LeonaBio Reports First Quarter 2026 Financial Results and Provides Business Update

May 7, 2026

Advancing Phase 3 Lasofoxifene Development Program of Novel Selective Estrogen Receptor Modulator, a Potential Multi-Billion Dollar Opportunity as Treatment Option for Patients with ESR1-Mutations

Expects to Complete Enrollment of Phase 3 Clinical Trial of Lasofoxifene in Treatment-Resistant ER-positive, HER2-negative, ESR1-mutated Metastatic Breast Cancer in 4Q 2026 with Topline Data Anticipated in 2H 2027

On-track to Initiate Phase 2 Proof-of-Concept Study of ATH-1105 in ALS patients in 2H 2026

Recent Events Include Three Appointments to Board of Directors and Virtual Key Opinion Leader Event Highlighting Potential of Lasofoxifene

BOTHELL, Wash., May 07, 2026 (GLOBE NEWSWIRE) -- **LeonaBio, Inc.**(NASDAQ: LONA), a clinical-stage biopharmaceutical company dedicated to the development of novel therapeutics for diseases with high unmet medical needs, today reported financial results for the quarter ended March 31, 2026, and provided recent pipeline and business updates.

“We entered 2026 with a clear focus on disciplined execution across our portfolio, led by the continued advancement of lasofoxifene in the Phase 3 ELAINE-3 clinical trial for patients with ESR1-mutated metastatic breast cancer, a population with significant unmet need,” said Mark Litton, Ph.D., President and Chief Executive Officer of LeonaBio. “As the treatment landscape continues to evolve, we remain confident in the ELAINE-3 program, supported by a body of nonclinical and clinical evidence that we believe reinforces lasofoxifene’s differentiated profile and its potential to meaningfully address endocrine resistance, including independent mechanistic data from animal models on its bone protective potential, which further strengthens our conviction in the scientific and clinical rationale underlying this pivotal study.”

“In parallel, we are continuing to advance ATH-1105 toward a Phase 2 proof-of-concept trial in amyotrophic lateral sclerosis (ALS), building on encouraging Phase 1 data demonstrating a favorable safety profile, pharmacokinetics, and central nervous system (CNS) penetration,” continued Dr. Litton. “With a strengthened balance sheet following our December 2025 $90 million common stock and warrant financing together with the potential additional $146 million upon exercise of the cash-exercisable warrants, we believe we are well positioned to execute on multiple value-creating milestones in 2026 and beyond as we work to bring transformative therapies to patients in urgent need of new medicines.”

Clinical Development & Pipeline Programs

Lasofoxifene – A novel, nonsteroidal selective estrogen receptor modulator (SERM) with a unique binding profile, designed to act as a potent antagonist against breast wild-type and mutant estrogen receptors, including the clinically significant ESR1 mutations commonly associated with resistance to endocrine therapy in metastatic breast cancer, while preserving estrogen signaling in nontarget tissues such as bone.

In December 2025, LeonaBio acquired an exclusive global license (excluding Asia and certain countries in the Middle East) from Sermonix Pharmaceuticals, Inc. (Sermonix) for rights to develop and commercialize lasofoxifene.

- Lasofoxifene is being advanced in a Phase 3 clinical trial (NCT05696626) in combination with abemaciclib, a CDK4/6 inhibitor, as a targeted therapy for estrogen receptor-positive (ER+), HER2-negative, ESR1-mutated metastatic breast cancer, a population with limited treatment options following progression on aromatase inhibitors and CDK4/6 inhibitors. The primary endpoint of the study is a statistically significant improvement in progression free survival (PFS) as determined by blinded, independent central review (BICR). The ongoing Phase 3 trial aims to establish a new standard of care for this genetically defined patient group.
- LeonaBio is amending the ELAINE-3 trial protocol to increase the sample size from 500 participants to up to 600 participants. The primary goal of the amendment is to help ensure that the trial will have the appropriate number of disease progression events.
    - The Company expects to complete enrollment of the Phase 3 ELAINE-3 clinical trial in the fourth quarter of 2026 and to have topline data in the second half of 2027.
- Independent researchers at Virginia Commonwealth University’s Massey Comprehensive Cancer Center presented nonclinical data on lasofoxifene and its potential bone protective role in metastatic breast cancer at the American Association for Cancer Research Annual Meeting 2026 (AACR26), which was consistent with LeonaBio’s data.
    - The results showed lasofoxifene protected against hormone withdrawal-induced bone loss and maintained a robust anti-tumor response in primary and metastatic animal models of ER+ breast cancer. The lasofoxifene studied by the researchers was obtained from an independent source for research use only, not related to LeonaBio’s clinical trials or its investigational product.
- Lasofoxifene was previously evaluated in two Phase 2 studies in patients with ER+, HER2-negative locally advanced or metastatic breast cancer expressing an ESR1 mutation, ELAINE-1 and ELAINE-2
    - ELAINE-1, an open-label, randomized trial comparing lasofoxifene to fulvestrant, showed improved outcomes for lasofoxifene as a potential monotherapy. Although the trial was not powered, results included longer median

