Beam Therapeutics Reports First Quarter 2025 Financial Results and Recent Business Highlights

May 6, 2025

First Patient Dosed in the Phase 1/2 Study of BEAM-301 in Glycogen Storage Disease Type Ia, Beam's Second Clinical Stage In Vivo *Editing Program*

Updated Data from BEACON Phase 1/2 Clinical Trial of BEAM-101 Accepted for Presentation at the European Hematology Association 2025 Congress in June

Following Positive Initial Data for BEAM-302 in Alpha-1 Antitrypsin Deficiency, Company Initiated Dosing of Fourth Cohort in Part A of Phase 1/2 Trial and Received Clearance of U.S. IND; Updated Data Expected to be Presented in Second Half of 2025

Ended First Quarter 2025 with $1.2 Billion in Cash, Cash Equivalents and Marketable Securities, Including Net Proceeds from $500 Million Financing; Cash Runway Expected to Support Operating Plans into 2028

CAMBRIDGE, Mass., May 06, 2025 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported first quarter 2025 financial results and provided an update on corporate and pipeline progress across the company's hematology and genetic disease franchises.

"Beam has had a tremendous start to what we anticipate will be a transformative year. In March, we achieved a historic milestone with BEAM-302, delivering the first-ever clinical genetic correction of a disease-causing mutation for alpha-1 antitrypsin deficiency. Building on this momentum, we have swiftly advanced the program—initiating the fourth cohort of Part A of the Phase 1/2 BEAM-302 study and securing U.S. FDA clearance for our investigational new drug application, positioning us for continued rapid progress," said John Evans, chief executive officer of Beam. "Additionally, we recently dosed the first patient in our second *in vivo* program, BEAM-301, a potential treatment for glycogen storage disease type Ia. In our hematology franchise, we are preparing to share updated clinical data from the BEACON Phase 1/2 trial of BEAM-101 in sickle cell disease at EHA in June and remain on track to complete dosing for 30 patients by mid-year. This significant clinical progress is supported by our strong financial foundation, further reinforced by our recent $500 million financing, which extends our projected cash runway into 2028."

First Quarter 2025 and Recent Progress

- Recently, the first patient was dosed in the U.S.-based Phase 1/2 clinical trial evaluating BEAM-301 as a potential treatment for patients with glycogen storage disease type Ia (GSDIa), an autosomal recessive disorder that disrupts glucose homeostasis. BEAM-301 is an investigational *in vivo* base editing treatment designed to correct the R83C mutation, the most common disease-causing mutation that results in the most severe form of GSDIa.
- Clinical data from the BEACON Phase 1/2 clinical trial of BEAM-101, an investigational genetically modified cell therapy for the treatment of sickle cell disease (SCD), have been accepted for presentation at the European Hematology Association (EHA) 2025 Congress, taking place in Milan from June 12-15, 2025.
- In March, the United States (U.S.) Food and Drug Administration (FDA) cleared the investigational new drug (IND) application for BEAM-302 for the treatment of alpha-1 antitrypsin deficiency (AATD), enabling the company to begin activating sites in the U.S. for its ongoing Phase 1/2 trial.
- Also in March, the company announced positive initial safety and efficacy data from the Phase 1/2 trial of BEAM-302, establishing clinical proof of concept for a potential treatment addressing AATD and for *in vivo* base editing. Preliminary results from the first three single-ascending dose cohorts in Part A of the trial demonstrated that BEAM-302 was well tolerated, with single doses of BEAM-302 leading to durable, dose-dependent correction of the disease-causing mutation. In April, an encore of the data was presented at the 2025 Alpha-1 Foundation 7th Global Research Conference and 10th Patient Congress, alongside updated biomarker data from the 60 mg cohort that provided further evidence of BEAM-302's clinical profile. Since reporting the data, Beam has initiated dosing in the fourth cohort of Part A, evaluating 75 mg of BEAM-302.
- In conjunction with the BEAM-302 initial data, Beam completed a $500 million oversubscribed, registered direct financing, enabling the company to fund its anticipated operating expenses and capital expenditure requirements into 2028.

Key Anticipated Milestones

Liver-targeted Genetic Disease Franchise

- Beam plans to continue the dose-escalation portion of Part A of the ongoing BEAM-302 Phase 1/2 clinical trial and expects to report further data at a medical conference in the second half of 2025.
- The company plans to dose the first patient in Part B of the ongoing BEAM-302 Phase 1/2 clinical trial, which will include AATD patients with mild to moderate liver disease, in the second half of 2025.

- The company plans to continue dosing in the Phase 1/2 clinical trial of BEAM-301 in GSDIa.

