



Beam Therapeutics Reports Fourth Quarter and Year-End 2025 Financial Results and Announces New Liver-Targeted Genetic Disease Program in Phenylketonuria (PKU)

February 24, 2026

New Program Designed as Platform-based Approach for Direct Correction of Mutations Causing PKU; Investigational New Drug (IND) Filing for BEAM-304 Anticipated in 2026

Updated Phase 1/2 Data and Next Steps for Pivotal Development for BEAM-302 in Alpha-1 Antitrypsin Deficiency (AATD) on Track for Q1 2026

Strategic Financing Agreement with Sixth Street Provides up to \$500 Million in Long-term, Non-dilutive Capital to Fund Anticipated Launch of Risto-cel in Sickle Cell Disease (SCD); U.S. Biologics License Application (BLA) Submission Expected as Early as Year-End 2026

Expected Cash Runway Now into Mid-2029 Through Execution of Key Clinical, Regulatory and Commercial Milestones

Beam to Host Investor Webcast Today, February 24, 2026, at 8:00 a.m. ET

CAMBRIDGE, Mass., Feb. 24, 2026 (GLOBE NEWSWIRE) -- [Beam Therapeutics Inc.](#) (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported financial results for the fourth quarter and year ended December 31, 2025, and reiterated 2026 milestones. In addition, the company announced the expansion of its liver-targeted genetic disease franchise with a new program, BEAM-304, for the treatment of phenylketonuria (PKU), a disease with significant unmet need that affects approximately 20,000 individuals in the U.S.

"In 2025, we established base editing as a best-in-class technology for genetic medicine, with positive proof-of-concept data and regulatory and clinical development paths to approval across multiple high-value programs," said John Evans, chief executive officer of Beam Therapeutics. "The announcement of BEAM-304 for PKU marks an important expansion of our pipeline and exemplifies the power and scalability of our platform. By combining our clinically validated base editing technology with our internally discovered and optimized lipid nanoparticle (LNP) delivery, we have the potential to bring forward one-time, durable treatments for the vast majority of PKU patients. Moreover, with BEAM-304 we are pursuing an innovative and efficient development approach designed to advance multiple base editors within a single clinical program to address different PKU patient populations – one of the first such programs to reach the clinic."

"As we look ahead to 2026, our focus is on execution across our most advanced programs, including reporting updated Phase 1/2 data and further defining the pivotal path forward for BEAM-302 and preparing for a potential BLA submission for risto-cel as early as year-end. Supported by a balance sheet that was further strengthened through the non-dilutive financing with Sixth Street and anticipated runway into mid-2029, we believe Beam is well positioned to deliver on our clinical, regulatory, and commercial objectives and to bring transformative genetic medicines to patients."

New Liver-targeted Genetic Disease Program: BEAM-304 for the Treatment of PKU

Beam's newest liver-targeted genetic disease program, 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 PKU. PKU is a rare, inherited metabolic disorder that results in toxic accumulation of phenylalanine (Phe), leading to serious neurologic and neurocognitive impairments and lifelong dietary management. By correcting mutations in the *PAH* gene, BEAM-304 aims to reduce toxic Phe to within recommended guidelines while enabling normalization of diet and freedom from medical food.

Beam is advancing BEAM-304 using an innovative development approach in which multiple mutation-specific base editors are developed efficiently within a single clinical program. With this approach, Beam's platform has the potential to create transformative, one-time therapies for the vast majority of patients with PKU. Initial clinical development will focus on base editors addressing the two most prevalent variants found in nearly half of patients with PKU in the U.S., with ongoing research effort to address additional pathogenic mutations. Preclinical data with both base editors demonstrate that BEAM-304 normalized plasma Phe levels in mouse models at clinically relevant doses with robust on-target editing in the liver.

Beam expects to file an investigational new drug (IND) application with the U.S. Food and Drug Administration (FDA) for BEAM-304 in 2026 following completion of pre-IND activities. The 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 and laying the foundation for future expansion to patients with additional *PAH* mutations.

Recent Highlights and 2026 Anticipated Milestones

Corporate

- Today, Beam [announced](#) that it has entered into a strategic financing agreement with Sixth Street for significant, long-term, non-dilutive capital to fund the potential launch of risto-cel in sickle cell disease (SCD). The \$500 million senior secured credit facility includes: \$100 million funded at close; an additional \$300 million available following the achievement of certain clinical, regulatory, and commercial milestones for risto-cel; and an additional \$100 million available at Beam's option, subject to mutual agreement between Sixth Street and Beam, during the seven-year term of the agreement, with principal repayment due by early 2033. Beam is expecting to draw a minimum of \$200 million of capital under the overall facility.

- In December 2025, at the completion of the four-year research collaboration agreement between Pfizer and Beam focused on *in vivo* base editing programs, Pfizer opted in to an exclusive, worldwide license for a liver-targeted development candidate. The development candidate employs Beam's proprietary, liver-targeting LNP to deliver base editing reagents. In connection with the opt-in, Pfizer will take an exclusive, worldwide license to the development candidate, after which it will be responsible for all development activities, as well as potential regulatory approvals, manufacturing, and commercialization. Beam will be eligible for development, regulatory and commercial milestone payments and will have a right to opt in, at the end of Phase 1/2 clinical trials, upon the payment of an option exercise fee, to a global co-development and co-commercialization agreement pursuant to which Beam and Pfizer would share net profits as well as development and commercialization (including manufacturing) costs in a 35%/65% ratio (Beam/Pfizer).

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 January, Beam shared that it has reached alignment with the FDA on a potential accelerated approval pathway for BEAM-302 based on AAT biomarkers evaluated over 12 months. To support a future biologics license application (BLA) submission, the company anticipates enrolling approximately 50 additional patients to be treated with the selected optimal biological dose of BEAM-302 in an expansion of the ongoing Phase 1/2 study.
- Beam expects to report updated data from the Phase 1/2 trial and next steps for pivotal development by the end of the first quarter of 2026.

