Beam Therapeutics Reports Third Quarter 2026 Financial Results and Highlights Clinical Progress Across Base Editing Portfolio

November 3, 2026

Updated BEAM-302 Phase 1/2 Clinical Data Presented at ERS Demonstrated Durable Increases in Functional AAT and Reductions in Mutant Z-AAT; Completion of Dosing in the Global Pivotal Cohort Expected in First Half of 2027

U.S. Biologics License Application (BLA) Submission for Risto-cel in Sickle Cell Disease Expected as Early as Year-End 2026

Dosing in Phase 1/2 Trial of BEAM-304 in PKU Anticipated to Initiate in Early 2027

Initial BEAM-301 Phase 1/2 Data Presented at ESGCT Demonstrated Clinical Proof of Concept for Genetic Correction of R83C Mutation in GSDIa; Enrollment in 60 mg Cohort Underway

Ended Third Quarter 2026 with $1.0 Billion in Cash, Cash Equivalents and Marketable Securities; Cash Runway Expected to Support Operating Plans into Mid-2029

CAMBRIDGE, Mass., Nov. 03, 2026 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported third quarter 2026 financial results and provided updates across the company's hematology and genetic disease franchises.

"Our progress this quarter reflects the growing maturity and momentum of Beam's platform, with predictable clinical translation across four distinct programs and multiple potentially transformative medicines advancing toward patients, bringing our vision for a new era of genetic medicine increasingly within reach," said John Evans, chief executive officer of Beam Therapeutics. "We are approaching a potential first BLA filing with risto-cel as early as the end of this year, and the pivotal cohort for BEAM-302 is rapidly advancing with completion of dosing expected in the first half of 2027. Recently, initial BEAM-301 clinical trial data showed exciting early evidence of clinical activity and provided further clinical validation of our *in vivo* LNP platform. Together with the planned initiation of BEAM-304 dosing in early 2027, these milestones reinforce our confidence in a platform designed to translate predictably, carry knowledge forward from one program to the next, and progressively expand the number of patients who can benefit from base editing."

Third Quarter 2026 and Recent Progress and Anticipated Milestones

Liver-targeted Genetic Diseases

BEAM-302: BEAM-302, Beam's lead genetic disease program, is an investigational base editing therapy designed to be a differentiated, first-in-class liver-targeting therapy for alpha-1 antitrypsin deficiency (AATD) that directly corrects the root cause of the disease and has the potential to address both liver and lung manifestations.

- In September, Beam presented updated clinical data from the Phase 1/2 trial of BEAM-302 at the European Respiratory Society (ERS) Congress 2026, reinforcing its potential as a one-time treatment for both the lung and liver manifestations of AATD. With up to 18 months of follow-up, the data demonstrated durable increases in total and functional AAT, decreased circulating neutrophil elastase activity, and reductions in mutant Z-AAT and toxic circulating Z-polymers following a single dose. Evidence of AAT production under normal physiological control further supports BEAM-302's potential to restore the body's natural protective response, with a safety profile consistent with lipid nanoparticle (LNP)-based therapies.
- Enrollment and dosing continue in the global pivotal cohort of the ongoing Phase 1/2 trial, which is designed to support a potential accelerated approval path in the United States. The company expects to complete dosing in the pivotal cohort in the first half of 2027.

BEAM-304: BEAM-304 is an investigational base editing program designed to leverage Beam's proprietary base editing technology and 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 a platform approach to develop mutation-specific base editors for the majority of patients with PKU.

- Beam nominated its third base editor within the BEAM-304 program targeting a third undisclosed PKU mutation. Now encompassing three different base editors for distinct mutations, the company believes the BEAM-304 program has the potential to address up to 50% of PKU patients in the U.S., with ongoing research effort to address additional pathogenic mutations.
- Beam continues clinical start-up activities for the planned Phase 1/2 trial of BEAM-304. The trial will initially evaluate safety, tolerability and reduction of blood phenylalanine (Phe) levels in patients with the R408W mutation, with the goal of establishing clinical proof of concept for base editing in PKU. The trial is designed to subsequently evaluate base editors for additional mutations within a single clinical program. Beam expects to initiate dosing in the Phase 1/2 trial in early 2027.

BEAM-301: BEAM-301, an investigational base editing therapy, aims to correct one of the most common disease-causing mutations, R83C, in

patients with glycogen storage disease type Ia (GSDIa).

