Beam Therapeutics Reports Pipeline Updates and First Quarter 2023 Financial Results

May 10, 2023

Focused on Executing Three Strategic Pillars – Hematology, Immunology/Oncology and Genetic Diseases – Potential for Long-Term Pipeline Growth and Sustained Impact on Patient Lives

Multiple Sites Activated and Additional Patients Enrolled in BEACON Clinical Trial of BEAM-101 for Patients with Sickle Cell Disease

Twenty-Year Industry Veteran, Gopi Shanker, Ph.D., Appointed as Chief Scientific Officer

Well-Capitalized with $1.1 Billion in Cash, Cash Equivalents and Marketable Securities at the End of the First Quarter of 2023

CAMBRIDGE, Mass., May 10, 2023 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported first quarter 2023 financial results and provided an update on its BEACON clinical trial and pipeline progress.

"As we work through the sentinel cohort process with our investigators, we are encouraged by the overall momentum of the BEACON trial, with multiple new sites activated, a growing number of patients on the wait list for future enrollment, and continuing progress preparing for internal GMP manufacturing in our North Carolina facility," said John Evans, chief executive officer of Beam. "In parallel to BEACON, we are preparing to initiate our second clinical trial this year, evaluating BEAM-201 in patients with a devastating form of blood cancer, while advancing our *in vivo* programs, BEAM-301 and BEAM-302, all of which remain on track. As leaders in the field of base editing, we have spent the last several years building a deep platform and pipeline and securing meaningful funding to support this ambitious set of goals. We plan to continue to follow the science, maintain a disciplined investment strategy, and preserve optionality in our portfolio, so that we can maximize our potential to create medicines that provide life-long cures for patients in need."

Hematology Program Updates

- Beam continues to advance its BEACON Phase 1/2 clinical trial evaluating BEAM-101 as a treatment for sickle cell disease (SCD), with five sites now active and a wait list for enrollment.
- In the sentinel cohort, which is designed to include three patients treated on a sequential basis, the first patient enrolled in the BEACON trial in late 2022 has been withdrawn from the study by the investigator due to personal, non-medical reasons prior to treatment with BEAM-101.
    - Two additional patients have been enrolled in the sentinel cohort and are undergoing the screening procedures required to enable mobilization and ultimately treatment with BEAM-101.
    - Beam expects to fully enroll the sentinel cohort in 2023.
- Enrollment of additional patients for the expansion cohort, which can occur in parallel with the sentinel cohort, is also anticipated to begin this year.
- The company continues to anticipate reporting initial data on multiple patients from the BEACON trial in 2024.

Immunology/Oncology Program Updates

- Beam is advancing 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 company expects to begin enrollment and dose the first patient in its Phase 1/2 clinical trial of BEAM-201 by mid-2023.
- Beam also continues to invest in and advance its next-generation allogeneic strategies designed to improve cell persistence and expand the utility and accessibility of cell therapies in cancer and other diseases. The company plans to share updates on these efforts in 2023.

Genetic Disease (*in vivo*) Program Updates

- Beam is advancing two *in vivo* base editing product candidates, BEAM-301 and BEAM-302, leveraging lipid nanoparticles (LNPs) for delivery to the liver. The company expects to:
    - Submit a regulatory application for authorization to initiate clinical trials for BEAM-301 for the treatment of glycogen storage disease 1a (GSD1a) by late 2023 or early 2024; and
    - Submit a regulatory application for authorization to initiate clinical trials for BEAM-302 for the treatment of alpha-1 antitrypsin deficiency (AATD) in early 2024.
- In 2023, Beam plans to continue advancement of multiple additional *in vivo* liver editing programs, including both its wholly owned and collaboration programs, through lead optimization, and advance its LNP delivery technologies for delivery of base editing medicines to the liver and other tissues.

Manufacturing Updates

- Beam expects to initiate current good manufacturing practice compliant operations at its North Carolina manufacturing facility in late 2023.

