Beam Therapeutics Reports Pipeline Updates and First Quarter 2024 Financial Results

May 7, 2024

Dosing Completed for Sentinel Cohort of BEACON Phase 1/2 Trial of BEAM-101 in Severe Sickle Cell Disease; Expansion Cohort Initiated

Clinical Trial Authorisation (CTA) Application Cleared for the Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency; Study to Initiate in First Half of 2024

BEAM-301 On Track for Submission of U.S. Investigational New Drug (IND) Application in First Half of 2024

Ended First Quarter 2024 with $1.1 Billion in Cash, Cash Equivalents and Marketable Securities; Cash Runway Expected to Support Operating Plans into 2027

CAMBRIDGE, Mass., May 07, 2024 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported first quarter 2024 financial results and provided updates across the company's hematology and genetic disease franchises.

"We're pleased to share progress across our high-priority programs that exemplify our commitment to rapid, focused execution and a dedication to developing differentiated, one-time medicines for serious genetic diseases," said John Evans, chief executive officer of Beam. "We have successfully completed dosing and engraftment for three sickle cell disease patients in the sentinel cohort of the BEAM-101 BEACON trial allowing us to now move forward with expansion phase dosing. We look forward to sharing data for multiple patients treated with BEAM-101 later this year. In addition, our team has done an incredible job executing our ex-U.S. clinical strategy for BEAM-302, securing CTA clearance in the UK and rapidly working toward the initiation of our Phase 1/2 trial in patients with alpha-1 antitrypsin deficiency. This study is designed to demonstrate proof-of-concept for correction of the disease-causing mutation that could potentially help patients with both lung and liver disease manifestations. These updates, supported by our robust balance sheet, mark a significant stride toward our goal of establishing base editing as a potentially transformative and differentiated therapeutic option for patients in need."

First Quarter 2024 and Recent Progress

- Sequential dosing and engraftment have been successfully completed for the three patients in the sentinel cohort of the BEACON Phase 1/2 clinical trial of BEAM-101 in severe sickle cell disease.
- Following clearance by the data monitoring committee, the expansion cohort of the BEACON trial of BEAM-101, in which patients can be dosed in parallel, is now open with dosing expected to begin imminently.
- In March 2024, Beam announced the clearance of its clinical trial authorisation (CTA) application by the United Kingdom (UK) Medicines and Healthcare Products Regulatory Agency for BEAM-302, the company's priority genetic disease program for the treatment of alpha-1 antitrypsin deficiency (AATD).

Key 2024 Anticipated Milestones

Hematology Franchise

- In the BEACON Phase 1/2 clinical trial of BEAM-101 in adults with severe sickle cell disease, Beam anticipates continuing to enroll and dose patients in the expansion cohort of the trial, with a total target of up to 45 treated patients.
- The company expects to report data from multiple patients in the BEACON trial in the second half of 2024.
- 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.

Genetic Disease Franchise

- Beam expects to initiate the Phase 1/2 clinical trial for BEAM-302 in AATD in the first half of 2024.
- Beam expects to submit an investigational new drug (IND) application in the U.S. for BEAM-301 for the potential treatment of glycogen storage disease type 1a (GSD1a) in the first half of 2024.

First Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.1 billion as of March 31, 2024, compared to $1.2 billion as of December 31, 2023.
- **Research & Development (R&D) Expenses:** R&D expenses were $84.8 million for the first quarter of 2024, compared to $99.6 million for the first quarter of 2023.
- **General & Administrative (G&A) Expenses:** G&A expenses were $26.7 million for the first quarter of 2024, compared to $23.5 million for the first quarter of 2023.

- **Net Loss:** Net loss was $98.7 million for the first quarter of 2024, or $1.21 per share, compared to $96.5 million for the first quarter of 2023, or $1.33 per share.

Cash Runway
Beam expects that its cash, cash equivalents and marketable securities as of March 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, as well as continued investments in platform advancements and manufacturing capabilities.

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 sickle cell disease, AATD, GSD1a, 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 estimated cash, cash equivalents and marketable securities as of March 31, 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 studies; 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 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)

	March 31, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	$ 1,094,554	$ 1,189,876
Total assets	1,359,796	1,459,714
Total liabilities	446,306	478,385
Total stockholders' equity	913,490	981,329

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

	Three Months Ended March 31,	
	2024	2023

License and collaboration revenue	$ 7,410	$ 24,208
Operating expenses:		
Research and development	84,818	99,646
General and administrative	26,724	23,490
Total operating expenses	111,542	123,136
Loss from operations	(104,132)	(98,928)
Other income (expense):		
Change in fair value of derivative liabilities	(2,900)	5,600
Change in fair value of non-controlling equity investments	(3,353)	(12,797)
Change in fair value of contingent consideration liabilities	(133)	(296)
Interest and other income (expense), net	11,849	9,961
Total other income (expense)	5,463	2,468
Net loss	$ (98,669)	$ (96,460)
Unrealized gain (loss) on marketable securities	(1,525)	1,665
Comprehensive loss	$ (100,194)	$ (94,795)
Net loss per common share, basic and diluted	$ (1.21)	$ (1.33)
Weighted-average common shares outstanding, basic and diluted	81,698,633	72,273,829