Beam Therapeutics Reports Second Quarter 2025 Financial Results and Provides Update on BEAM-302 Development Progress in Alpha-1 Antitrypsin Deficiency (AATD)

August 5, 2025

With 17 Patients Dosed in the Phase 1/2 Trial, BEAM-302 Continues to Demonstrate Durable Correction of the Disease-causing Mutation, Restoration of AAT Physiology, and a Well Tolerated Safety Profile

BEAM-302 Expanded Dose Exploration Underway in Part A and Enrollment Initiated in Part B; Data from Parts A and B of the Phase 1/2 Trial and Clinical Development Update Expected in Early 2026

Dosing Complete for 30 Sickle Cell Disease Patients and First Adolescent Patient Dosed in BEACON Phase 1/2 Trial of BEAM-101; Updated Data Expected by End of 2025

Ended Second Quarter 2025 with $1.2 Billion in Cash, Cash Equivalents and Marketable Securities; Cash Runway Expected to Support Operating Plans into 2028

CAMBRIDGE, Mass., Aug. 05, 2025 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported second quarter 2025 financial results, provided an update on clinical development progress for BEAM-302 in alpha-1 antitrypsin deficiency (AATD), and reiterated recent corporate and pipeline progress across the company's hematology and genetic disease franchises.

"In the first half of 2025, we made significant clinical, operational and regulatory progress across each of our high-priority programs, and we aim to harness this momentum heading into key catalysts at the end of 2025 and early next year," said John Evans, chief executive officer at Beam. "We have now dosed 30 patients in the BEACON trial of BEAM-101, which has a potential best-in-class profile as a one-time therapy for severe sickle cell disease, marking an important milestone on our path to a BLA filing. We look forward to sharing additional data from this trial later this year."

Mr. Evans continued, "Today, we're pleased to provide an update on the BEAM-302 Phase 1/2 trial in alpha-1 antitrypsin deficiency, where we have mounting evidence to suggest that BEAM-302 is fundamentally altering the disease to restore the key physiologic functions of alpha-1 antitrypsin with a single course of treatment. Having now dosed 17 patients across four cohorts, all doses tested as of August 1 continue to be well tolerated and resulted in durable, dose-dependent correction of the disease-causing mutation. Treatment with BEAM-302 restored production of functional, corrected M-AAT, as well as markedly reduced the mutant protein, Z-AAT, which is the key contributor to disease manifestations. To finalize dose selection for registrational development, we are expanding the dose exploration phase of Part A and have initiated enrollment in Part B with patients who have mild to moderate liver disease. We look forward to providing a full program update, including data from both parts of the trial and next steps for BEAM-302 development in early 2026. We are committed to rapidly advancing this promising novel therapeutic for AATD patients, who have a significant need for treatments that can address the underlying cause of their disease."

BEAM-302 Development Progress and Clinical Update

Positive initial safety and efficacy data from the Phase 1/2 trial of BEAM-302 were previously reported in March 2025, establishing clinical proof of concept for BEAM-302 as a potential single-course treatment for AATD through *in vivo* base editing correction of the causative genetic mutation. Preliminary results for nine patients from the first three single-ascending dose cohorts from Part A of the trial, designed to evaluate BEAM-302 in AATD patients with lung disease, demonstrated that treatment was well tolerated, and single doses of BEAM-302 led to increases in total and functional alpha-1 antitrypsin (AAT) to therapeutic levels, as well as a significant reduction in the mutant protein (Z-AAT), thereby addressing the underlying pathophysiology of both the liver and lung disease.

To finalize dose selection and prepare BEAM-302 for registrational development, Beam has expanded dose exploration in Part A of the Phase 1/2 trial and initiated enrollment in Part B, designed to evaluate AATD patients with mild to moderate liver disease with or without lung disease. In Part A, Beam is enrolling a total of six patients each in the 60 mg and 75 mg cohorts and initiated screening for a multi-dose cohort of two 60 mg doses administered eight weeks apart. Continued dose escalation may be evaluated based on ongoing safety and efficacy findings. In Part B, patients will initially receive 30 mg of BEAM-302, followed by additional dose escalation cohorts.

As of August 1, 2025, a total of 17 patients have been dosed in Part A, with follow-up ranging from three days to 14 months. All adverse events (AEs) were mild to moderate, with no serious AEs and no dose-limiting toxicities reported. All liver transaminase elevations continued to be Grade 1 and resolved to normal without intervention. All infusion-related reactions were mild to moderate. Treatment with BEAM-302 continued to demonstrate restoration of AAT physiology by inducing production of corrected and functional M-AAT, reducing circulating mutant Z-AAT, and correcting the disease-causing mutation in a dose-dependent manner, as measured by the percent change in total circulating AAT from baseline. Changes in total and functional AAT levels and the ratio of circulating M-AAT to Z-AAT continued to be durable for all patients.