Hematology Franchise

- Beam expects to dose 30 patients in the BEACON Phase 1/2 clinical trial of BEAM-101 in adults with severe SCD by mid-2025.
- The company also plans to present updated data from the BEACON Phase 1/2 trial during EHA 2025 in June.
- The company expects to initiate a Phase 1 healthy volunteer clinical trial of BEAM-103, the ESCAPE monoclonal antibody, by the end of 2025.

First Quarter 2025 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.2 billion as of March 31, 2025, compared to $850.7 million as of December 31, 2024.
- **Research & Development (R&D) Expenses:** R&D expenses were $98.8 million for the first quarter of 2025, compared to $84.8 million for the first quarter of 2024.
- **General & Administrative (G&A) Expenses**: G&A expenses were $27.9 million for the first quarter of 2025, compared to $26.7 million for the first quarter of 2024.
- **Net Income (Loss):** Net loss was $109.3 million, or $1.24 per share, for the first quarter of 2025, compared to $98.7 million, or $1.21 per share, for the first quarter of 2024.

Cash Runway
Beam expects that its cash, cash equivalents and marketable securities as of March 31, 2025, including net proceeds from the recent $500 million financing, will enable the company to fund its anticipated operating expenses and capital expenditure requirements into 2028. This expectation includes funding directed toward reaching each of the key anticipated milestones for BEAM-101, ESCAPE, BEAM-301 and BEAM-302 described above.

About Beam Therapeutics
Beam Therapeutics (Nasdaq: BEAM) is a biotechnology company committed to establishing the leading, fully integrated platform for precision genetic medicines. To achieve this vision, Beam has assembled a platform with integrated gene editing, delivery and internal manufacturing capabilities. Beam's suite of gene editing technologies is anchored by base editing, a proprietary technology that is designed to enable precise, predictable and efficient single base changes, at targeted genomic sequences, without making double-stranded breaks in the DNA. This has the potential to enable a wide range of therapeutic editing strategies that Beam is using to advance a diversified portfolio of base editing programs. Beam is a values-driven organization committed to its people, cutting-edge science, and a vision of providing life-long cures to patients suffering from serious diseases.

Cautionary Note Regarding Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Investors are cautioned not to place undue reliance on these forward-looking statements, including, but not limited to, statements related to: the therapeutic applications and potential of our technology, including with respect to SCD, AATD, GSDIa, and ESCAPE; our plans, and anticipated timing, to advance our programs, including the clinical trial designs and expectations for BEAM-101, BEAM-103, BEAM-301 and BEAM-302; the sufficiency of our capital resources to fund operating expenses and capital expenditure requirements and the period in which such resources are expected to be available; our plans to present data at upcoming medical conferences; and our ability to develop life-long, curative, precision genetic medicines for patients through base editing. Each forward-looking statement is subject to important risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement, including, without limitation, risks and uncertainties related to: our ability to develop, obtain regulatory approval for, and commercialize our product candidates, which may take longer or cost more than planned; our ability to raise additional funding, which may not be available; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; the uncertainty that our product candidates will receive regulatory approval necessary to advance human clinical trials; that preclinical testing of our product candidates and preliminary or interim data from preclinical studies and clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that initiation and enrollment of, and anticipated timing to advance, our clinical trials may take longer than expected; that our product candidates or the delivery modalities we rely on to administer them may cause serious adverse events; that our product candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; and the other risks and uncertainties identified under the headings "Risk Factors Summary" and "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2024, and in any subsequent filings with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release. Factors or events that could cause our actual results to differ may emerge from time to time, and it is not possible for us to predict all of them. We undertake no obligation to update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable law.

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Condensed Consolidated Balance Sheet Data (unaudited)
(in thousands)

	March 31, 2025	December 31, 2024
Cash, cash equivalents, and marketable securities	$ 1,219,952	$ 850,740
Total assets	1,466,920	1,103,824
Total liabilities	343,784	370,279
Total stockholders' equity	1,123,136	733,545

Condensed Consolidated Statement of Operations (unaudited)
(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2025	2024
License and collaboration revenue	$ 7,470	$ 7,410
Operating expenses:		
Research and development	98,816	84,818
General and administrative	27,940	26,724
Total operating expenses	126,756	111,542
Loss from operations	(119,286)	(104,132)
Other income (expense):		
Change in fair value of derivative liabilities	2,260	(2,900)
Change in fair value of non-controlling equity investments	(2,081)	(3,353)
Change in fair value of contingent consideration liabilities	(27)	(133)
Interest and other income (expense), net	9,864	11,849
Total other income (expense)	10,016	5,463
Net Loss	$ (109,270)	$ (98,669)
Unrealized gain (loss) on marketable securities	(519)	(1,525)
Comprehensive loss	$ (109,789)	$ (100,194)
Net loss per common share, basic and diluted	$ (1.24)	$ (1.21)
Weighted-average common shares outstanding, basic and diluted	87,975,311	81,698,633