Beam Therapeutics Reports Pipeline Updates and Second Quarter 2023 Financial Results

August 8, 2023

BEACON Trial of BEAM-101 in Sickle Cell Disease Progressing with Consented Patients Projected to Fill Sentinel Cohort and to Initiate Expansion Cohort

First Patient Consented in Phase 1/2 Trial of BEAM-201 in T-ALL/T-LL and Expected to be Dosed in The Third Quarter of 2023

Company to Accelerate Development of BEAM-302 for Treatment of AATD; Program Now Expected to be First In Vivo Liver Regulatory Filing in First Quarter of 2024

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

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

“The first half of 2023 has been marked by focused execution across the business, with the singular goal of making an impact on the lives of people suffering from serious diseases,” said John Evans, chief executive officer of Beam. “We are very pleased with the continued enrollment progress in the BEACON trial, having now consented enough patients projected to both fill the sentinel cohort and initiate the expansion cohort. In addition, the BEAM-201 trial is now open for enrollment at multiple clinical sites, with the first patient having been consented and dosing expected this quarter. We have also continued to accelerate development of BEAM-302, a potential best-in-class product candidate for patients with alpha-1 anti-trypsin deficiency, and are now prioritizing a BEAM-302 regulatory filing in the first quarter of 2024 as our first *in vivo* program, with a regulatory filing for BEAM-301 expected to follow shortly thereafter. Our critical manufacturing capability in North Carolina is anticipated to be cGMP ready for both cell manufacturing and LNP manufacturing this year. Finally, Beam is well capitalized to pursue the next wave of growth in its innovative research platform, from non-genotoxic conditioning with ESCAPE in hematology, to next-generation allogeneic cell therapies in cancer and immunology, to a growing number of wholly owned and partnered base editing programs targeting the liver. We believe we are well positioned to establish an industry-leading platform in precision genetic medicine.”

Second Quarter 2023 Business Updates and Key 2023-2024 Anticipated Milestones

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).
    - Beam has continued to consent additional patients in the BEACON trial, all of whom are now moving in parallel through the screening, transfusion and mobilization activities required to enable treatment with BEAM-101.
    - Beam now anticipates that currently consented patients are sufficient to both fill the sentinel cohort (n=3) and to initiate the expansion cohort. 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.
    - 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.
    - The company continues to anticipate 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.

Immunology/Oncology Portfolio

- Beam continues to advance 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). Multiple sites for the Phase 1/2 clinical trial of BEAM-201 are now open for enrollment.
- The first patient has consented and is expected to be dosed in the third quarter of 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 by year-end 2023.

Genetic Disease (in vivo) Portfolio

- Beam continues to advance its two *in vivo* base editing product candidates, BEAM-301 for the treatment of glycogen

storage disease 1a (GSD1a) and BEAM-302 for the treatment of alpha-1 antitrypsin deficiency (AATD), leveraging lipid nanoparticles (LNPs) for delivery to the liver.

- To promote speed to the clinic of a top priority program, the company has leveraged the learnings and capability build from BEAM-301 to accelerate development of BEAM-302, which is now expected to be its first *in vivo* liver regulatory filing, followed by BEAM-301.
- The company expects to:
  - Submit a regulatory application for authorization to initiate clinical trials for BEAM-302 in the first quarter of 2024; and
  - Submit a regulatory application for authorization to initiate clinical trials for BEAM-301 in the first half of 2024.
- Beam continues to advance multiple additional *in vivo* editing programs targeting the liver, 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 continues to expect initiation of current good manufacturing practice compliant operations at its North Carolina manufacturing facility in late 2023.
- Beam is now planning to enable cGMP manufacturing of both autologous cell products in support of its sickle cell programs as well as LNP products in support of its liver programs BEAM-302 and BEAM-301 in its North Carolina facility.

Recent Nature Genetics Preclinical Publication Suggests Base Editing Enables More Uniform HbF Upregulation than Nuclease Editing

- In July, Beam co-founder David Liu, Ph.D., and St. Jude Children's Research Hospital collaborators Jonathan Yen, Ph.D., and Mitchell Weiss, M.D., Ph.D., published preclinical research comparing five gene editing strategies in CD34+ hematopoietic stem and progenitor cells using either Cas9 nuclease or adenine base editors to induce fetal hemoglobin (HbF) red blood cells. Notably, the data suggest that base editing can provide a strategy for potent, uniform induction of HbF, yielding a consistent, predictable, and precise editing outcome. Conversely, nuclease editing of either the fetal hemoglobin gene or the BCL11A enhancer created a complex, uncontrolled distribution of alleles with a wide range of outcomes for induction of fetal hemoglobin, including numerous cells with minimal or no induction detected. These data illustrate the potential advantages of base editing's mechanism of action, including the creation of predictable and consistent gene modifications with well-characterized, uniform biological effects across edited cells, as compared to the uncontrolled mixture of allele outcomes that result from nuclease-based knockout through double-stranded breaks.

Second Quarter 2023 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.1 billion as of June 30, 2023, as compared to $1.1 billion as of December 31, 2022.
- **Research & Development (R&D) Expenses:** R&D expenses were $97.6 million for the second quarter of 2023, compared to $74.6 million for the second quarter of 2022.
- **General & Administrative (G&A) Expenses:** G&A expenses were $24.7 million for the second quarter of 2023, compared to $24.1 million for the second quarter of 2022.
- **Net Loss:** Net loss was $82.8 million for the second quarter of 2023, or $1.08 per share, compared to $72.0 million for the second quarter of 2022, or $1.02 per share.

Cash Runway

Beam expects that its cash, cash equivalents and marketable securities as of June 30, 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 June 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; our anticipated timing for initiating current good manufacturing practice compliant operations at our North Carolina manufacturing facility; 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, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, our Quarterly Report on Form 10-Q that we will file for the quarter ended June 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)

	June 30, 2023	December 31, 2022
Cash, cash equivalents, and marketable securities	$ 1,073,016	$ 1,078,134
Total assets	1,353,887	1,341,714
Total liabilities	542,244	608,240
Total stockholders’ equity	811,643	733,474

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
License and collaboration revenue	$ 20,116	$ 16,652	$ 44,324	$ 25,084
Operating expenses:				
Research and development	97,608	74,556	197,254	139,966
General and administrative	24,656	24,062	48,146	43,309
Total operating expenses	122,264	98,618	245,400	183,275
Loss from operations	(102,148)	(81,966)	(201,076)	(158,191)
Other income (expense):				
Change in fair value of derivative liabilities	(900)	12,200	4,700	25,800
Change in fair value of non-controlling equity investments	6,148	(4,124)	(6,649)	(11,809)
Change in fair value of contingent consideration liabilities	2,171	(120)	1,875	332
Interest and other income (expense), net	11,953	2,060	21,914	2,704
Total other income (expense)	19,372	10,016	21,840	17,027
Net loss	$ (82,776)	$ (71,950)	$ (179,236)	$ (141,164)
Unrealized gain (loss) on marketable securities	(1,250)	(1,481)	415	(4,140)
Comprehensive loss	$ (84,026)	$ (73,431)	$ (178,821)	$ (145,304)
Net loss per common share, basic and diluted	$ (1.08)	$ (1.02)	$ (2.41)	$ (2.03)

Weighted-average common shares outstanding, basic and diluted	76,335,175	70,210,227	74,315,721	69,461,207