Beam Therapeutics Reports Pipeline and Business Updates and Third Quarter 2023 Financial Results

November 8, 2023

Recent Portfolio Prioritization Focuses Business on Key Near-term Value Drivers and Long-term Growth of Precision Genetic Medicines Pipeline

Lilly Acquires Beam's Opt-In Rights to Verve Therapeutics' Base Editing Cardiovascular Programs for up to $600 Million in Combined Upfront Payment, Equity Investment and Potential Future Development-Stage Payments

GMP Operations Initiated at Beam's North Carolina Manufacturing Facility

Strong Balance Sheet Provides Anticipated Operating Runway into the Second Half of 2026

CAMBRIDGE, Mass., Nov. 08, 2023 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported third quarter 2023 financial results and provided an update on the business and its clinical and pipeline progress.

"This year, Beam has established an exceptional foundation for future growth, with significant financial strength, a broad portfolio of differentiated base editing programs in large addressable markets driving toward clinical milestones, and a fully operational suite of research, development, and manufacturing capabilities for precision genetic medicines," said John Evans, chief executive officer of Beam. "We have made critical decisions on where to focus and how to prioritize our investments over the coming years, creating significant momentum in our business and portfolio. In the BEACON trial, patient enrollment, mobilization, and manufacturing are progressing well, and we remain on track to treat the first trial patient with BEAM-101 this year and report clinical trial data on multiple patients next year. We're also advancing our first *in vivo* program, BEAM-302, for patients with alpha-1 anti-trypsin deficiency with a regulatory filing expected in the first quarter of next year, followed by our planned IND submission for BEAM-301 shortly thereafter. External interest in our base editing technology continues to grow, illustrated most recently by our transaction with Lilly. With a strong balance sheet, a focused team, and leading capabilities, we are well positioned to advance our base editing programs and platform, which we believe have transformative potential for patients."

Pipeline Updates and Key 2023-2024 Anticipated Milestones

Beam recently announced portfolio priorities and plans to streamline its business operations to support potential near-term value drivers and long-term growth and, as such, is now focusing its efforts as follows:

Hematology Portfolio

- Beam continues to advance its BEACON Phase 1/2 clinical trial, an open-label, single-arm, multicenter study evaluating the safety and efficacy of BEAM-101 in adult patients with severe sickle cell disease (SCD). Treatment with BEAM-101, in which the edited cell product is delivered in an autologous bone marrow transplant, will occur on a sequential basis for the first three patients treated in the trial, and then will be given in parallel for all subsequent patients.
    - Beam has continued to consent additional patients in the BEACON trial, all of whom are now moving in parallel through the screening, mobilization, and manufacturing activities required to enable treatment with BEAM-101. Beam anticipates that currently consented patients are sufficient to both fill the sentinel cohort (n=3) and to initiate the expansion cohort.
    - The first patient in the trial is expected to be treated with BEAM-101 by year-end 2023.
    - Beam will continue adding additional patients to the BEACON trial through the end of year and beyond, with a total target of 45 treated patients.
    - The company anticipates reporting initial data on multiple patients from the BEACON trial in 2024.
- Beam continues to advance and invest in its Engineered Stem Cell Antibody Paired Evasion (ESCAPE) conditioning platform. ESCAPE aims to avoid toxicity challenges associated with currently available conditioning regimens for patients with SCD and beta-thalassemia ahead of autologous transplant.
- In addition, the company is exploring the potential for *in vivo* base editing programs for SCD, in which base editors would be delivered to the patient through an infusion of lipid nanoparticles (LNPs) targeted to hematopoietic stem cells (HSCs), eliminating the need for transplantation altogether.

Genetic Disease (in vivo) Portfolio

- Beam continues to advance its two lead *in vivo* base editing product candidates, BEAM-302 for the treatment of alpha-1 antitrypsin deficiency (AATD) and BEAM-301 for the treatment of glycogen storage disease Ia (GSDIa). BEAM-302 and BEAM-301 have the potential to be the first clinical programs to directly correct a genetic mutation back to a normal functional gene sequence.

- BEAM-302 is a liver-targeting LNP formulation of base editing reagents designed to correct the PiZ allele, the most common gene variant associated with severe AATD. The company expects to submit a regulatory application in the first quarter of 2024 for authorization to initiate clinical trials initially outside of the U.S., with an Investigational New Drug (IND) expected to be filed with the U.S. Food and Drug Administration (FDA) subsequently during early development.
    - In September 2023, Beam reported the first preclinical data demonstrating the ability of BEAM-302 to significantly increase levels of corrected and functional alpha-1 antitrypsin (AAT) and reduce mutant PiZ AAT in multiple *in vivo* rodent disease models at clinically relevant doses in an oral presentation at the Alpha-1 Antitrypsin Deficiency 2023 Meeting in Naples, Italy. These findings support the potential of BEAM-302 to efficiently correct the disease-causal PiZ mutation after a single dose and potentially address both the liver and lung disease associated with AATD.
    - An encore presentation at the ESGCT/SFTCG/NVGCT Collaborative Congress in October 2023 showed additional preclinical data supporting the durable effects of BEAM-302.
- BEAM-301 is a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation, the most common disease-causing mutation that results in the most severe form of GSDIa. Given its rare nature and geographic distribution of disease burden, the company will focus development of BEAM-301 in the U.S. and expects to submit an IND application in the first half of 2024.
    - In October 2023, Beam presented new preclinical data demonstrating the ability of a single administration of BEAM-301 to directly and durably correct the R83C mutation *in vivo*, with results lasting more than one year, in an oral presentation at the 30th Annual European Society of Gene & Cell Therapy (ESGCT) Congress in Brussels.

