Beam Therapeutics Announces Pipeline and Business Highlights and Reports Second Quarter 2022 Financial Results

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Patient Enrollment into BEACON Phase 1/2 Trial of BEAM-101 On-track for Second Half of 2022

BEAM-201 IND Submitted to FDA; Currently on Clinical Hold

BEAM-102 IND Submission and BEAM-301 IND-enabling Studies On-track for Second Half of 2022

John Lo, Ph.D., Appointed as Chief Commercial Officer

Ended Second Quarter 2022 with $1.2 Billion in Cash, Cash Equivalents and Marketable Securities to Support Advancement of Broad Precision Genetic Medicines Portfolio

CAMBRIDGE, Mass., Aug. 09, 2022 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today provided pipeline and business updates and reported financial results for the second quarter ended June 30, 2022.

“2022 is a critical year for Beam’s transition to becoming a multi-program clinical-stage company, as we prepare for the near-term initiation of patient enrollment in our BEACON Phase 1/2 trial, the first clinical trial evaluating BEAM-101 in patients with sickle cell disease,” said John Evans, chief executive officer of Beam. “In June, we submitted our IND for BEAM-201 for CD7-positive T-cell malignancies and recently received notification from the FDA of a clinical hold on the IND. We look forward to receiving more detail from the FDA and working with them in an effort to advance BEAM-201 for these difficult-to-treat cancer indications. We are on track to further expand our portfolio with a steady cadence of clinical and preclinical milestones expected in the quarters ahead, including the IND submission for BEAM-102, our second program in sickle cell disease, and the initiation of IND-enabling studies for BEAM-301, our first liver-directed base editing program in glycogen storage disease, both targeted in the second half of this year.”

Mr. Evans added, “As pioneers and leaders in the field of base editing, we’ve continued to extend the potential reach of our base editing technology and applications with the development of new base editors, as well as novel base editing-enabled therapeutic strategies, such as our work on non-genotoxic conditioning to improve transplant regimens. We’ve also continued to enhance our team, and I’m thrilled to welcome John Lo as chief commercial officer. John has a deep science background and an extensive track record in the strategic development and commercialization of novel medicines, including cell therapy products, at leading companies. I can’t wait to work with him to advance our portfolio and our vision of providing a new class of precision genetic medicines to patients.”

Pipeline Updates & Anticipated Milestones

Ex Vivo HSC Programs

- Beam remains on track to begin patient enrollment in its BEACON trial, an open-label, single-arm, multicenter, Phase 1/2 clinical trial evaluating the safety and efficacy of BEAM-101 in adult patients with severe sickle cell disease (SCD) in the second half of 2022.
- BEAM-102 continues to progress, and the company plans to submit an investigational new drug (IND) application for BEAM-102 for the treatment of SCD in the second half of 2022.

Ex Vivo T Cell Programs

- Beam submitted its IND for BEAM-201 to the U.S. Food and Drug Administration (FDA) in June 2022, and on July 29, 2022, was notified via e-mail that the IND was placed on clinical hold. The FDA indicated it will provide an official clinical hold letter to Beam within 30 days. Beam plans to provide additional updates pending interaction with the FDA.
- Beyond BEAM-201, Beam is focused on identifying the collection of multiplex base edits required to make cells fully allogeneic, with internal and external data suggesting a higher number of edits will be required to meet this goal. As a result, Beam does not expect to nominate a second CAR-T development candidate in 2022 and anticipates providing further updates in 2023.

In Vivo LNP Liver-targeting Programs

- Beam presented updated preclinical data from its BEAM-301 program at the American Society of Cell and Gene Therapy (ASGCT) meeting, highlighting that BEAM-301 demonstrated high and durable editing efficiency in a mouse model of glycogen storage disease 1a (GSDIa) out to 35 weeks. BEAM-301, a liver-targeting lipid nanoparticle (LNP) formulation of base editing reagents designed to correct the R83C mutation, the most common disease-causing mutation of GSDIa, is on track for initiation of IND-enabling studies in the second half of 2022.

- At ASGCT, Beam also presented new preclinical data from its base editing program targeting the treatment of alpha-1 antitrypsin deficiency, highlighting optimizations made to the editor and the guide RNA that have led to two-fold increases in observed editing potency in mice, leading to potentially clinically relevant increases in circulating alpha-1 antitrypsin at doses below 1 mg/kg.
- Beam plans to present new *in vivo* preclinical data from its multiplex base editing program for the potential treatment of hepatitis B virus (HBV) in a poster titled, "Cytosine base editing inhibits Hepatitis B Virus replication and reduces HBsAg expression *in vitro* and *in vivo*," at the 2022 International HBV Meeting from Sept. 18-22. The data will build on initial *in vitro* data presented in September 2021, which showed that base editing can introduce permanent mutations in covalently closed circular DNA (cccDNA) and prevent HBV rebound in relevant models.
- Beam continues to anticipate the nomination of a second liver-targeted development candidate in 2022.

