# Beam Therapeutics Reports Third Quarter 2024 Financial Results and Progress Across Priority Programs

November 5, 2024

*Initial Clinical Data for BEAM-101 and Preclinical Non-human Primate Data for ESCAPE Accepted for Presentation at American Society of Hematology (ASH) Annual Meeting*

*35 Patients Enrolled and Eight Patients Dosed in BEACON Phase 1/2 Trial of BEAM-101 in Sickle Cell Disease*

*First Cohort Dosing Completed in Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency; Initial Clinical Data Expected in 2025*

*Ended Third Quarter 2024 with $925.8 Million in Cash, Cash Equivalents and Marketable Securities; Expected Operating Runway into 2027*

*Company to Host Conference Call Today, November 5, 2024, at 8:30 a.m. ET*

CAMBRIDGE, Mass., Nov. 05, 2024 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported third quarter 2024 financial results and provided updates across the company's hematology and genetic disease franchises.

"In the third quarter, we demonstrated strong execution across our priority hematology and liver genetic disease programs, with progress in clinical site activation, patient enrollment and dosing," said John Evans, chief executive officer of Beam. "The imminent presentation of our BEAM-101 clinical data in patients with sickle cell disease, along with preclinical data for ESCAPE, at the ASH Annual Meeting is an important milestone for Beam's base editing technology. In our *in vivo* portfolio, the BEAM-302 clinical program in alpha-1 antitrypsin deficiency is expanding globally, with dosing complete for the first cohort of patients and data anticipated in 2025, while BEAM-301 has achieved an open IND with the FDA upon first review. We are now entering a catalyst-rich period for Beam, with a strong cash position supporting both the execution of our development programs and our long-term investment in a highly differentiated product engine."

**Third Quarter 2024 and Recent Progress**

- Four Beam abstracts were accepted for presentation at the upcoming American Society of Hematology (ASH) Annual Meeting, including two abstracts on the initial clinical data from the BEACON trial of BEAM-101, one on the clinical data for BEAM-201, and one on the preclinical non-human primate (NHP) data for the Engineered Stem Cell Antibody Paired Evasion (ESCAPE) conditioning platform. Beam will host an investor webcast and conference call today at 8:30 a.m. ET to review the abstracts. Abstracts will be available on the ASH website today at 9 a.m. ET.
- To date, 35 patients have cleared screening and enrolled in the BEACON Phase 1/2 clinical trial of BEAM-101, an investigational genetically modified cell therapy for the treatment of sickle cell disease (SCD). Of these patients, eight have been dosed with BEAM-101, with the other enrolled patients going through pre-transplant stages, including cell collection and drug product manufacturing.
- Patient enrollment in the Phase 1/2 clinical trial of BEAM-302 in patients with alpha-1 antitrypsin deficiency (AATD) is progressing, with continued site activation globally and dosing completed for the first cohort.
- Beam has nominated a development candidate for its ESCAPE technology comprised of two investigational drug products: BEAM-103, an anti-CD117 monoclonal antibody (mAb), and BEAM-104, a cell therapy that includes the same therapeutic edit as BEAM-101 (editing the HBG1/2 genes to elevate fetal hemoglobin), plus an additional edit to CD117 which is designed to prevent binding of BEAM-103, allowing the edited cells to evade suppression by the antibody. The company intends to advance BEAM-103 and BEAM-104 for development in SCD and beta-thalassemia, potentially building on the same regulatory, manufacturing, clinical and commercial foundations being established for BEAM-101.

**Key Anticipated Milestones**

*Hematology Franchise*

- In conjunction with the ASH Annual Meeting taking place December 7-10, 2024, Beam will present additional data from the BEACON Phase 1/2 clinical trial of BEAM-101 and additional preclinical NHP data for ESCAPE. The company will host an investor event to review the data on Sunday, December 8, at 8 p.m. PT.
- The company anticipates initiating Phase 1-enabling preclinical studies for ESCAPE by the end of 2024.

*Genetic Disease Franchise*

- Beam expects to report initial clinical data from multiple cohorts in the Phase 1/2 clinical trial of BEAM-302 in patients with AATD in 2025.

- The company is continuing site activation activities for the Phase 1/2 clinical trial for BEAM-301 in glycogen storage disease type 1a (GSDIa), with patient dosing expected to commence in early 2025.

