 2024, at 9:30 am ET, fireside chat
- 2024 Cantor Global Healthcare Conference (New York, New York)
  September 18, 2024, at 3:40 pm ET, corporate presentation
- Deutsche Bank Depositary Receipts Virtual Investor Conference (Virtual)
  September 25, 2024, at 11:00 am ET, corporate presentation

A webcast of each presentation can be accessed under the "Events" tab on the investor relations section of the Belite Bio website at: https://investors.belitebio.com/presentations-events/events. The replays will be archived for 90 days following the presentation date.

Belite Bio Announces Appointment of Hendrik P. N. Scholl, MD, MA, as Chief Medical Officer

- *Dr. Scholl is a globally recognized leader in the field of ophthalmology and the coordinating principal investigator of the largest ever natural history study of Stargardt disease*

Sept. 01, 2024 -- Belite Bio, Inc announced that its board of directors has appointed Hendrik P. N. Scholl, MD, MA, as the Chief Medical Officer of the Company, effective immediately. Dr. Scholl is the foremost globally recognized authority on Stargardt disease and age-related macular degeneration (AMD), bringing decades of expertise in treating retinal diseases, including the two key indications targeted by Belite Bio's lead drug candidate, Tinlarebant.

Dr. Scholl served as the founding and scientific co-director of the Institute of Molecular and Clinical Ophthalmology Basel (IOB) and Professor of Ophthalmology at the University of Basel, where he also led the Department of Ophthalmology as its Chairman. He currently serves as President of the European Vision Institute as well as Chairman of the largest clinical research network in ophthalmology in Europe, EVICR.net, and its Expert Committee on Retinal Dystrophies. He is also the Founder and President of the Swiss Association for Research in Vision and Ophthalmology (ARVO-SWISS).
Dr. Scholl's distinguished career in academia includes leadership positions at several key academic institutions. Recently, he served as Professor of Ophthalmology and Endowed Chair at the Wilmer Eye Institute of Johns Hopkins University Medical School. At the Johns Hopkins Hospital, he was the Head of the Retinal Degeneration Clinic and the Director of the Visual Neurophysiology Service. For the Wilmer Eye Institute, he also served as the Co-director of the Johns Hopkins Center for Stem Cells and Ophthalmic Regenerative Medicine.

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