Immunology/Oncology Portfolio

- In September 2023, Beam announced dosing of the first patient in 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). The Phase 1/2 trial continues to enroll, and the company expects to report initial data in 2024.
- Beam plans to generate a focused clinical dataset for BEAM-201 and seek potential partnership for this and other potential *ex vivo* CAR-T programs, including Beam's ongoing research into creating next-generation allogeneic cell therapies with multiplex base editing.

Research Portfolio

- Beam plans to focus near-term research and platform investments on specific applications leveraging Beam's *in vivo* editing capabilities in the liver targeting both rare genetic and common disorders, as well as select opportunities in hematology and immunology/oncology.

Corporate Updates

- **Manufacturing:** Beam has initiated current good manufacturing practice (GMP) operations at its North Carolina manufacturing facility, where capabilities include both CD34 cell manufacturing and LNP production, with additional capabilities expected to be added in the future.
- **Business Development:** In October 2023, Beam announced that Eli Lilly and Company (Lilly) acquired certain rights under Beam's amended collaboration and license agreement with Verve Therapeutics, Inc. (Verve), including Beam's opt-in rights to co-develop and co-commercialize Verve's base editing programs for cardiovascular disease, which includes programs targeting PCSK9, ANGPTL3, and an undisclosed liver-mediated, cardiovascular target. Beam received a $200 million upfront payment and a $50 million equity investment from Lilly. Beam is also eligible to receive up to $350 million in potential future development-stage payments from Lilly upon the completion of certain clinical, regulatory and alliance events for a total of up to $600 million in potential total deal consideration.

Third Quarter 2023 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities, excluding upfront proceeds from Lilly, were $1.0 billion as of September 30, 2023, as compared to $1.1 billion as of December 31, 2022.
- **Research & Development (R&D) Expenses:** R&D expenses were $100.0 million for the third quarter of 2023, compared to $85.3 million for the third quarter of 2022.
- **General & Administrative (G&A) Expenses:** G&A expenses were $25.4 million for the third quarter of 2023, compared to $21.8 million for the third quarter of 2022.
- **Net Loss:** Net loss was $96.1 million for the third quarter of 2023, or $1.22 per share, compared to $109.6 million for the third quarter of 2022, or $1.56 per share.

Cash Runway

Beam expects that its cash, cash equivalents and marketable securities as of September 30, 2023, together with upfront and equity investment proceeds received from Lilly, will enable the company to fund its anticipated operating expenses and capital expenditure requirements into the second

half of 2026. This expectation assumes anticipated cost savings related to the company's portfolio prioritization and streamlining of operations and includes funding directed toward reaching each of the key anticipated milestones for BEAM-101, BEAM-201, 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 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: our preclinical development plans; the therapeutic applications and potential of our technology, including with respect to SCD, beta-thalassemia, GSDIa, T-ALL/T-LL, and AATD; our plans, and anticipated timing, to advance our clinical trials and programs, including our 2023-2024 anticipated milestones; our estimated cash, cash equivalents and marketable securities as of September 30, 2023 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; any future payments we may receive under our agreement with Lilly; the potential impact of the portfolio prioritization and strategic restructuring on our operations and development timelines; our ability to seek, establish and maintain a collaboration or partnership to develop our programs with a collaborator or partner; 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 the portfolio prioritization and strategic restructuring, including our ability to seek, establish and maintain partners for certain of our programs; 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 potential impact of pandemics and other health emergencies, including their impact on the global supply chain; 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, 2022, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, our Quarterly Report on Form 10-Q that we will file for the quarter ended September 30, 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.

This press release contains hyperlinks to information that is not deemed to be incorporated by reference in this press release.

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Condensed Consolidated Balance Sheet Data (unaudited)
(in thousands)

	September 30, 2023	December 31, 2022
Cash, cash equivalents, and marketable securities	$ 1,015,457	$ 1,078,134
Total assets	1,290,534	1,341,714
Total liabilities	511,593	608,240
Total stockholders' equity	778,941	733,474

Condensed Consolidated Statement of Operations (unaudited)

(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
License and collaboration revenue	$ 17,193	$ 15,799	$ 61,517	$ 40,883
Operating expenses:				0
Research and development	100,050	85,287	297,304	225,253
General and administrative	25,410	21,815	73,556	65,124
Total operating expenses	125,460	107,102	370,860	290,377
Loss from operations	(108,267)	(91,303)	(309,343)	(249,494)
Other income (expense):				0
Change in fair value of derivative liabilities	4,700	(4,900)	9,400	20,900
Change in fair value of non-controlling equity investments	(11,221)	10,431	(17,870)	(1,378)
Change in fair value of contingent consideration liabilities	6,002	(875)	7,877	(543)
Interest and other income (expense), net	12,698	4,982	34,612	7,686
Total other income (expense)	12,179	9,638	34,019	26,665
Net loss before income taxes	(96,088)	(81,665)	(275,324)	(222,829)
Provision for income taxes	—	(2,410)	—	(2,410)
Loss from equity method investment	—	(25,500)	—	(25,500)
Net loss	$ (96,088)	$ (109,575)	$ (275,324)	$ (250,739)
Unrealized gain (loss) on marketable securities	(9)	(484)	406	(4,624)
Comprehensive loss	$ (96,097)	$ (110,059)	$ (274,918)	$ (255,363)
Net loss per common share, basic and diluted	$ (1.22)	$ (1.56)	$ (3.63)	$ (3.59)
Weighted-average common shares outstanding, basic and diluted	79,024,647	70,343,196	75,902,612	69,758,434