Beam Therapeutics Reports Fourth Quarter and Year-End 2024 Financial Results and Reiterates Anticipated Catalysts

February 25, 2025

Enrollment Target for Adult Sickle Cell Disease Patients Achieved in BEACON Trial of BEAM-101; Dosing of 30 Patients and Updated Data Expected by Mid-2025

Initial Data from Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency Expected in First Half 2025

Dosing Expected to Initiate in Phase 1/2 Trial of BEAM-301 in Glycogen Storage Disease Type 1a in Early 2025

IND-enabling Studies of ESCAPE Nongenotoxic Conditioning Approach Ongoing; Healthy Volunteer Study of BEAM-103 Antibody Expected to Initiate by Year-end

Ended Fourth Quarter 2024 with $850.7 Million in Cash, Cash Equivalents and Marketable Securities; Cash Runway Expected to Support Operating Plans into 2027

CAMBRIDGE, Mass., Feb. 25, 2025 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported fourth quarter and full year 2024 financial results and reiterated anticipated milestones across the company's hematology and genetic disease franchises.

"We are incredibly proud of the significant progress and momentum across our hematology and liver-targeted genetic disease franchises over the last year," said John Evans, chief executive officer of Beam. "On the heels of the initial positive results from the BEACON trial for BEAM-101 in patients with sickle cell disease, we recently achieved our adult enrollment target in the study and enrolled our first adolescent patients. Additionally, we remain on track to deliver initial data for our lead *in vivo* program, BEAM-302 in alpha-1 antitrypsin deficiency, in the first half of 2025, where we have the potential for a one-time treatment to address both the lung and liver manifestations of disease. With a strong financial position and important catalysts on the horizon, we are well equipped to continue driving forward our mission of providing life-long cures to patients suffering from serious diseases."

Fourth Quarter 2024 and Recent Progress

- The company has achieved its adult enrollment target for the BEACON Phase 1/2 clinical trial of BEAM-101, an investigational genetically modified cell therapy for the treatment of sickle cell disease (SCD). In addition, multiple adolescent patients have cleared screening and enrolled in the trial.
- In December, Beam presented initial results for the BEACON trial at the 66th American Society of Hematology (ASH) Annual Meeting, which demonstrated that treatment with BEAM-101 induced robust and durable increases in fetal hemoglobin and reductions in sickle hemoglobin, rapid neutrophil and platelet engraftment, and normalized or improved markers of hemolysis. The presentation subsequently received the "Best of ASH" distinction, and an encore of the data was presented at the 2025 Tandem Meetings of ASTCT and CIBMTR.
- Also at ASH, Beam presented proof-of-concept data in non-human primates for its Engineered Stem Cell Antibody Evasion (ESCAPE) platform demonstrating engraftment of base-edited cells using only antibody conditioning and no chemotherapy. The company initiated Phase 1-enabling preclinical toxicology studies for ESCAPE in December.
- The company continues to advance global regulatory and site activation activities for BEAM-302, an *in vivo* base editor being developed for the potential treatment of alpha-1 antitrypsin deficiency (AATD), with sites now open in the United Kingdom, New Zealand, Australia and Netherlands.
- In January, Beam activated the first clinical trial site for the Phase 1/2 clinical trial of BEAM-301, an *in vivo* base editor being developed for the potential treatment of glycogen storage disease type 1a (GSD1a).
- In December, Beam appointed Sravan Emany as chief financing officer. In addition, Chirfi Guindo, chief marketing officer of Human Health at Merck & Co., Inc., was appointed to its board of directors.

Key 2025 Anticipated Milestones

Hematology Franchise

- In the BEACON Phase 1/2 clinical trial of BEAM-101 in adults with severe SCD, Beam expects to present updated data in mid-2025.
- Beam expects to dose 30 patients in the BEACON trial by mid-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.

Liver-targeted Genetic Disease Franchise

- Beam expects to report initial data from multiple cohorts from the Phase 1/2 study of BEAM-302 in AATD in the first half of 2025.
- Patient dosing in the Phase 1/2 clinical trial of BEAM-301 in GSD1a is expected to commence in early 2025.

Fourth Quarter and Full Year 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $850.7 million as of December 31, 2024, compared to $1.2 billion as of December 31, 2023.
- **Research & Development (R&D) Expenses:** R&D expenses were $101.4 million for the fourth quarter of 2024 and $367.6 million for the full year ended December 31, 2024, compared to $140.1 million for the fourth quarter of 2023 and $437.4 million for the full year ended December 31, 2023.
- **General & Administrative (G&A) Expenses**: G&A expenses were $28.7 million for the fourth quarter of 2024 and $111.5 million for the full year ended December 31, 2024, compared to $43.3 million for the fourth quarter of 2023 and $116.8 million for the full year ended December 31, 2023.
- **Net Income (Loss):** Net loss attributable to common stockholders was $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 year ended December 31, 2024, compared to net income attributable to common stockholders of $142.8 million, or $1.77 per basic share and $1.73 per diluted share, for the fourth quarter of 2023 and net loss attributable to common stockholders of $132.5 million, or $1.72 per share, for the year ended December 31, 2023.

Cash Runway
Beam expects that its cash, cash equivalents and marketable securities as of December 31, 2024, will enable the company to fund its anticipated operating expenses and capital expenditure requirements into 2027. 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 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 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, GSD1a, and ESCAPE; our plans, and anticipated timing, to advance our programs; 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; 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 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, 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, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	$ 850,740	$ 1,189,876
Total assets	1,103,824	1,459,714
Total liabilities	370,279	478,385
Total stockholders' equity	733,545	981,329

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

	Three Months Ended December 31,		Years Ended December 31,	
	2024	2023	2024	2023
License and collaboration revenue	$ 30,067	$ 316,192	$ 63,518	$ 377,709
Operating expenses:				
Research and development	101,444	140,077	367,561	437,381
General and administrative	28,660	43,257	111,525	116,813
Total operating expenses	130,104	183,334	479,086	554,194
Income (loss) from operations	(100,037)	132,858	(415,568)	(176,485)
Other income (expense):				
Change in fair value of derivative liabilities	(128)	(1,900)	2,272	7,500
Change in fair value of non-controlling equity investments	(1,090)	(722)	(14,093)	(18,592)
Change in fair value of contingent consideration liabilities	(27)	1,863	1,592	9,740
Interest and other income (expense), net	10,928	12,064	49,094	46,676
Total other income (expense)	9,683	11,305	38,865	45,324
Net income (loss) before income taxes	(90,354)	144,163	(376,703)	(131,161)
Provision for income taxes	—	(1,366)	(39)	(1,366)
Loss from equity method investment	—	—	—	—
Net income (loss)	$ (90,354)	$ 142,797	$ (376,742)	$ (132,527)
Unrealized gain (loss) on marketable securities	(1,080)	2,628	75	3,034
Comprehensive income (loss)	$ (91,434)	$ 145,425	$ (376,667)	$ (129,493)
Net income (loss) per common share - basic	$ (1.09)	$ 1.77	$ (4.58)	$ (1.72)
Basic weighted-average common shares outstanding	82,824,151	80,858,517	82,313,008	77,151,771
Net income (loss) per common share - diluted	$ (1.09)	$ 1.73	$ (4.58)	$ (1.72)
Diluted weighted-average common shares outstanding	82,824,151	82,702,302	82,313,008	77,151,771