Beam Therapeutics Reports Fourth Quarter and Year-End 2023 Financial Results and Reiterates Anticipated Milestones

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Patient Dosing and Enrollment Continue to Progress in Beacon Phase 1/2 Study of BEAM-101 in Severe Sickle Cell Disease; First Clinical Data Anticipated in Second Half of 2024

Phase 1 Trial Initiation for BEAM-302 in Alpha-1 Antitrypsin Deficiency on Track for First Half of 2024 Pending European Clinical Trial Application (CTA) Acceptance

Ended Fourth Quarter 2023 with $1.2 Billion in Cash, Cash Equivalents and Marketable Securities; Cash Runway Expected to Support Operating Plans into 2027

CAMBRIDGE, Mass., Feb. 27, 2024 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported fourth quarter and full year 2023 financial results and reiterated anticipated milestones across the company's sickle cell disease and genetic disease portfolios.

"This year has the potential to be transformative for Beam as we work to advance multiple base editing programs in the clinic, anchoring key high-value franchises through near-term catalysts – all backed by a strong balance sheet," said John Evans, chief executive officer of Beam. "In 2024, we expect to initiate our first *in vivo* clinical studies and report the first in-human data from our *ex vivo* base editing clinical programs. Operationally, we've made significant progress in the BEACON study of BEAM-101 in sickle cell disease, and we're on track to complete the sentinel cohort and initiate dosing in the expansion cohort in the first half of the year. In addition, we filed our CTA for BEAM-302 in alpha-1 anti-trypsin deficiency ahead of schedule and remain on track to initiate that study in the first half of 2024. We are convinced that base editing has the potential to provide new options and meaningful advantages over existing therapies for patients suffering from serious diseases."

Key 2024 Anticipated Milestones

Sickle Cell Disease (SCD) Franchise

- In the BEACON Phase 1/2 clinical trial of BEAM-101 in adults with severe SCD, Beam anticipates completing dosing in patients in the sentinel cohort and initiating dosing in patients in the expansion cohort in the first half of 2024.
- The company is on track to report initial data on multiple patients from the BEACON trial in the second half of 2024.
- Beam continues to advance and invest in its Engineered Stem Cell Antibody Paired Evasion (ESCAPE) conditioning platform and anticipates initiating Phase 1-enabling preclinical studies for the program in 2024.

Genetic Disease Franchise

- Beam has filed a European clinical trial application (CTA) for BEAM-302, the company's priority genetic disease program for the treatment for alpha-1 antitrypsin deficiency (AATD), and, assuming CTA acceptance, plans to initiate a Phase 1 clinical trial for BEAM-302 in the first half of 2024.
- Beam expects to submit an investigational new drug (IND) application in the U.S. for BEAM-301 for the potential treatment of glycogen storage disease type 1a (GSD1a) in the first half of 2024.

Fourth Quarter and Full Year 2023 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.2 billion as of December 31, 2023, compared to $1.1 billion as of December 31, 2022.
- **Research & Development (R&D) Expenses:** R&D expenses were $140.1 million for the fourth quarter of 2023 and $437.4 million for the full year ended December 31, 2023, compared to $86.3 million for the fourth quarter of 2022 and $311.6 million for the full year ended December 31, 2022.
- **General & Administrative (G&A) Expenses:** G&A expenses were $43.3 million for the fourth quarter of 2023 and $116.8 million for the full year ended December 31, 2023, compared to $22.7 million for the fourth quarter of 2022 and $87.8 million for the full year ended December 31, 2022.
- **Net Income (Loss):** Net income attributable to common stockholders was $142.8 million, or $1.77 per basic share and $1.73 per diluted share, for the fourth quarter of 2023 and net loss attributable to common stockholders was $132.5 million, or $1.72 per share, for the year ended December 31, 2023, compared to net losses of $38.3 million, or $0.54 per share, for the fourth quarter of 2022 and $289.1 million, or $4.13 per share, for the full year ended December 31, 2022.

Cash Runway

Beam expects that its cash, cash equivalents and marketable securities as of December 31, 2023, will enable the company to fund its anticipated

operating expenses and capital expenditure requirements into 2027. This expectation includes funding directed toward reaching each of the key anticipated milestones for BEAM-101, ESCAPE, 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 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 expectations for transitioning to a multi-program clinical stage company; the therapeutic applications and potential of our technology, including with respect to SCD, AATD, GSD1a, and ESCAPE; our plans, and anticipated timing, to advance our programs, the clinical trial designs and expectations for BEAM-101, BEAM-301, BEAM-302 and ESCAPE; our estimated cash, cash equivalents and marketable securities as of December 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; 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 our portfolio prioritization and strategic restructuring; 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 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; whether our actual audited results will be consistent with our estimated cash, cash equivalents and marketable securities as of December 31, 2023; 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, 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.

Contacts:

Investors:
Holly Manning
THRUST Strategic Communications
holly@thrustsc.com

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

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

	December 31, 2023	December 31, 2022
Cash, cash equivalents, and marketable securities	$ 1,189,876	$ 1,078,134
Total assets	1,459,714	1,341,714
Total liabilities	478,385	608,240
Total stockholders' equity	981,329	733,474

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

	Three Months Ended December 31,		Years Ended December 31,	
	2023	2022	2023	2022
License and collaboration revenue	$ 316,192	$ 20,037	$ 377,709	$ 60,920

Operating expenses:				
Research and development	140,077	86,341	437,381	311,594
General and administrative	43,257	22,681	116,813	87,805
Total operating expenses	183,334	109,022	554,194	399,399
Income (loss) from operations	132,858	(88,985)	(176,485)	(338,479)
Other income (expense):				
Change in fair value of derivative liabilities	(1,900)	3,000	7,500	23,900
Change in fair value of non-controlling equity investments	(722)	21,578	(18,592)	20,200
Change in fair value of contingent consideration liabilities	1,863	19,447	9,740	18,904
Interest and other income (expense), net	12,064	7,611	46,676	15,297
Total other income (expense)	11,305	51,636	45,324	78,301
Net income (loss) before income taxes	144,163	(37,349)	(131,161)	(260,178)
Provision for income taxes	(1,366)	(1,000)	(1,366)	(3,410)
Loss from equity method investment	—	—	—	(25,500)
Net income (loss)	$ 142,797	$ (38,349)	$ (132,527)	$ (289,088)
Unrealized gain (loss) on marketable securities	2,628	2,244	3,034	(2,380)
Comprehensive income (loss)	$ 145,425	$ (36,105)	$ (129,493)	$ (291,468)
Net income (loss) per common share - basic	$ 1.77	$ (0.54)	$ (1.72)	$ (4.13)
Basic weighted-average common shares outstanding	80,858,517	70,777,452	77,151,771	70,015,305
Net income (loss) per common share - diluted	$ 1.73	$ (0.54)	$ (1.72)	$ (4.13)
Diluted weighted-average common shares outstanding	82,702,302	70,777,452	77,151,771	70,015,305