progression-free survival (5.6 vs. 3.7 months), higher objective response rates (13.3% vs. 2.9%) and a durable complete response lasting more than 2.5 years. The treatment was well-tolerated with patients reporting quality-of-life benefits.
    - ELAINE-2, an open-label study evaluating lasofoxifene in combination with abemaciclib, demonstrated clinical benefits in heavily pretreated patients, with a median progression-free survival of approximately 13 months, an objective response rate of 56% and a clinical benefit rate of 65.5%. The combination was generally well-tolerated with most adverse events being low grade.

ATH-1105 – A novel, orally available, brain-penetrant, next-generation small molecule drug candidate designed to positively modulate the neurotrophic HGF system for potential treatment of neurodegenerative diseases, including ALS, Alzheimer's disease, and Parkinson's disease. ATH-1105 is currently in clinical development for the potential treatment of ALS.

- In August 2025, LeonaBio presented results from the first-in-human Phase 1 clinical trial (NCT06432647) of ATH-1105 in healthy volunteers at the ALS Nexus 2025 conference.
    - Results from the Phase 1 trial demonstrated a favorable safety and tolerability profile as well as dose-proportional pharmacokinetics and CNS penetration.
- Previously, the Company presented data from the Phase 1 clinical trial of ATH-1105 at the 4th Annual ALS Drug Development Summit. Key highlights from the presentation include:
    - ATH-1105 showed a favorable safety profile and was well tolerated in both single and multiple ascending dose studies in healthy volunteers.
    - ATH-1105 showed dose proportional pharmacokinetics and CNS penetration.
    - ATH-1105 demonstrated consistent and robust beneficial effects in preclinical models of ALS.
- LeonaBio conducted the first-in-human Phase 1 double-blind, placebo-controlled clinical trial that enrolled 80 healthy volunteers to evaluate single and multiple oral ascending doses of ATH-1105. The study was completed in November 2024 and evaluated the safety and tolerability of ATH-1105 and included measurements of pharmacokinetic outcomes.
- ATH-1105's potential is supported by a growing body of preclinical evidence demonstrating statistically significant improvements in nerve and motor function, biomarkers of inflammation and neurodegeneration, and survival in various models of ALS.
- LeonaBio is on track to dose ALS patients in a Phase 2 proof-of-concept clinical trial in the second half of 2026.

Corporate Updates

LeonaBio announced the appointment of Fred Callori, J.D., Natalie Holles, and Peter B. Silverman, J.D. to its Board of Directors, effective as of May 5, 2026. The company also announced that John Fluke, Jr., who has served on the Board since 2014, retired effective May 4, 2026.

- Fred Callori, J.D., has served as a Partner and Managing Director at Perceptive Advisors LLC, an investment firm that specializes in investing in biotechnology stocks, since January 2018.
- Natalie Holles has served as the Chief Executive Officer and member of the Board of Directors of Aura Biosciences, a clinical-stage biotechnology company, since April 2026. Ms. Holles served as the Chief Executive Officer of Third Harmonic Bio, a biopharmaceutical company, from August 2021 to December 2025.
- Peter B. Silverman, J.D., served as Chief Operating Officer of Merus N.V. (formerly, Nasdaq:MRUS), a biotechnology company, from January 2023 until its acquisition by Genmab A/S in December 2025, and prior to that, Mr. Silverman held several leadership roles at Merus. Mr. Silverman has served as a member of the board of directors of Kinaset Therapeutic, a biopharmaceutical company, since January 2026.

