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

May 9, 2022

*BEAM-101 Patient Enrollment, BEAM-102 and BEAM-201 IND Submissions and BEAM-301 IND-Enabling Studies All On-track for Second Half of 2022*

*Natural History Study Initiated in People with Sickle Trait to Provide Insights into Key Characteristics of Sickle Cell Trait and Sickle Cell Disease*

*Manmohan Singh, Ph.D., Appointed to Executive Leadership Team, and Anne Marie Woodland Appointed as Head of Regulatory*

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

CAMBRIDGE, Mass., May 09, 2022 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced pipeline and business highlights and financial results for the first quarter ended March 31, 2022. In addition, as part of a long-term effort to better understand sickle cell disease (SCD), a genetic disease in which individuals carry two copies of the sickle cell mutation, and sickle cell trait, in which individuals carry only one copy of the mutation, Beam will fund and collaborate with the National Alliance of Sickle Cell Centers (NASCC) to initiate the AUNT (Achieving Understanding of the Natural History of Sickle Trait) Study, a natural history study of sickle cell trait.

“Base editing has the potential to offer life-changing medicines for a broad range of diseases, and we are committed to better understanding the pathophysiology of the diseases in our pipeline, and their impacts on the lives of patients and their families,” said John Evans, chief executive officer of Beam. “We are excited to be collaborating with the Globin Research Network for Data and Discovery (GRNDad) on the AUNT natural history study in people with sickle cell trait. We are on track and expect to commence SCD patient enrollment in our Phase 1/2 BEACON-101 clinical trial to evaluate the safety and efficacy of BEAM-101 in patients with SCD, as well as make our planned IND submission for BEAM-102, which is also in development for the treatment of SCD. Our immunology and liver-directed pipelines are also progressing, with plans for additional regulatory submissions, research studies and program nominations throughout the year, and we continue to build upon our delivery capabilities, including our novel LNP technology platform, potentially allowing us to expand the future reach of our programs. We believe the parallel advancement of these diverse programs creates a broad foundation for our future growth and is evidence of our commitment to developing new and better treatments for multiple patient populations and to unlocking the full potential of precision genetic medicine.”

**Pipeline Highlights & Anticipated Milestones**

*Ex Vivo* HSC Programs

- Beam continues to advance its BEAM-101 program for the treatment of SCD and expects to enroll the first patient in its Phase 1/2 clinical trial evaluating the safety and efficacy of BEAM-101 for the treatment of SCD, referred to as the BEACON-101 trial, in the second half of 2022.
- Beam’s second SCD-focused program, 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’s BEAM-201 program for the treatment of relapsed/refractory T cell acute lymphoblastic leukemia/T cell lymphoblastic lymphoma is progressing with submission of an IND application anticipated in the second half of 2022.
- Beam continues to make progress towards its planned nomination of a second CAR-T development candidate in 2022.

*In Vivo* LNP Liver-targeting Programs

- Beam plans to present updated preclinical data from its BEAM-301 program at the American Society of Cell and Gene Therapy (ASGCT) meeting, demonstrating high and durable editing efficiency in a mouse model of glycogen storage disease 1a (GSDIa) out to 35 weeks. Beam plans to initiate IND-enabling studies in 2022 for BEAM-301, a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation, the most common disease-causing mutation of GSDIa.
- Also at ASGCT, Beam will present 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 continues to anticipate the nomination of a second liver-targeted development candidate in 2022.

**Initiation of AUNT Natural History Study**

SCD is a severe, inherited blood disorder that alters the structure and function of oxygen-carrying hemoglobin in red blood cells and is caused by a single point mutation in the beta globin gene. Carriers of the disease, or individuals with sickle cell trait, have only one copy of the sickle mutation and produce variable amounts of the abnormal sickle hemoglobin (25-45% of total hemoglobin). Despite the high prevalence of sickle cell trait (estimated to affect 300 million individuals worldwide), research to understand its full biology and clinical features has been limited.

Beam is developing two programs for SCD, BEAM-101 and BEAM-102. BEAM-101 is designed to raise fetal hemoglobin while lowering abnormal sickle hemoglobin to <40% of total, which is similar to levels seen in individuals with sickle cell trait. BEAM-102 is designed to replace the sickle mutation with a normal human variant of hemoglobin, HbG-Makassar, potentially reducing even further the abnormal sickle hemoglobin in patient cells. A better understanding of sickle cell trait, along with an in-depth understanding of SCD, will help better establish the relationship between levels of the abnormal sickle hemoglobin and long-term clinical outcomes.

