



Beam Therapeutics Reports First Quarter 2026 Financial Results and Recent Business Updates

May 7, 2026

Recent BEAM-302 Topline Data in Alpha-1 Antitrypsin Deficiency (AATD) Demonstrate Strong Single-dose Safety and Efficacy Profile, with 60 mg Selected as Optimal Biological Dose; Global Pivotal Cohort Expected to Initiate in Second Half of 2026

Data from Phase 1/2 BEACON Clinical Trial of Risto-cel in Sickle Cell Disease Published in April 1 Issue of the New England Journal of Medicine; U.S. Biologics License Application (BLA) Submission Expected as Early as Year-End 2026

Investigational New Drug (IND) Application for BEAM-304 in PKU and Data from BEAM-301 in GSDIa Anticipated in 2026

Ended First Quarter 2026 with \$1.2 Billion in Cash, Cash Equivalents and Marketable Securities; Cash Runway Expected to Support Operating Plans into mid-2029

CAMBRIDGE, Mass., May 07, 2026 (GLOBE NEWSWIRE) -- [Beam Therapeutics Inc.](#) (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported first quarter 2026 financial results and provided updates across the company's hematology and genetic disease franchises.

"The first quarter of 2026 was a defining period for Beam, marked by meaningful clinical advances across our portfolio and key steps toward becoming a commercial-stage company. The updated topline data from BEAM-302 – including robust increases in total AAT and a well-tolerated safety profile – give us high confidence in the 60 mg optimal biological dose and a clear path to initiating the pivotal cohort in the second half of this year," said John Evans, chief executive officer of Beam Therapeutics. "Publication of the BEACON trial data in the *New England Journal of Medicine* underscores the differentiated profile of risto-cel, and we remain on track to submit our BLA as early as year-end 2026, a milestone toward bringing a potentially transformative treatment to patients with sickle cell disease. With BEAM-304 in PKU, we are extending the reach of our clinically validated base editing platform to directly correct disease-causing mutations in a new indication, further demonstrating the breadth of what precision genetic medicine can achieve. With a strong cash position extending our runway into mid-2029, we have the financial foundation to execute across all of these priorities and deliver on our mission to bring precision genetic medicines to patients who need them most."

First Quarter 2026 and Recent Progress and Anticipated Milestones

Corporate

- Beam Therapeutics has been selected for the [TIME100 Most Influential Companies](#) list by TIME Magazine, highlighting 100 companies making an extraordinary impact around the world. In February, John Evans, chief executive officer of Beam, was recognized as part of the [TIME100 Health list](#), TIME's list honoring leaders who are advancing care, shaping policy and driving innovations that transform lives.

Liver-targeted Genetic Disease Franchise

BEAM-302: Beam's lead genetic disease program is designed to be a best-in-class and first-in-class liver-targeting therapy for alpha-1 antitrypsin deficiency (AATD) that addresses the underlying pathophysiology of both liver and lung disease.

- In March, Beam [announced](#) updated safety and efficacy data from the ongoing Phase 1/2 trial of BEAM-302. Treatment with BEAM-302 led to rapid and durable increases of total and functional AAT, decreases in mutant Z-AAT, and new production of corrected M-AAT, with a well-tolerated safety profile across single doses up to 75 mg.
- Amy Simon, M.D., chief medical officer of Beam, will give a [presentation](#) on translating scientific discovery in gene editing to clinical progress for patients with lung disease, featuring the recent clinical data from the BEAM-302 trial, at the upcoming American Thoracic Society (ATS) International Conference 2026, being held in Orlando, Florida, May 15-20. The presentation will be included in the scientific symposium titled "Rewriting the Code: Precision Delivery Vectors and Gene Therapy for Pulmonary Vascular Diseases" at 3:30 p.m. ET on Monday, May 18, 2026.
- Based on feedback from the U.S. Food and Drug Administration (FDA), Beam intends to pursue an accelerated approval pathway for BEAM-302. To support a future biologics licensing application (BLA) submission, the company anticipates enrolling approximately 50 additional patients with AATD-associated lung disease, with or without liver disease, in an expansion of the ongoing open-label Phase 1/2 trial. Beam expects to initiate this pivotal cohort in the second half of 2026.
- In addition, Beam expects to present detailed and updated BEAM-302 data at a medical congress in 2026.

BEAM-304: BEAM-304 leverages Beam's proprietary and clinically validated base editing technology and lipid nanoparticle (LNP) delivery capabilities to directly and durably correct mutations in the phenylalanine hydroxylase (PAH) gene that cause phenylketonuria (PKU).

- A planned Phase 1/2 trial will initially evaluate safety, tolerability, and reduction of blood Phe levels in PKU patients with the R408W mutation, followed thereafter by a base editor for a second mutation, with a goal of establishing clinical proof of

concept for base editing in PKU.

