Belite Bio Reports First Quarter 2025 Financial Results and Provides Corporate Update

May 14, 2025

- Following a pre-specified interim analysis, an independent Data Safety Monitoring Board (DSMB) recommended the pivotal Phase 3 trial (DRAGON) of Tinlarebant in adolescent Stargardt disease (STGD1) patients proceed without any modification; trial completion expected Q4 2025 (including a three-month follow-up period)
- DSMB also recommended the Company submit the interim data for further regulatory review for drug approval
- A pivotal global Phase 3 trial (PHOENIX) of Tinlarebant in geographic atrophy (GA) patients is ongoing with 464 of targeted 500 subjects enrolled
- Raised $15 million in gross proceeds in a registered direct offering on February 5, 2025
- Conference call and webcast on Wednesday, May 14, 2025, at 4:30 p.m. ET

SAN DIEGO, May 13, 2025 (GLOBE NEWSWIRE) -- Belite Bio, Inc (NASDAQ: BLTE), a clinical-stage biopharmaceutical drug development company focused on advancing novel therapeutics targeting degenerative retinal diseases that have significant unmet medical needs, today announced its financial results for the first quarter ended March 31, 2025, and provided a business update.

"We continue to advance the clinical development of Tinlarebant, reaching a major milestone with the favorable interim analysis of our Phase 3 DRAGON trial earlier this year," said Dr. Tom Lin, Chairman and CEO of Belite Bio. "We are excited by the encouraging feedback from the DSMB on the safety and efficacy outcomes in DRAGON as we work toward trial completion by the end of 2025. We are focused on maintaining strong execution across our late-stage clinical programs as we aim to deliver new treatment options for people living with degenerative retinal diseases, where there is significant unmet need."

First Quarter 2025 Business Highlights and Upcoming Milestones:

Clinical Highlights

Tinlarebant is an oral, once-daily, potent retinol binding protein 4 (RBP4) antagonist that decreases RBP4 levels in the blood and reduces vitamin A (retinol) delivery to the eye without disrupting systemic retinol delivery to other tissues. Vitamin A is critical for normal vision but can accumulate as toxic byproducts (bisretinoids) in individuals affected with STGD1 and GA, the advanced form of dry age-related macular degeneration (AMD), leading to retinal cell death and loss of vision.

- **Stargardt disease (STGD1):** Accumulation of cytotoxic bisretinoids compounds has been implicated in the onset and progression of STGD1, for which there are no approved treatments. Tinlarebant has been granted Fast Track and Rare Pediatric Disease Designations 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.
    - DRAGON Trial: Ongoing, 24-month, randomized (2:1, active: placebo), double-masked, placebo-controlled, global, multi-center, pivotal Phase 3 trial in adolescent STGD1 patients
        - Following a pre-specified interim analysis, an independent DSMB recommended trial continuation without modifications, maintaining a sample size of 104 subjects
        - In addition, the DSMB recommended submitting the data for further regulatory review for drug approval
        - Primary efficacy endpoint is the growth rate of atrophic lesions; safety and tolerability will also be assessed
        - Trial completion expected by Q4 2025 (including a three-month follow-up period)
    - DRAGON II Trial: Combination of a Phase 1b open-label trial to evaluate the pharmacokinetics and pharmacodynamics of Tinlarebant in adolescent Japanese STGD1 patients and a Phase 2/3, 24-month, randomized (1:1, active: placebo), double-masked, placebo-controlled, multicenter trial in adolescent STGD1 patients
        - Enrolled 16 subjects in the Phase 2/3 trial, with a target enrollment of approximately 60 subjects, aged 12 to 20 years old, including approximately 10 Japanese subjects; data from the Japanese subjects is intended to facilitate a future new drug application in Japan
        - Primary efficacy endpoint is the growth rate of atrophic lesions; safety and tolerability will also be assessed

