Beam Therapeutics Reports Second Quarter 2026 Financial Results and Announces First Patient Dosed in Global Pivotal Cohort of BEAM-302 Trial in Alpha-1 Antitrypsin Deficiency (AATD)

August 4, 2026

Updated BEAM-302 Phase 1/2 Clinical Data Selected for Late-Breaking Oral Presentation at the European Respiratory Society (ERS) Congress 2026

Dosing Complete for All Adult and Adolescent Patients in Phase 1/2 BEACON Trial of Risto-cel in Sickle Cell Disease; U.S. Biologics License Application (BLA) Submission Expected as Early as Year-End 2026

Clinical Trial Start-up Activities Underway Following U.S. FDA Clearance of Investigational New Drug (IND) Application for BEAM-304 in Phenylketonuria (PKU)

Ended Second 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., Aug. 04, 2026 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported second quarter 2026 financial results and provided updates across the company's hematology and genetic disease franchises.

"The second quarter marked another period of rapid progress and disciplined execution on the clinical, regulatory and operational milestones we set for Beam," said John Evans, chief executive officer of Beam Therapeutics. "Importantly, this progress enabled dosing of the first patient in the global pivotal cohort evaluating BEAM-302, the most advanced genetic medicine in development for AATD, which has the potential to fundamentally change the treatment paradigm for patients. In addition, we completed dosing for all adult and adolescent SCD patients in the BEACON trial of risto-cel and received FDA clearance of the IND for BEAM-304 in PKU, paving the way to the clinic for this next potentially high-value franchise. Looking ahead, we believe Beam is positioned to achieve several important milestones, including updated clinical data for BEAM-302 at the ERS Congress, rapid enrollment in the BEAM-302 pivotal cohort, first-in-human data for BEAM-301 in GSDIa, and the expected BLA submission for risto-cel. With the continued expansion and advancement of our base editing pipeline, we have the potential to deliver transformative therapies for many patients with serious genetic diseases."

Second Quarter 2026 and Recent Progress and Anticipated Milestones

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 directly corrects the root cause of the disease and therefore has the potential to address both liver and lung manifestations of AATD.

- In July, Beam dosed the first patient in the global pivotal cohort of the ongoing Phase 1/2 trial evaluating BEAM-302 in patients with AATD-associated lung disease, with or without liver disease. The cohort is designed to support a potential accelerated approval path in the United States.
- Detailed and updated clinical data for BEAM-302 were selected for a late-breaking oral presentation at the European Respiratory Society (ERS) Congress, taking place September 5-9, 2026, in Barcelona, Spain.

BEAM-304: BEAM-304 is designed to leverage 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). Beam aims to advance BEAM-304 using an innovative platform approach with the goal of creating mutation-specific base editors for the majority of patients with PKU.

- In June, Beam announced that the U.S. Food and Drug Administration (FDA) cleared the investigational new drug (IND) application for BEAM-304.
- Beam has initiated clinical start-up activities for the planned Phase 1/2 trial for BEAM-304. The trial will initially evaluate safety, tolerability, and reduction of blood Phe levels in PKU patients with the R408W mutation, one of the most prevalent disease-causing mutations among patients with PKU in the U.S., with a goal of establishing clinical proof of concept for base editing in PKU. The trial will subsequently evaluate base editors for additional mutations within a single clinical program.
- In July, updated preclinical data for BEAM-304 were presented at the Federation of American Societies for Experimental Biology (FASEB) Genome Engineering: Research and Applications Conference. The presentation is available on the Presentation and Publications page of Beam's website beamtx.com.

BEAM-301: BEAM-301 aims to correct one of the most common disease-causing mutations, 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).

- Dosing is complete in all adult and adolescent patients enrolled in the Phase 1/2 BEACON trial.
- Beam expects to report updated data for the BEACON trial by year-end 2026 and submit a biologics license application (BLA) for risto-cel as early as year-end 2026.

Next-generation Programs in Sickle Cell Disease and Hematology:

- Beam completed enrollment and dosing in the Phase 1 healthy volunteer clinical trial of BEAM-103, an anti-CD117 monoclonal antibody with the potential to enable non-genotoxic *ex vivo* and *in vivo* therapies for SCD. Treatment with BEAM-103 was well tolerated across all doses tested.
- Beam continues to make significant investments in developing targeted LNPs to deliver gene editing to hematopoietic stem cells (HSCs). Targeted LNPs for HSC delivery have been identified and are in lead optimization.

Second Quarter 2026 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.2 billion as of June 30, 2026, compared to $1.2 billion as of December 31, 2025.
- **Research & Development (R&D) Expenses:** R&D expenses were $95.1 million for the second quarter of 2026, compared to $101.8 million for the second quarter of 2025.
- **General & Administrative (G&A) Expenses**: G&A expenses were $31.9 million for the second quarter of 2026, compared to $26.9 million for the second quarter of 2025.
- **Net Income (Loss):** Net loss was $122.7 million, or $1.18 per share, for the second quarter of 2026, compared to net loss of $102.1 million, or $1.00 per share, for the second quarter of 2025.

Cash Runway
Beam expects that its cash, cash equivalents and marketable securities as of June 30, 2026, together with an additional $200 million expected to be drawn from the company's facility with Sixth Street, 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 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 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 expectations regarding the anticipated launch of risto-cel; the potential for an accelerated approval path for BEAM-302; our expected presentations at upcoming medical conferences, including at the ERS Congress; our anticipated regulatory interactions and filings; our expectations regarding the funds that will be available to draw under our credit facility; 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 we may not achieve the funding milestones under our credit facility; 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.

Contacts:
Investors:
Holly Manning
Beam Therapeutics
hmanning@beamtx.com

Media:
Josie Butler
1AB
josie@1abmedia.com

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

	June 30, 2026	December 31, 2025
Cash, cash equivalents, and marketable securities	$ 1,152,927	$ 1,245,210
Total assets	1,386,355	1,481,177
Total liabilities	318,913	242,819
Total stockholders' equity	1,067,442	1,238,358

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
License and collaboration revenue	$ 490	$ 8,466	$ 32,228	$ 15,936
Operating expenses:				
Research and development	95,096	101,758	199,620	200,574
General and administrative	31,947	26,859	66,376	54,799
Total operating expenses	127,043	128,617	265,996	255,373
Loss from operations	(126,553)	(120,151)	(233,768)	(239,437)
Other income (expense):				
Change in fair value of derivative liabilities	(4,200)	1,300	(1,700)	4,500
Change in fair value of non-controlling equity investments	338	4,415	354	2,334
Change in fair value of contingent consideration liabilities	(205)	(28)	309	(55)
Gain on sale of equity method investment	455	—	455	—
Interest and other income (expense), net	7,487	12,326	17,354	22,190
Total other income (expense)	3,875	18,013	16,772	28,969
Net loss	$ (122,678)	$ (102,138)	$ (216,996)	$ (210,468)
Unrealized gain (loss) on marketable securities	(1,119)	(150)	(3,300)	(669)
Comprehensive loss	$ (123,797)	$ (102,288)	$ (220,296)	$ (211,137)
Net loss per common share, basic and diluted	$ (1.18)	$ (1.00)	$ (2.09)	$ (2.21)
Weighted-average common shares outstanding, basic and diluted	104,325,436	101,995,184	103,797,276	95,023,977