- Beam expects to file an investigational new drug (IND) application with the FDA for BEAM-304 in 2026 following completion of pre-IND activities.

BEAM-301: BEAM-301 aims to correct the most common disease-causing mutation, R83C, in patients with glycogen storage disease type Ia (GSDIa).

- BEAM-301 is currently being evaluated in an open-label Phase 1/2 dose-exploration trial in patients with GSDIa.
- Beam expects to report initial clinical data in 2026.

Hematology Franchise

Risto-cel: Ristoglogene autogetemcel (risto-cel, formerly known as BEAM-101) is an investigational autologous cell therapy with a potential best-in-class profile for the treatment of sickle cell disease (SCD).

- Data from the ongoing Phase 1/2 BEACON clinical trial evaluating risto-cel for the treatment of SCD with severe vaso-occlusive crises (VOCs) were [published](#) in [the New England Journal of Medicine](#). Data demonstrate risto-cel's differentiated profile, including deep resolution of SCD markers, rapid engraftment, reduced hospitalization, and a predictable manufacturing process that may improve patient experience and treatment center capacity and reduce the length of the transplant process.
- Beam expects to submit a BLA for risto-cel as early as year-end 2026.

Next-generation Programs in Sickle Cell Disease and Hematology:

- The ongoing Phase 1 healthy volunteer clinical trial of BEAM-103, an anti-CD117 monoclonal antibody that enables ESCAPE, is expected to complete dosing in the first half of 2026.

First Quarter 2026 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$1.2 billion as of March 31, 2026, compared to \$1.2 billion as of December 31, 2025.
- **Research & Development (R&D) Expenses:** R&D expenses were \$104.5 million for the first quarter of 2026, compared to \$98.8 million for the first quarter of 2025.
- **General & Administrative (G&A) Expenses:** G&A expenses were \$34.4 million for the first quarter of 2026, compared to \$27.9 million for the first quarter of 2025.
- **Net Income (Loss):** Net loss was \$94.3 million, or \$0.91 per share, for the first quarter of 2026, compared to net losses of \$108.3 million, or \$1.23 per share, for the first quarter of 2025.

Cash Runway

Beam expects that its cash, cash equivalents and marketable securities as of March 31, 2026, which includes \$100 million from the close of the company's financing [agreement](#) with Sixth Street, along with an anticipated additional \$100 million from the Sixth Street facility, will fund anticipated operating expenses and capital expenditure requirements into mid-2029, funding the company through the anticipated launch of risto-cel in SCD, execution of the BEAM-302 pivotal development plan in AATD, and clinical proof of concept for BEAM-304 in PKU.

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 potential 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 lifelong 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, PKU, ESCAPE and GSDIa; our plans, and anticipated timing, to advance our programs and present data from ongoing clinical trials; the clinical trial designs and expectations for risto-cel, BEAM-103, BEAM-301, BEAM-302 and BEAM-304; our planned submission of a BLA for risto-cel; our expected presentations at upcoming medical conferences, including at ATS 2026; our anticipated regulatory interactions and filings; 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; and our ability to develop lifelong, 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 initiate or continue 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, including 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, 2025, 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)

	<u>March 31,</u> <u>2026</u>	<u>December 31,</u> <u>2025</u>
Cash, cash equivalents, and marketable securities	\$ 1,211,652	\$ 1,245,210
Total assets	1,480,798	1,481,177
Total liabilities	316,360	242,819
Total stockholders' equity	1,164,438	1,238,358

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

	<u>Three Months Ended March 31,</u>	
	<u>2026</u>	<u>2025</u>
License and collaboration revenue	\$ 31,738	\$ 7,470
Operating expenses:		
Research and development	104,524	98,816
General and administrative	34,429	27,940
Total operating expenses	<u>138,953</u>	<u>126,756</u>
Loss from operations	(107,215)	(119,286)
Other income (expense):		
Change in fair value of derivative liabilities	2,500	3,200
Change in fair value of non-controlling equity investments	16	(2,081)
Change in fair value of contingent consideration liabilities	514	(27)
Interest and other income (expense), net	<u>9,867</u>	<u>9,864</u>
Total other income (expense)	<u>12,897</u>	<u>10,956</u>
Net loss	<u>\$ (94,318)</u>	<u>\$ (108,330)</u>
Unrealized gain (loss) on marketable securities	(2,181)	(519)
Comprehensive loss	<u>\$ (96,499)</u>	<u>\$ (108,849)</u>
Net loss per common share, basic and diluted	<u>\$ (0.91)</u>	<u>\$ (1.23)</u>
Weighted-average common shares outstanding, basic and diluted	<u>103,262,001</u>	<u>87,975,311</u>