The AUNT Study will create a first of its kind multi-center, prospective, longitudinal cohort of individuals with sickle trait, targeting a large enrollment of approximately 1,000 participants. This research is designed to establish an understanding of the hematologic and clinical phenotype of people with sickle cell trait, including blood rheology, potential complications, and genetic modifiers, in an effort to better understand the hematologic phenotype that is associated with good health and lack of organ dysfunction, as well as provide increased counseling to people with sickle cell trait.

**Business Updates**

- Manmohan Singh, Ph.D., senior vice president, pharmaceutical sciences & delivery technologies, was appointed to the company's executive leadership team. Dr. Singh joined Beam in 2018, bringing more than 24 years of drug discovery and development experience from Takeda Pharmaceuticals, Novartis and Chiron Corporation.
- Anne Marie Woodland joined Beam as senior vice president, regulatory affairs, bringing more than 20 years of experience to Beam, previously serving in regulatory focused roles at Replimune, UniQure and BioVex.

**Upcoming ASGCT Presentation Details**
**Title:** *Single, systemic administration of BEAM-301 mitigates fasting hypoglycemia and restores metabolic function in a transgenic mouse model of glycogen storage disease type Ia*
**Date & Time:** Monday, May 16, 2022, from 4:15-4:30 p.m. ET

**Title:** *Optimized base editing reagents yield more potent genetic correction in a mouse model of alpha-1 antitrypsin deficiency* (poster M-123)
**Date & Time:** Monday May 16, 2022, from 5:30-6:30 p.m. ET

**Title:** *Efficient LNP delivery of mRNA in vivo and in vitro to T and NK cells* (poster Tu-107)
**Date & Time:** Tuesday May 17, 2022, from 5:30-6:30 p.m. ET

**First Quarter 2022 Financial Results**

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.2 billion as of March 31, 2022, as compared to $965.6 million as of December 31, 2021.
- **Research & Development (R&D) Expenses:** R&D expenses were $65.4 million for the first quarter of 2022, compared to $190.1 million for the first quarter of 2021. R&D expenses for the first quarter of 2021 includes $155.0 million of expense related to in-process research and development acquired from Guide Therapeutics, Inc.
- **General & Administrative (G&A) Expenses:** G&A expenses were $19.2 million for the first quarter of 2022, compared to $10.3 million for the first quarter of 2021.
- **Net Loss:** Net loss was $69.2 million for the first quarter of 2022, or $1.01 per share, compared to $201.6 million for the first quarter of 2021, or $3.35 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: the intended design and planned initiation of the AUNT Study; our presentations at ASGCT, our plans, and anticipated timing, to nominate additional development candidates, initiate IND-enabling studies, and submit IND applications; the therapeutic applications and potential of our technology, including with respect to SCD, T-ALL/T-LL, GSDIa, Alpha-1, CAR-T cells and LNPs, including our ability to deliver base editors to target organs in and beyond the liver; the planned initiation and design of our BEACON-101 clinical trial, including the timing of enrolling the first subject in the trial; 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; 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.

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

| | March 31, 2022 | December 31, 2021 |
|---|---|---|
| Cash, cash equivalents, and marketable securities | $ 1,222,600 | $ 965,647 |
| Total assets | 1,453,402 | 1,474,453 |
| Total liabilities | 624,334 | 647,715 |
| Total stockholders’ equity | 829,068 | 826,738 |

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

| | Three Months Ended March 31, | |
|---|---|---|
| | 2022 | 2021 |
| License and collaboration revenue | $ 8,432 | $ 6 |
| Operating expenses: | | |
| Research and development | 65,410 | 190,106 |
| General and administrative | 19,247 | 10,273 |
| Total operating expenses | 84,657 | 200,379 |
| Loss from operations | (76,225) | (200,373) |
| Other income (expense): | | |
| Change in fair value of derivative liabilities | 13,600 | (1,900) |
| Change in fair value of non-controlling equity investments | (7,685) | 1,039 |
| Change in fair value of contingent consideration liabilities | 452 | (305) |
| Interest and other income (expense), net | 644 | (21) |
| Total other income (expense) | 7,011 | (1,187) |
| Net loss | $ (69,214) | $ (201,560) |
| Net loss per common share, basic and diluted | $ (1.01) | $ (3.35) |
| Weighted-average common shares outstanding, basic and diluted | 68,703,864 | 60,210,120 |