Belite Bio Presented Interim Results of LBS-008 Phase 1b/2 Study in Adolescent STGD1 at ARVO Annual Meeting 2022

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- *LBS-008 (aka Tinlarebant) showed an acceptable safety profile in adolescent Stargardt disease (STGD1) subjects*
- *A trend for stabilized or improved visual acuity was observed*
- *Observed adverse events were anticipated based on the mechanism of LBS-008 action*
- *STGD1 subjects from the Phase 1b study are now participating in the 2-year, Phase 2 extension*

SAN DIEGO, May 05, 2022 (GLOBE NEWSWIRE) -- Belite Bio, Inc (the "Company") (Nasdaq: BLTE), a San Diego based clinical stage biopharmaceutical drug development company targeting currently untreatable eye diseases, such as atrophic Age-related Macular Degeneration (dry AMD) and Stargardt disease (STGD1), and metabolic diseases, presented interim results of a Phase 1b/2 study of LBS-008 in adolescent STGD1 at The Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting 2022. LBS-008 is Belite Bio's lead asset being developed for the treatment of STGD1 and Dry AMD. To view the full abstract click here.

John Grigg, the study's principal investigator and Head Specialty of Ophthalmology at the University of Sydney and Consultant Ophthalmologist at the Sydney Children's Hospitals Network Westmead and Sydney Eye Hospital provided a presentation of the study and data.

Study Background
STGD1 is the most common inherited macular dystrophy (causing blurring or loss of central vision). To date there are no treatments. This Phase 1b/2 study examined the safety and tolerability of LBS-008, a retinol binding protein 4 (RPB4) antagonist, in adolescent STGD1 subjects. Preclinical studies have shown that RBP4 inhibition slows disease progression and prevents retinal degeneration in a STGD1 animal model.

Disease
Childhood-onset STGD1 is characterized by accumulation of bisretinoids which cause progressive retinal atrophy leading to rapid visual loss. Because bisretinoid toxins are derived from circulating vitamin A (retinol), reduction of retinol delivery to the eye, via antagonism of RPB4, has been explored as a means to slow disease progression in STGD1. LBS-008 is an orally administered, potent and specific non-retinoid antagonist of RPB4 and has been developed in order to determine whether reduction of circulating RPB4-retinol is a safe and effective treatment approach for STGD1 subjects.

Trial Design

- The Phase 1b/2 study is a multicenter, single arm, open-label study followed by a 2-year extension to evaluate safety, tolerability and efficacy of LBS-008.
- Thirteen subjects aged 12-18 years will receive oral LBS-008 (5 mg) daily over a 2-year treatment period. One (1/13) subject is dosing at 2mg a day.
- Treatment emergent adverse events (TEAEs), PK/PD, and visual function outcomes will be evaluated.

Results

- All 13 subjects have received at least 7 months of treatment and have completed the scheduled assessments at the first 6-month interval.
- The preliminary safety results of the Phase 1b/2 study show that the only drug-related adverse events reported were: delayed dark adaptation (DDA), reported by 9 of 13 subjects (or 69.2%) of which, 6 subjects having recovered; xanthopsia/chromatopsia was reported by 9 subjects (or 69.2%), with 3 subjects having recovered; and night vision impairment, reported by 1 patient (or 7.7%). All AEs were graded as mild. No severe AEs were reported and no AEs required discontinuation of treatment.
- No clinically significant findings in relation to vital signs, physical exams, cardiac health, or organ functions.
- The majority of subjects (8 of 13, 61.5%) recorded a gain in BCVA (ETDRS score) in at least one eye indicating stabilization of visual acuity, including 2 subjects with visual improvement in both eyes.
- Retinal imaging by fundus autofluorescence photography showed a trend for preventing or slowing expansion of autofluorescence. Areas of questionably decreased autofluorescence were either unchanged or reduced in one or both

eyes in 8 of 13 subjects. There was no conversion to definitely decreased autofluorescence (atrophic retina) in 12 of 13 subjects.
- 6 subjects showed an improvement (narrowing) of the Ellipsoid Zone defect width in at least one eye, including 3 subjects showing improvements in both eyes.
- Where quantitative autofluorescence was available qAF was correlated with improvements in VA.

Conclusions

- LBS-008 (5 mg p.o., daily) effectively reduces RBP4 levels by 80-90%.
- Safe and well tolerated at the 7-month time point
- A trend for preventing or slowing expansion of autofluorescence
- A trend for improvement of the EZ defect width
- Visual acuity has stabilized in a majority of subjects

The Company expects the next near-term data readout in its STGD1 Phase 2 trial to occur in the last quarter of 2022 when all subjects have completed 12 months of treatment.

About LBS-008

LBS-008 is a novel oral therapy that prevents the buildup of toxins in the eye that cause STGD1 and contribute to dry AMD. These toxins are by-products of the visual cycle, which is dependent on the supply of vitamin A (retinol) to the eye. LBS-008 works by reducing and maintaining the levels of serum retinol binding protein 4 (RBP4), a carrier protein that transports retinol to the eye. By modulating the amount of retinol entering the visual cycle, LBS-008 reduces the formation of the toxins which have been implicated in progression of STGD1 and dry AMD in order to maintain the health of retinal tissues. LBS-008 has been granted Fast Track Designation by the U.S. Food and Drug Administration (FDA) for the treatment of STGD1.

Dry Age-related Macular Degeneration

Dry AMD is a leading cause of vision loss in the U.S. for which no approved treatments are available. There are an estimated 11 million dry 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 San Diego based clinical stage biopharmaceutical drug development company targeting currently untreatable eye diseases, such as dry AMD and Stargardt disease, and 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 certain "forward-looking statements" within the meaning of federal securities laws. All statements, other than statements of historical facts, included herein are "forward-looking statements" including, among other things, statements about Belite's beliefs and expectations. The expectations reflected in these forward-looking statements involve significant assumptions, risks and uncertainties, and these expectations may prove to be incorrect. Investors should not place undue reliance on these forward-looking statements, which speak only as of the date of this press release. Potential risks and uncertainties include, but are not limited to, risks discussed in Belite's filings with the U.S. Securities and Exchange Commission at www.sec.gov. Other than as required under the securities laws, the Company does not assume a duty to update these forward-looking statements.

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