- **Geographic Atrophy (GA):** GA is a chronic degenerative disease of the retina that leads to blindness in the elderly. Accumulation of cytotoxic vitamin A byproducts (bisretinoids) has been implicated in the progression of GA. There are currently no FDA-approved, orally administered treatments for GA.
    - PHOENIX Trial: Ongoing, 24-month, randomized (2:1, active: placebo), double-masked, placebo-controlled, global, multi-center, pivotal Phase 3 trial in GA patients
        - 464 of the targeted 500 subjects have been enrolled to date
        - Primary efficacy endpoint is the growth rate of atrophic lesions; safety and tolerability will also be assessed
        - Company expects to conduct an interim analysis

Corporate Highlights

- In February 2025, Belite completed a registered direct offering priced at the market, raising gross proceeds of $15 million, with the potential for additional proceeds of approximately $15 million from the exercise of five-year warrants issued in the offering.

First Quarter 2025 Financial Results:

Current Assets:

As of March 31, 2025, the Company had $157.4 million in cash, liquidity funds, time deposits, and U.S treasury bills.

R&D Expenses:

For the three months ended March 31, 2025, research and development expenses were $9.4 million compared to $6.8 million for the same period in 2024. The increase in research and development expenses was primarily attributable to (i) share-based compensation granted in the third quarter of 2024 and first quarter of 2025, (ii) slightly higher clinical trial expenses related to the PHOENIX trial.

G&A Expenses:

For the three months ended March 31, 2025, general and administrative expenses were $6.1 million compared to $1.6 million for the same period in 2024. The increase resulted primarily from an increase in share-based compensation granted in the third quarter of 2024 and first quarter of 2025.

Other Income:

For the three months ended March 31, 2025, other income was $1.2 million compared to $0.5 million for the same period in 2024. The increase in other income was attributable to accrued interest from time deposits and U.S. treasury bills.

Net Loss:

For the three months ended March 31, 2025, the Company reported a net loss of $14.3 million, compared to a net loss of $7.9 million for the same period in 2024.

Webcast Information

Belite Bio will host a webcast on Wednesday, May 14, 2025, at 4:30 p.m. Eastern Time to discuss the Company's financial results and provide a business update. To join the webcast, please visit https://events.q4inc.com/attendee/137642555. A replay will be available for approximately 90 days following the event at the Company's Investor Relations website at https://investors.belitebio.com/presentations-events/events.

About Belite Bio

Belite Bio is a clinical-stage biopharmaceutical drug development company focused on advancing novel therapeutics targeting degenerative retinal diseases that have significant unmet medical need, 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. Belite's lead candidate, Tinlarebant, an oral therapy intended to reduce the accumulation of 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 patients and a Phase 3 study (PHOENIX) in patients with GA. For more information, follow us on X, 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 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. 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.

BELITE BIO, INC
UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(Amounts in thousands of US Dollars, except share and per share amounts)

	For the Three Months Ended March 31,	
	2024	**2025**
Expenses		
Research and development	6,765	9,396
General and administrative	1,563	6,121
Total operating expenses	8,328	15,517
Loss from operations	(8,328)	(15,517)
Other income:		
Total other income, net	463	1,240
Loss before income tax	(7,865)	(14,277)
Income tax expense	6	-
Net loss	(7,871)	(14,277)
Other comprehensive income (loss)		
Foreign currency translation adjustments, net of nil tax	(96)	18
Total comprehensive loss	(7,967)	(14,259)
Weighted average number of ordinary shares used in per share calculation:		
- Basic and Diluted	29,677,173	32,084,106
Net loss per ordinary share		
- Basic and Diluted	$ (0.27)	$ (0.45)

BELITE BIO, INC
UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS
(Amounts in thousands of US Dollars, except share amounts)

	December 31, 2024	**March 31, 2025**
Current assets	$ 147,073	$ 159,287
Other assets	5,059	4,914
TOTAL ASSETS	$ 152,132	$ 164,201
TOTAL LIABILITIES	$ 6,311	$ 6,131
TOTAL SHAREHOLDERS' EQUITY	145,821	158,070
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 152,132	$ 164,201
Ordinary shares authorized	400,000,000	400,000,000
Ordinary shares issued	31,857,802	32,595,001
Ordinary shares outstanding	31,826,549	32,544,784

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