Beam Therapeutics Reports Pipeline Updates and Second Quarter 2024 Financial Results

August 6, 2024

U.S. Food and Drug Administration Cleared Investigational New Drug (IND) Application for BEAM-301 in Glycogen Storage Disease Type Ia (GSDIa)

More than 20 Patients Enrolled and Six Patients Dosed in BEACON Phase 1/2 Trial of BEAM-101 in Severe Sickle Cell Disease

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

First Patient Dosed in the Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency (AATD); Initial Clinical Data Expected in 2025

Ended Second Quarter 2024 with $1.0 Billion in Cash, Cash Equivalents and Marketable Securities; Expected Operating Runway into 2027

CAMBRIDGE, Mass., Aug. 06, 2024 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported second quarter 2024 financial results and provided updates across the company's hematology and genetic disease franchises.

"This quarter we've made significant progress across our rapidly expanding clinical portfolio, where each program utilizes the power and precision of base editing technology to provide potential best-in-class genetic medicines for patients," said John Evans, chief executive officer of Beam. "In our genetic disease franchise, we're pleased to announce the clearance of our U.S. investigational new drug (IND) application for BEAM-301, our first U.S. *in vivo* regulatory filing. We're focused on initiating site activation activities for BEAM-301 as well as continuing to enroll our BEAM-302 Phase 1/2 clinical trial in alpha-1 antitrypsin deficiency (AATD) following study initiation in June. We look forward to reporting the first data from the BEAM-302 trial next year. In addition, enrollment in the BEACON trial of BEAM-101 in sickle cell disease (SCD) has exceeded expectations, with more than 20 patients enrolled and six dosed, plus additional patients consented and in the screening process. Initial BEAM-101 clinical data have been submitted for presentation at the American Society of Hematology (ASH) Annual Meeting taking place in December, along with abstracts for the first clinical data for BEAM-201 as well as our first ESCAPE preclinical data in non-human primates."

Second Quarter 2024 and Recent Progress

- To date, more than 20 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 SCD. Of these patients, six have been dosed with BEAM-101, with the other enrolled patients going through pre-transplant stages including mobilization and manufacturing.
- In June, Beam reported data at the European Hematology Association (EHA) Hybrid Congress highlighting its optimized, closed and automated manufacturing process for its base-edited CD34+ hematopoietic stem and progenitor cell genetic medicines, which is currently being deployed for the manufacturing of BEAM-101 in the BEACON Phase 1/2 clinical trial. The data, which include both preclinical and GMP clinical manufacturing experience to date, demonstrate that the use of base editing technology plus the advanced CD34+ manufacturing process employed by Beam are achieving reproducible and robust product yields and viability that meet high-quality standards.
- In June, Beam announced that the first patient was treated with BEAM-302, an investigational *in vivo* base editing medicine designed to precisely correct the underlying cause of severe AATD that is currently being evaluated in a Phase 1/2 clinical trial.
- The U.S. Food and Drug Administration has cleared the IND application for BEAM-301, an investigational *in vivo* base editing medicine designed to directly correct the R83C mutation, one of the primary disease-causing mutations of glycogen storage disease type Ia (GSDIa).

Key Anticipated Milestones

Hematology Franchise

- Initial data from the BEACON Phase 1/2 clinical trial have been submitted for presentation at the ASH Annual Meeting, taking place December 7-10, 2024. Pending acceptance, Beam anticipates presenting data on all patients from the sentinel cohort as well as multiple patients from the expansion cohort.
- Beam continues to advance and invest in its Engineered Stem Cell Antibody Paired Evasion (ESCAPE) conditioning platform and anticipates initiating Phase 1-enabling preclinical studies for the program in 2024. Preclinical data for ESCAPE in non-human primates have been submitted for presentation at ASH.

Genetic Disease Franchise

- Beam continues to enroll the Phase 1/2 clinical trial of BEAM-302 in patients with AATD and expects to report initial clinical data in 2025.

- The company is now initiating site activation activities for the Phase 1/2 clinical trial for BEAM-301 in GSDIa with patient dosing expected to commence in early 2025.

Oncology

- Initial 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), have been submitted for presentation at the ASH Annual Meeting.

Second Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.0 billion as of June 30, 2024, compared to $1.2 billion as of December 31, 2023.
- **Research & Development (R&D) Expenses:** R&D expenses were $87.0 million for the second quarter of 2024, compared to $97.6 million for the second quarter of 2023.
- **General & Administrative (G&A) Expenses:** G&A expenses were $29.6 million for the second quarter of 2024, compared to $24.7 million for the second quarter of 2023.
- **Net Loss:** Net loss was $91.1 million for the second quarter of 2024, or $1.11 per share, compared to $82.8 million for the second quarter of 2023, or $1.08 per share.

Cash Runway
Beam expects that its cash, cash equivalents and marketable securities as of June 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, ESCAPE, 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.

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 that includes a suite of gene editing and delivery technologies and is in the process of building 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-301, BEAM-302 and ESCAPE; our potential presentations at the ASH annual meeting; our estimated cash, cash equivalents and marketable securities as of June 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 successfully achieve the benefits of our portfolio prioritization and strategic restructuring; 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, 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, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	$ 1,008,165	$ 1,189,876
Total assets	1,261,266	1,459,714
Total liabilities	407,172	478,385
Total stockholders' equity	854,094	981,329

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
License and collaboration revenue	$ 11,772	$ 20,116	$ 19,182	$ 44,324
Operating expenses:				
Research and development	87,041	97,608	171,859	197,254
General and administrative	29,626	24,656	56,350	48,146
Total operating expenses	116,667	122,264	228,209	245,400
Loss from operations	(104,895)	(102,148)	(209,027)	(201,076)
Other income (expense):				
Change in fair value of derivative liabilities	5,500	(900)	2,600	4,700
Change in fair value of non-controlling equity investments	(7,586)	6,148	(10,939)	(6,649)
Change in fair value of contingent consideration liabilities	1,779	2,171	1,646	1,875
Interest and other income (expense), net	14,190	11,953	26,039	21,914
Total other income (expense)	13,883	19,372	19,346	21,840
Net loss before income taxes	$ (91,012)	$ (82,776)	$ (189,681)	$ (179,236)
Provision for income taxes	(39)	—	(39)	—
Net loss	$ (91,051)	$ (82,776)	$ (189,720)	$ (179,236)
Unrealized gain (loss) on marketable securities	(189)	(1,250)	(1,714)	415
Comprehensive loss	$ (91,240)	$ (84,026)	$ (191,434)	$ (178,821)
Net loss per common share, basic and diluted	$ (1.11)	$ (1.08)	$ (2.31)	$ (2.41)
Weighted-average common shares outstanding, basic and diluted	82,312,467	76,335,175	82,005,550	74,315,721