- In October, Beam presented initial clinical data from the ongoing Phase 1/2 trial of BEAM-301 at the European Society of Gene & Cell Therapy (ESGCT) Congress. Initial BEAM-301 clinical data demonstrated dose-dependent improvements in time to fasting hypoglycemia (TTH) across all patients, with two of three patients in the 40 mg cohort achieving the protocol-defined maximum controlled fasting duration of 15 hours, consistent with restored endogenous glucose production, and complete elimination of starch supplementation. BEAM-301 demonstrated an acceptable safety profile across both doses, consistent with the known risks associated with LNP-based therapies.
- Based on the safety and clinical findings observed to date, Beam is enrolling patients in a 60 mg cohort of the ongoing open-label Phase 1/2 dose-exploration study and plans to engage with the U.S. FDA regarding a potential path to approval.

Hematology

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

- Beam expects to report updated clinical data by year-end 2026 and submit a U.S. biologics license application (BLA) for risto-cel as early as year-end 2026.

Next-generation Programs in Sickle Cell Disease and Hematology:

- 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 advanced lead optimization.

Corporate Updates

- In September, Beam appointed Eric Foster as chief commercial officer to lead the company's commercial organization and strategy as its hematology and genetic disease programs advance toward potential launches. Mr. Foster is a seasoned biopharmaceutical executive with more than 25 years of commercial leadership experience, including senior roles at Ardelyx, Horizon Therapeutics, and GSK.

Upcoming Events
Beam management will participate in fireside chats at the following upcoming investor events:

- **Guggenheim 3rd Annual Healthcare Innovation Conference in Boston:**
  Tuesday, November 10, 2026, at 9:00 a.m. ET.
- **UBS Global Biopharma Conference in London:**
  Tuesday, November 17, 2026, at 2:00 p.m. GMT.
- **Jefferies Global Healthcare Conference in London:**
  Wednesday, November 18, 2026, at 1:00 p.m. GMT.
- **9th Annual Evercore Healthcare Conference in Miami:**
  Wednesday, December 2, 2026, at 8:20 a.m. ET.

The live webcasts will be available in the investor section of the company's website at www.beamtx.com and will be archived for 60 days following each presentation.

Third Quarter 2026 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.0 billion as of September 30, 2026, compared to $1.2 billion as of December 31, 2025.
- **Research & Development (R&D) Expenses:** R&D expenses were $110.1 million for the third quarter of 2026, compared to $109.8 million for the third quarter of 2025.
- **General & Administrative (G&A) Expenses:** G&A expenses were $31.5 million for the third quarter of 2026, compared to $26.7 million for the third quarter of 2025.
- **Net Income (Loss):** Net loss was $134.4 million, or $1.28 per share, for the third quarter of 2026, compared to net loss of $112.6 million, or $1.10 per share, for the third quarter of 2025.

Cash Runway

Beam expects that its cash, cash equivalents and marketable securities as of September 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, assuming satisfaction of the conditions applicable to those additional borrowings. This runway is expected to support the company through the potential 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, and our next-generation programs, including our development of targeted LNPs for hematopoietic stem cell delivery; 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, including the anticipated timing of clinical trial initiation and expansion; our planned submission of a BLA for risto-cel; our expectations regarding the potential launch of risto-cel; the potential for an accelerated approval path for BEAM-302; our anticipated regulatory interactions and filings; our expectations regarding the funds that will be available to draw under our credit facility with Sixth Street; 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 intentions regarding management's participation in future investor events; 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 with Sixth Street; 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)

	September 30, 2026	December 31, 2025
Cash, cash equivalents, and marketable securities	$ 1,043,873	$ 1,245,210
Total assets	1,269,072	1,481,177
Total liabilities	316,398	242,819
Total stockholders' equity	952,674	1,238,358

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
License and collaboration revenue	$ 307	$ 9,698	$ 32,535	$ 25,634
Operating expenses:				
Research and development	110,110	109,769	309,730	310,343
General and administrative	31,519	26,740	97,895	81,539
Total operating expenses	141,629	136,509	407,625	391,882
Loss from operations	(141,322)	(126,811)	(375,090)	(366,248)
Other income (expense):				

Change in fair value of derivative liabilities	4,700	(2,600)	3,000	1,900
Change in fair value of non-controlling equity investments	(1,021)	4,937	(667)	7,271
Change in fair value of contingent consideration liabilities	(2,856)	1,000	(2,547)	945
Gain on sale of equity method investment	—	—	455	—
Interest and other income (expense), net	6,094	10,903	23,448	33,093
Total other income (expense)	6,917	14,240	23,689	43,209
Net loss	$ (134,405)	$ (112,571)	$ (351,401)	$ (323,039)
Unrealized gain (loss) on marketable securities	(1,245)	780	(4,545)	111
Comprehensive loss	$ (135,650)	$ (111,791)	$ (355,946)	$ (322,928)
Net loss per common share, basic and diluted	$ (1.28)	$ (1.10)	$ (3.38)	$ (3.31)
Weighted-average common shares outstanding, basic and diluted	104,698,517	102,570,801	104,100,991	97,567,229