Recent Events

LeonaBio hosted a virtual Key Opinion Leader event with two leading physician experts in the breast cancer field to discuss the current and evolving treatment landscape in metastatic breast cancer and the potential for lasofoxifene to transform the standard of care for patients with treatment-resistant estrogen receptor-positive (ER+), HER2-negative, ESR1-mutated metastatic breast cancer.

The event titled, *"Modulation and Combination: the Potential for Lasofoxifene to Transform the Standard-of-Care in Metastatic Breast Cancer,"* featured a discussion with **David Portman, M.D**., Chief Executive Officer of Sermonix Pharmaceuticals and an oncology consultant to LeonaBio, along with two physician experts in the breast cancer field:

- Matthew P. Goetz, M.D. – Erivan K. Haub Family Professor of Cancer Research Honoring Richard F. Emslander, M.D, Mayo Clinic, Principal investigator and director, Breast Cancer Specialized Program of Research Excellence (SPORE), Mayo Clinic Comprehensive Cancer Center and Enterprise Deputy Director, Translational Research, Mayo Clinic Comprehensive Cancer Center.
- Seth Wander, M.D., Ph.D. – Director of Precision Medicine, Termeer Center for Targeted Therapies, Director of Translational Research, Breast Oncology Program, Mass General Brigham Cancer Institute, Assistant Professor of Medicine, Harvard Medical School.

A replay of the event is available on the LeonaBio website under Events in the Investor Relations here.

Financial Results

- **Cash Position.** Cash, cash equivalents and investments were $67.7 million as of March 31, 2026, compared to $88.3 million as of December 31, 2025. Net cash used in operations was $20.9 million for the quarter ended March 31, 2026, compared to $14.7 million for the quarter ended March 31, 2025. In conjunction with the December 2025 license agreement with Sermonix, LeonaBio announced a $90 million private placement financing of common stock and warrants, with the warrants providing, if exercised, up to an additional $146 million to support development through key clinical and regulatory milestones.
- **Research and Development (R&D) Expenses.** R&D expenses were $10.3 million for the quarter ended March 31, 2026, compared to $4.3 million for the quarter ended March 31, 2025. The increase was driven primarily by clinical trial spend related to the ELAINE-3 trial for lasofoxifene.
- **General and Administrative (G&A) Expenses.** G&A expenses were $6.9 million for the quarter ended March 31, 2026, compared to $5.2 million for the quarter ended March 31, 2025. The increase was driven primarily by professional service fees.
- **Net Loss.** Net loss was $32.9 million, or $1.73 per share, for the quarter ended March 31, 2026, compared to a net loss of $9.1 million, or $2.34 per share, for the quarter ended March 31, 2025.

About LeonaBio

LeonaBio, headquartered in the Seattle, Washington area, is a clinical-stage biopharmaceutical company dedicated to the development of novel therapeutics for diseases with high unmet medical needs, including treatment-resistant metastatic breast cancer and amyotrophic lateral sclerosis (ALS), with the goal of improving patients' lives. Our lead drug candidates, lasofoxifene and ATH-1105, are novel, small molecule therapies with the potential to address devastating diseases where current treatment options are limited or ineffective. With a strong commitment to scientific excellence and patient-centered innovation, we are dedicated to developing meaningful new therapies for those who need them most.
For more information, visit www.leonabio.com.