Leadership Addition

- In March 2023, Beam appointed Gopi Shanker, Ph.D., as chief scientific officer. Dr. Shanker is a scientific leader with more than 20 years of drug development experience as well as deep expertise in novel genetic medicine modalities.

Upcoming Presentations at ASGCT
Beam plans to present new *in vivo* data from its Engineered Stem Cell Antibody Paired Evasion (ESCAPE) platform, which it continues to advance in an effort to enable utility of its base editing investigational medicines in as many patients as possible, and from its base editing program for the treatment of Stargardt disease at the American Society of Gene and Cell Therapy 26th Annual Meeting. The meeting is being held May 16-20, 2023, in Los Angeles.

Details of the presentations are as follows:

Title: Paired HSC Epitope Engineering of CD117 (Ckit) for Antibody-Mediated Autologous Hematopoietic Stem Cell Therapy Conditioning for the Potential Treatment of Hemoglobinopathies
Date & Time: Friday, May 19, 2023 12:00 pm – 2:00 pm PT
Session Title: Friday Poster Session

Title: (308) In Vivo Genetic Eye Disease Correction Using Split AAV-Mediated Adenine Base Editing
Date & Time: Friday May 19, 2023, 4:30 pm - 4:45 pm PT
Session Title: Gene Targeting and Gene Correction: Hemoglobin, Muscle, and Eye

First Quarter 2023 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.1 billion as of March 31, 2023, as compared to $1.1 billion as of December 31, 2022.
- **Research & Development (R&D) Expenses:** R&D expenses were $99.6 million for the first quarter of 2023, compared to $65.4 million for the first quarter of 2022.
- **General & Administrative (G&A) Expenses:** G&A expenses were $23.5 million for the first quarter of 2023, compared to $19.2 million for the first quarter of 2022.
- **Net Loss:** Net loss was $96.5 million for the first quarter of 2023, or $1.33 per share, compared to $69.2 million for the first quarter of 2022, or $1.01 per share.

Cash Runway
Beam expects that its cash, cash equivalents and marketable securities as of March 31, 2023, will enable the company to fund its anticipated operating expenses and capital expenditure requirements at least into 2025. This expectation 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: the therapeutic applications and potential of our technology, including with respect to SCD, GSDIa, T-ALL/TLL, 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 March 31, 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; our anticipated timing for initiating current good manufacturing practice compliant operations at our North Carolina manufacturing facility; our upcoming presentations; 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 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, 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.

Contacts:

Investors:
Chelcie Lister
THRUST Strategic Communications
chelcie@thrustsc.com

Media:
Dan Budwick
1AB
dan@1abmedia.com

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

	March 31, 2023	December 31, 2022
Cash, cash equivalents, and marketable securities	$ 1,059,471	$ 1,078,134
Total assets	1,332,352	1,341,714
Total liabilities	570,846	608,240
Total stockholders' equity	761,506	733,474

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

	Three Months Ended March 31,	
	2023	2022
License and collaboration revenue	$ 24,208	$ 8,432
Operating expenses:		
Research and development	99,646	65,410
General and administrative	23,490	19,247
Total operating expenses	123,136	84,657
Loss from operations	(98,928)	(76,225)
Other income (expense):		
Change in fair value of derivative liabilities	5,600	13,600
Change in fair value of non-controlling equity investments	(12,797)	(7,685)
Change in fair value of contingent consideration liabilities	(296)	452
Interest and other income (expense), net	9,961	644
Total other income (expense)	2,468	7,011
Net loss	$ (96,460)	$ (69,214)
Unrealized gain (loss) on marketable securities	1,665	(2,659)
Comprehensive loss	$ (94,795)	$ (71,873)
Net loss per common share, basic and diluted	$ (1.33)	$ (1.01)
Weighted-average common shares outstanding, basic and diluted	72,273,829	68,703,864