Beam expects to finalize dose selection for registrational development based on the totality of data from the BEAM-302 trial. Clinical data from Part A and Part B are expected to be shared in early 2026, along with an updated clinical development plan for BEAM-302 in patients with AATD.

Second Quarter 2025 and Recent Progress

- In July, Beam completed dosing of 30 patients in the BEACON Phase 1/2 study of BEAM-101, an investigational genetically modified cell therapy for the treatment of patients with sickle cell disease (SCD) with severe vaso-occlusive

crises (VOCs). In addition, the first adolescent patient has been dosed in the trial. Enrollment is complete in both the adult and adolescent cohorts in BEACON.
- New clinical data with more patients and longer follow-up from the BEACON Phase 1/2 clinical trial of BEAM-101 were presented at the European Hematology Association (EHA) 2025 Congress in June, further supporting its differentiated profile as a potential best-in-class treatment for SCD.
- Also in June, the United States (U.S.) Food and Drug Administration (FDA) granted orphan drug designation to BEAM-101. The designation is designed to support the development and evaluation of treatments for rare diseases.
- In May, the U.S. FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to BEAM-302. The designation is designed to support the development and evaluation of regenerative medicines, with the intention of addressing serious or life-threatening diseases that have unmet medical needs.
- Also in May, the U.S. FDA granted orphan drug designation to BEAM-302.

Key Anticipated Milestones

Liver-targeted Genetic Disease Franchise

- Beam expects to report data from the dose-escalation portions of Part A and Part B of the BEAM-302 Phase 1/2 trial and provide a clinical development update in early 2026.
- Beam plans to continue dosing in the Phase 1/2 clinical trial of BEAM-301 in glycogen storage disease Ia (GSDIa).

Hematology Franchise

- Beam plans to present updated data from the BEACON Phase 1/2 trial at the end of 2025.
- The company expects to initiate a Phase 1 healthy volunteer clinical trial of BEAM-103, the ESCAPE monoclonal antibody, by the end of 2025.

Second Quarter 2025 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.2 billion as of June 30, 2025, compared to $850.7 million as of December 31, 2024.
- **Research & Development (R&D) Expenses:** R&D expenses were $101.8 million for the second quarter of 2025, compared to $87.0 million for the second quarter of 2024.
- **General & Administrative (G&A) Expenses:** G&A expenses were $26.9 million for the second quarter of 2025, compared to $29.6 million for the second quarter of 2024.
- **Net Income (Loss):** Net loss was $102.3 million, or $1.00 per share, for the second quarter of 2025, compared to $91.1 million, or $1.11 per share, for the second quarter of 2024.

Cash Runway
Beam expects that its cash, cash equivalents and marketable securities as of June 30, 2025, 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 and anticipated timing to present data from ongoing clinical trials; 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, our Quarterly Reports on Form 10-Q, 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)

	June 30, 2025	December 31, 2024
Cash, cash equivalents, and marketable securities	$ 1,150,337	$ 850,740
Total assets	1,391,157	1,103,824
Total liabilities	344,344	370,279
Total stockholders' equity	1,046,813	733,545

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
License and collaboration revenue	$ 8,466	$ 11,772	$ 15,936	$ 19,182
Operating expenses:				
Research and development	101,758	87,041	200,574	171,859
General and administrative	26,859	29,626	54,799	56,350
Total operating expenses	128,617	116,667	255,373	228,209
Loss from operations	(120,151)	(104,895)	(239,437)	(209,027)
Other income (expense):				
Change in fair value of derivative liabilities	1,147	5,500	3,407	2,600
Change in fair value of non-controlling equity investments	4,415	(7,586)	2,334	(10,939)
Change in fair value of contingent consideration liabilities	(28)	1,779	(55)	1,646
Interest and other income (expense), net	12,326	14,190	22,190	26,039
Total other income (expense)	17,860	13,883	27,876	19,346
Net loss before income taxes	$ (102,291)	$ (91,012)	$ (211,561)	$ (189,681)
Provision for income taxes	—	(39)	—	(39)
Net loss	$ (102,291)	$ (91,051)	$ (211,561)	$ (189,720)
Unrealized gain (loss) on marketable securities	(150)	(189)	(669)	(1,714)
Comprehensive loss	$ (102,441)	$ (91,240)	$ (212,230)	$ (191,434)
Net loss per common share, basic and diluted	$ (1.00)	$ (1.11)	$ (2.23)	$ (2.31)
Weighted-average common shares outstanding, basic and diluted	101,995,184	82,312,467	95,023,977	82,005,550