*Oncology*

- Data from the Phase 1/2 clinical trial of BEAM-201, a multiplex-edited allogeneic CAR-T product candidate for the treatment of relapsed/refractory T-cell acute lymphoblastic leukemia (T-ALL)/T-cell lymphoblastic lymphoma (T-LL), will be presented at the ASH Annual Meeting.

**Third Quarter 2024 Financial Results**

- **Cash Position:** Cash, cash equivalents and marketable securities were $925.8 million as of September 30, 2024, compared to $1.2 billion as of December 31, 2023.
- **Research & Development (R&D) Expenses:** R&D expenses were $94.3 million for the third quarter of 2024, compared to $100.0 million for the third quarter of 2023.
- **General & Administrative (G&A) Expenses:** G&A expenses were $26.5 million for the third quarter of 2024, compared to $25.4 million for the third quarter of 2023.
- **Net Loss:** Net loss was $96.7 million for the third quarter of 2024, or $1.17 per share, compared to $96.1 million for the third quarter of 2023, or $1.22 per share.

**Cash Runway**
Beam expects that its cash, cash equivalents and marketable securities as of September 30, 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, BEAM-103, BEAM-104, BEAM-301 and BEAM-302 described above, as well as continued investments in platform advancements and manufacturing capabilities, and excludes commercial spend related to the potential launch of BEAM-101.

**Conference Call and Webcast Details**
Beam will host a conference call and webcast to discuss these updates today, November 5, 2024, at 8:30 a.m. ET. A live webcast of the presentation will be available here and under "Events & Presentations" in the Investors section of the company's website at www.beamtx.com. A replay of the webcast will be archived on the company's website for 60 days following the presentation.

**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, T-ALL/T-LL, AATD, GSDIa, and ESCAPE; our plans, and anticipated timing, to advance our programs; the clinical trial designs and expectations for BEAM-101, BEAM-201, BEAM-301, BEAM-302 and ESCAPE; our potential presentations at the ASH annual meeting; our estimated cash, cash equivalents and marketable securities as of September 30, 2024 and our expectations related thereto; 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 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, 2023, 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)**

| | September 30, 2024 | December 31, 2023 |
|---|---|---|
| Cash, cash equivalents, and marketable securities | $ 925,757 | $ 1,189,876 |
| Total assets | 1,171,367 | 1,459,714 |
| Total liabilities | 380,050 | 478,385 |
| Total stockholders' equity | 791,317 | 981,329 |

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|---|---|---|
| | 2024 | 2023 | 2024 | 2023 |
| License and collaboration revenue | $ 14,269 | $ 17,193 | $ 33,451 | $ 61,517 |
| Operating expenses: | | | | |
| Research and development | 94,258 | 100,050 | 266,117 | 297,304 |
| General and administrative | 26,515 | 25,410 | 82,865 | 73,556 |
| Total operating expenses | 120,773 | 125,460 | 348,982 | 370,860 |
| Loss from operations | (106,504) | (108,267) | (315,531) | (309,343) |
| Other income (expense): | | | | |
| Change in fair value of derivative liabilities | (200) | 4,700 | 2,400 | 9,400 |
| Change in fair value of non-controlling equity investments | (2,064) | (11,221) | (13,003) | (17,870) |
| Change in fair value of contingent consideration liabilities | (27) | 6,002 | 1,619 | 7,877 |
| Interest and other income (expense), net | 12,127 | 12,698 | 38,166 | 34,612 |
| Total other income (expense) | 9,836 | 12,179 | 29,182 | 34,019 |
| Net loss before income taxes | $ (96,668) | $ (96,088) | $ (286,349) | $ (275,324) |
| Provision for income taxes | — | — | (39) | — |
| Net loss | $ (96,668) | $ (96,088) | $ (286,388) | $ (275,324) |
| Unrealized gain (loss) on marketable securities | 2,869 | (9) | 1,155 | 406 |
| Comprehensive loss | $ (93,799) | $ (96,097) | $ (285,233) | $ (274,918) |
| Net loss per common share, basic and diluted | $ (1.17) | $ (1.22) | $ (3.49) | $ (3.63) |
| Weighted-average common shares outstanding, basic and diluted | 82,410,095 | 79,024,647 | 82,141,383 | 75,902,612 |