Forward-Looking Statements
This communication contains "forward-looking statements" within the meaning of Section 27A of the Securities Act, Section 21E of the Securities Exchange Act of 1934 and the Private Securities Litigation Reform Act of 1995. These forward-looking statements are not based on historical fact and include statements regarding: the beneficial characteristics, safety and efficacy of LeonaBio's drug candidates; the potential of any subsequent clinical trials to show the beneficial characteristics, safety and efficacy of ATH-1105; the potential of LeonaBio to complete the Phase 3 ELAINE-3 clinical trial for lasofoxifene and to meet the trial endpoints, and any subsequent clinical trials to show the clinical benefits of lasofoxifene; LeonaBio's drug candidates as potential treatments for metastatic breast cancer, amyotrophic lateral sclerosis and other diseases; LeonaBio's future development plans and the timing thereof; the potential learnings from preclinical studies and other nonclinical data and their ability to inform and improve future clinical development plans; the markets for LeonaBio's drug candidates, if approved for commercial use, and LeonaBio's ability to serve those markets; anticipated development milestone timelines, such as the initiation of clinical trials and the timing of data releases, and LeonaBio's ability to meet such timelines; LeonaBio's ability to obtain and maintain regulatory approval of its drug candidates in the United States and other jurisdictions and the timing thereof, and any related restrictions, limitations or warnings in the label of any approved drug candidate; LeonaBio's ability to commercialize any approved products; and the sufficiency of LeonaBio's capital resources to advance both its oncology and neurodegeneration programs. Forward-looking statements generally include statements that are predictive in nature and depend upon or refer to future events or conditions, and include words such as "may," "will," "should," "on track," "would," "expect," "plan," "believe," "intend," "pursue," "continue," "suggest," "potential," "target" and similar expressions. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with the possible failure to realize certain anticipated benefits of the license relating to lasofoxifene and the recent private placement financing, including with respect to future financial and operating results; the data from preclinical and clinical trials may not support the safety, efficacy and tolerability of LeonaBio's drug candidates; development of drug candidates may cease or be delayed; regulatory authorities could object to protocols, amendments and other submissions; future potential regulatory milestones for drug candidates, including those related to current and planned clinical studies, may be insufficient to support regulatory submissions or approval; whether LeonaBio's trials are sufficiently powered to meet the planned endpoints; LeonaBio may not be able to recruit sufficient patients for its clinical trials; the outcome of legal proceedings that may in the future be instituted against LeonaBio, its directors and officers; possible negative interactions of LeonaBio's drug candidates with other treatments; FDA regulatory delays and uncertainty and new policies, including executive orders, changes in the leadership of federal agencies such as the FDA and SEC, staff layoffs, budget cuts to agency programs and research and changes in drug pricing controls; LeonaBio's assumptions regarding its financial condition and the sufficiency of its cash, cash equivalents and investments to fund its planned operations may be incorrect; adverse conditions in the general domestic and global economic markets, including as a result of tariffs; the impact of competition; the impact of drug candidate development and clinical activities on operating expenses; the impact of new or changing laws and regulations; as well as the other risks detailed in LeonaBio's filings with the SEC from time to time. These forward-looking statements speak only as of the date hereof and LeonaBio undertakes no obligation to update forward-looking statements. LeonaBio may not actually achieve the plans, intentions or expectations disclosed in its forward-looking statements, and you should not place undue reliance on the forward-looking statements.

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LeonaBio, Inc.
Condensed Consolidated Balance Sheets
(Amounts in thousands)

	March 31, 2026	December 31, 2025
	(unaudited)	
Assets		
Cash and cash equivalents	$ 32,833	$ 69,276
Short-term investments	34,846	19,055
Other short-term assets	1,523	1,127
Other long-term assets	2,388	2,693
Total assets	$ 71,590	$ 92,151
Liabilities and stockholders' equity		
Current liabilities	$ 6,666	$ 10,015
Sermonix pre-funded warrant	—	37,488
Milestone liability	14,187	15,116
Long-term liabilities	1,434	1,742
Total liabilities	22,287	64,361
Stockholders' equity	49,303	27,790
Total liabilities and stockholders' equity	$ 71,590	$ 92,151

LeonaBio, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(Amounts in thousands, except share and per share amounts)
(Unaudited)

	Three Months Ended March 31,	
	2026	2025
Operating expenses:		
Research and development	$ 11,262	$ 4,302
Milestone liability change in fair value	(929)	—
General and administrative	6,881	5,234
Total operating expenses	17,214	9,536
Loss from operations	(17,214)	(9,536)
Other income, net	586	393
Sermonix pre-funded warrant change in fair value	(16,320)	—
Net loss	$ (32,948)	$ (9,143)
Unrealized (loss) gain on available-for-sale securities	(9)	(5)
Comprehensive loss attributable to common stockholders	$ (32,957)	$ (9,148)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.73)	$ (2.34)
Weighted-average shares used in computing net loss per share attributable to common stockholders, basic and diluted	19,027,087	3,904,244