Recent Research Highlights

- At the Federation of American Societies for Experimental Biology (FASEB) Genome Engineering Conference in June, Beam presented the first research highlighting the company's internal efforts to develop improved transplant conditioning regimens for patients with SCD undergoing hematopoietic stem cell transplantation (HSCT). With a goal of overcoming limitations of today's conditioning regimens, Beam leveraged its base editing capabilities to develop a potentially non-genotoxic approach that combines antibody-based conditioning with multiplex gene-edited hematopoietic stem cells (HSCs) called ESCAPE, or Engineered Stem Cell Antibody Paired Evasion. These improved conditioning regimens could potentially be paired with BEAM-101 and BEAM-102, as well as other future programs.
- At the CRISPR 2.0 conference in June, Beam highlighted research that led to the creation of an improved class of cytosine base editors (CBEs), leveraging a TadA enzyme-based CBE (CBE-T), that are capable of editing at levels comparable to traditional CBEs but with lower off-target editing potential. Further, Beam disclosed an additional editor, CABE-T, that can conduct both C-to-T and A-to-G edits with a single TadA deaminase.

Business Updates

- Beam recently appointed John Lo, Ph.D., as chief commercial officer, where he will be responsible for commercial readiness, as well as leading product and portfolio strategy. Dr. Lo joins Beam after serving as an advisor to multiple private- and public-stage biotechnology companies, including Beam. Dr. Lo has held a number of global strategic and operating roles of increasing responsibility within the biopharmaceutical industry, including as senior vice president, worldwide hematology at Bristol Myers Squibb; head of global marketing and market access at Astra Zeneca; corporate vice president, hematology and oncology at Celgene; and multiple P&L roles at Novartis. While at these companies, Dr. Lo successfully helped launch numerous drugs in the U.S. and globally, including Tagrisso in lung cancer and two cell therapies. Dr. Lo has also helped build strategies and grow R&D pipelines as co-chair of the Development Committee at Celgene, leading to multiple pivotal study investments. Dr. Lo also spent several years as an associate principal at McKinsey & Company and holds a Ph.D. in molecular biology from MIT.
- In July, Beam and Verve Therapeutics amended their collaboration and license agreement, originally executed in April 2019. The amended agreement returned two targets to Beam, while adding a new, third target toward an additional liver-mediated, cardiovascular disease target. Beam has the right to opt in on this target after Phase 1 to share 35% of worldwide costs and profits from the program. The two lead targets in the collaboration, PCSK9 and ANGPTL3, are unchanged. Verve also granted to Beam licenses and options to certain delivery technologies.

Second Quarter 2022 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.2 billion as of June 30, 2022, as compared to $965.6 million as of December 31, 2021.
- **Research & Development (R&D) Expenses:** R&D expenses were $74.6 million for the second quarter of 2022, compared to $45.6 million for the second quarter of 2021.
- **General & Administrative (G&A) Expenses:** G&A expenses were $24.1 million for the second quarter of 2022, compared to $13.4 million for the second quarter of 2021.
- **Net Loss:** Net loss was $72.0 million for the second quarter of 2022, or $1.02 per share, compared to $76.3 million for the second quarter of 2021, or $1.23 per share.

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: our upcoming presentation at the 2022 International HBV Meeting; our plans, and anticipated timing, to nominate additional development candidates, initiate IND-enabling studies, submit IND applications, and initiate clinical trials; our expectation that we are on-track to further expand our portfolio with a steady cadence of clinical and preclinical milestones expected in the quarters ahead; our expectations for transitioning to a multi-program clinical stage company; the potential economic benefits that may be achieved under our amended collaboration agreement with Verve Therapeutics; the therapeutic applications and potential of our technology, including with respect to SCD and our conditioning regimens, T-ALL/T-LL, GSDIa, Alpha-1, HBV, and CAR-T cells; the expected timing of enrolling the first subject in our BEACON Phase 1/2 clinical trial of BEAM-101; the clinical hold on our BEAM-201 IND, including the FDA's communication plans related to, and our plans and expectations for interactions with the FDA and the outcomes in connection therewith; the sufficiency of our capital resources to fund operating expenses and capital expenditure requirements; 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 potential impact of the COVID-19 pandemic, including its impact on the global supply chain; the uncertainty that our product candidates, including BEAM-201, will receive regulatory approval necessary to initiate human clinical studies; uncertainty in the FDA's plans to communicate and discuss the clinical hold on the BEAM-201 IND with us and the risk that those discussions may be delayed; the uncertainty in the outcome of our interactions with the FDA regarding the clinical hold on the BEAM-201 IND; 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 enrollment of 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, 2021, 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)

	June 30, 2022	December 31, 2021
Cash, cash equivalents, and marketable securities	$ 1,166,115	$ 965,647
Total assets	1,418,375	1,474,453
Total liabilities	618,214	647,715
Total stockholders' equity	800,161	826,738

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
License and collaboration revenue	$ 16,652	$ 6	$ 25,084	$ 12
Operating expenses:				
Research and development	74,556	45,577	139,966	235,683
General and administrative	24,062	13,403	43,309	23,676
Total operating expenses	98,618	58,980	183,275	259,359
Loss from operations	(81,966)	(58,974)	(158,191)	(259,347)
Other income (expense):				
Change in fair value of derivative liabilities	12,200	(42,300)	25,800	(44,200)
Change in fair value of non-controlling equity investments	(4,124)	25,814	(11,809)	26,852
Change in fair value of contingent consideration liabilities	(120)	(741)	332	(1,046)

Interest and other income (expense), net	2,060	(52)	2,704	(72)
Total other income (expense)	10,016	(17,279)	17,027	(18,466)
Net loss	$ (71,950)	$ (76,253)	$ (141,164)	$ (277,813)
Net loss per common share, basic and diluted	$ (1.02)	$ (1.23)	$ (2.03)	$ (4.54)
Weighted-average common shares outstanding, basic and diluted	70,210,227	62,210,239	69,461,207	61,215,705