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. Dosing is complete in the first cohort and enrollment has been initiated in the second cohort.
- 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 SCD.

- Updated data from the ongoing BEACON Phase 1/2 trial [presented](#) at the 67th American Society of Hematology (ASH) Annual Meeting continue to demonstrate risto-cel's differentiated profile, including deep resolution of SCD markers, reduced hospitalization, rapid engraftment, and a predictable manufacturing process that may improve patient experience and treatment center capacity and reduce the length of the transplant process.
- Manufacturing of all clinical doses in the BEACON Phase 1/2 trial is complete.
- 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.

Fourth Quarter and Full-year 2025 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$1.25 billion as of December 31, 2025, compared to \$850.7 million as of December 31, 2024.
- **Research & Development (R&D) Expenses:** R&D expenses were \$99.3 million for the fourth quarter of 2025 and \$409.6 million for the full year ended December 31, 2025, compared to \$101.4 million for the fourth quarter of 2024 and \$367.6 million for the full year ended December 31, 2024.
- **General & Administrative (G&A) Expenses:** G&A expenses were \$32.3 million for the fourth quarter of 2025 and \$113.8 million for the full year ended December 31, 2025, compared to \$28.7 million for the fourth quarter of 2024 and \$111.5 million for the full year ended December 31, 2024.
- **Net Income (Loss):** Net income attributable to common stockholders was \$244.3 million, or \$2.37 per basic share and \$2.33 per diluted share, for the fourth quarter of 2025 and net loss attributable to common stockholders was \$80.0 million, or \$0.81 per share, for the year ended December 31, 2025, compared to net losses of \$90.4 million, or \$1.09 per share, for the fourth quarter of 2024 and \$376.7 million, or \$4.58 per share, for the full year ended December 31, 2024.

Cash Runway

Beam expects that its cash, cash equivalents and marketable securities as of December 31, 2025, combined with the anticipated \$200 million minimum drawdown from the Sixth Street facility, will enable the company to cover its 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.

Investor Webcast Information

Beam will host a conference call and webcast today, February 24, 2026, at 8:00 a.m. ET to review the PKU program, Sixth Street facility, and fourth quarter and year-end 2025 financial results. A live webcast of the presentation will be available under "Events" in the Investors section of the company's website at www.beamtx.com, and a replay will be available shortly after the event.

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 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, 2024, our Quarterly Report on Form 10-Q for the quarter ended September 30, 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)

| | December 31, 2025 | December 31, 2024 |
|---|----------------------|----------------------|
| Cash, cash equivalents, and marketable securities | \$ 1,245,210 | \$ 850,740 |
| Total assets | 1,481,177 | 1,103,824 |
| Total liabilities | 242,819 | 370,279 |
| Total stockholders' equity | 1,238,358 | 733,545 |

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

| | Three Months Ended December 31, | | Year Ended December 31, | |
|-----------------------------------|---------------------------------|-----------|-------------------------|-----------|
| | 2025 | 2024 | 2025 | 2024 |
| License and collaboration revenue | \$ 114,109 | \$ 30,067 | \$ 139,743 | \$ 63,518 |
| Operating expenses: | | | | |

| | | | | |
|--|--------------------|--------------------|--------------------|---------------------|
| Research and development | 99,275 | 101,444 | 409,618 | 367,561 |
| General and administrative | 32,279 | 28,660 | 113,818 | 111,525 |
| Total operating expenses | <u>131,554</u> | <u>130,104</u> | <u>523,436</u> | <u>479,086</u> |
| Loss from operations | (17,445) | (100,037) | (383,693) | (415,568) |
| Other income (expense): | | | | |
| Change in fair value of derivative liabilities | 50 | (128) | 700 | 2,272 |
| Change in fair value of non-controlling equity investments | (3,329) | (1,090) | 3,942 | (14,093) |
| Change in fair value of contingent consideration liabilities | (765) | (27) | 180 | 1,592 |
| Gain on sale of equity method investment | 255,146 | — | 255,146 | — |
| Interest and other income (expense), net | 10,640 | 10,928 | 43,733 | 49,094 |
| Total other income (expense) | <u>261,742</u> | <u>9,683</u> | <u>303,701</u> | <u>38,865</u> |
| Net loss before income taxes | <u>\$ 244,297</u> | <u>\$ (90,354)</u> | <u>\$ (79,992)</u> | <u>\$ (376,703)</u> |
| Provision for income taxes | — | — | — | (39) |
| Net loss | <u>\$ 244,297</u> | <u>\$ (90,354)</u> | <u>\$ (79,992)</u> | <u>\$ (376,742)</u> |
| Unrealized gain (loss) on marketable securities | 321 | (1,080) | 432 | 75 |
| Comprehensive loss | <u>\$ 244,618</u> | <u>\$ (91,434)</u> | <u>\$ (79,560)</u> | <u>\$ (376,667)</u> |
| Net income (loss) per common share - basic | <u>\$ 2.37</u> | <u>\$ (1.09)</u> | <u>\$ (0.81)</u> | <u>\$ (4.58)</u> |
| Basic weighted-average common shares outstanding | <u>102,876,980</u> | <u>82,824,151</u> | <u>98,905,577</u> | <u>82,313,008</u> |
| Net income (loss) per common share - diluted | <u>\$ 2.33</u> | <u>\$ (1.09)</u> | <u>\$ (0.81)</u> | <u>\$ (4.58)</u> |
| Diluted weighted-average common shares outstanding | <u>104,927,041</u> | <u>82,824,151</u> | <u>98,905,577</u> | <u>82,313,008</u> |