Beam Therapeutics Reports Pipeline and Business Highlights, Planned 2022 Milestones and Fourth Quarter and Full Year 2021 Financial Results

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Research Collaboration with Pfizer Underway, with Upfront and Potential Milestone Payments of Up to $1.35 Billion

Executing First Wave of Long-term Strategy for Sickle Cell Disease with Planned Initiation of BEAM-101 Clinical Trial and IND Submission for BEAM-102 in the Second Half of 2022

Strong Cash Position of $1.2 Billion, Including Pfizer Upfront, to Support Robust Set of Milestones Across Ex Vivo and In Vivo Base Editing Programs Anticipated in 2022 and Beyond

CAMBRIDGE, Mass., Feb. 28, 2022 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced pipeline and business highlights, outlined key 2022 anticipated milestones and reported fourth quarter and full year 2021 financial results.

"Throughout 2021, we made important advancements across our platform and portfolio, culminating in the clearance of our first IND submission late last year and our recently launched collaboration with Pfizer, both of which provide further validation for the potential of our base editing and delivery technologies," said John Evans, chief executive officer of Beam. "In 2022, we plan to continue this momentum by executing the first wave of our long-term strategy for sickle cell disease with the planned initiation of our trial with BEAM-101, marking our transition to a clinical-stage company, and our anticipated IND submission for BEAM-102. There is a significant need for novel treatments for sickle cell disease and other severe genetic blood disorders, and we believe that our strategy and suite of technologies – base editing, improved conditioning and *in vivo* delivery for editing HSCs – has the potential to make an important impact on the treatment landscape for these patients."

Mr. Evans continued, "In parallel, we plan to actively advance the remainder of our pipeline in 2022, notably with expected milestones including an IND submission for BEAM-201 for the treatment of relapsed and refractory acute T-cell leukemia and lymphoblastic lymphoma, IND-enabling work for BEAM-301 for the treatment of glycogen storage disease Ia, the naming of additional development candidates from our pipeline, and continued advancement of our comprehensive technology platform in precision genetic medicine. This is an exciting time for Beam, and I'm optimistic about the year ahead as we work to bring potentially life-changing medicines to patients."

Pipeline and Business Highlights

- **Executed Multi-Target Research Collaboration with Pfizer to Advance Novel *In Vivo* Base Editing Programs for a Range of Rare Diseases:** Beam and Pfizer entered into a four-year research collaboration focused on *in vivo* base editing programs for three targets for rare genetic diseases of the liver, muscle and central nervous system. Under the terms of the agreement, Beam received an upfront payment of $300 million and, assuming Pfizer exercises its opt-in license rights for all three targets, is eligible for development, regulatory and commercial milestone payments for potential total deal consideration of up to $1.35 billion. Beam is also eligible to receive royalties on global net sales for each licensed program. Beam will conduct all research activities through development candidate selection, and Pfizer may opt in to exclusive, worldwide licenses to each development candidate, after which it will be responsible for all development activities, as well as potential regulatory approvals and commercialization, for each such candidate. Beam has a right to opt in, at the end of Phase 1/2 studies, upon the payment of an option exercise fee, to a global co-development and co-commercialization agreement with respect to one program licensed under the collaboration pursuant to which Pfizer and Beam would share net profits, as well as development and commercialization costs in a 65%/35% ratio (Pfizer/Beam). The collaboration has an initial term of four years and may be extended up to one additional year.

- **Outlined Long-term Strategy for Base Editing Programs in Sickle Cell Disease at ASH 2021:** At the 63rd American Society for Hematology Annual Meeting & Exposition in December 2021, Beam shared a long-term, staged development strategy for its base editing approach to treat sickle cell disease (SCD). Beam's stepwise strategy involves three waves:
  - **Wave 1: *Ex Vivo* Base Editing via Autologous Transplant**
    - Beam is advancing *ex vivo* base editing programs, in which cells will be collected from a patient, edited and then infused back into the patient following a conditioning regimen, such as treatment with busulfan, the standard of care in hematopoietic stem cell (HSC) transplantation today. This approach will be deployed in the company's BEAM-101 and BEAM-102 base editing programs and is intended to allow the company to pursue an efficient path for development using increasingly validated clinical endpoints and regulatory strategies.
  - **Wave 2: Improved Conditioning**
    - In parallel with Wave 1 development, Beam also aims to improve the transplant conditioning regimen for SCD patients undergoing HSC transplantation, reducing toxicity challenges associated with standard of care

conditioning, a critical component necessary to prepare a patient's body for effective treatment. Beam has a collaboration with Magenta Therapeutics to evaluate the potential utility of MGTA-117, Magenta's novel antibody drug conjugate that is designed to spare immune cells and precisely target hematopoietic stem and progenitor cells. Beam is also conducting its own research into novel conditioning strategies. If successful, improved conditioning regimens could potentially be paired with BEAM-101 and BEAM-102, as well as other base editing programs in hematology.

- **Wave 3: *In Vivo* Base Editing via HSC-targeted LNPs**
  - Beam is also exploring the potential for *in vivo* base editing programs for SCD, in which base editors would be delivered to the patient through an infusion of lipid nanoparticles (LNPs) targeted to HSCs, eliminating the need for transplantation altogether. This approach could provide a more accessible option for patients, particularly in regions where *ex vivo* treatment is challenging. Building on its acquisition of Guide Therapeutics, Beam is using a DNA-barcoded LNP screening technology to enable high-throughput *in vivo* identification of LNPs with novel biodistribution and selectivity for target organs beyond the liver.

- **Presented Preclinical Data Highlighting Approach to Creating Multiplex Edited CAR T-cells to Target CD5-positive Tumors at SITC:** At the Society for Immunotherapy of Cancer's 36th Annual Meeting in November 2021, Beam announced new preclinical research demonstrating the ability of the company's multiplex edited CAR T-cells to target CD5-positive tumors, leading to tumor clearance *in vivo*. Beam's process utilizes base editing designed to simultaneously silence five target genes, including CD5 and PD1, to create allogeneic anti-CD5 CAR T-cells with enhanced effector function for potential use as off-the-shelf treatments for T-cell malignancies.

Key 2022 Anticipated Milestones

Ex Vivo HSC Programs

- Enroll the first subject in the Phase 1/2 clinical trial of BEAM-101 for the treatment of SCD, which is referred to as the BEACON-101 trial, in the second half of 2022
- 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

- Submit an IND application for BEAM-201 for the treatment of relapsed/refractory T cell acute lymphoblastic leukemia/T cell lymphoblastic lymphoma (T-ALL/TLL) in the second half of 2022
- Nominate a second CAR T development candidate in 2022

In Vivo LNP Liver-targeting Programs

- Initiate IND-enabling studies 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 glycogen storage disorder Ia (GSDIa), in 2022
- Nominate a second liver-targeted development candidate in 2022

Fourth Quarter and Full Year 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $965.6 million as of December 31, 2021 (which does not include the upfront payment from the Pfizer collaboration), compared to $299.7 million as of December 31, 2020.
- **Research & Development (R&D) Expenses:** R&D expenses were $96.8 million for the fourth quarter of 2021 and $387.1 million for the full year ended December 31, 2021, compared to $32.5 million for the fourth quarter of 2020 and $103.2 million for the full year ended December 31, 2020.
- **General & Administrative (G&A) Expenses:** G&A expenses were $17.8 million for the fourth quarter of 2021 and $57.2 million for the full year ended December 31, 2021, compared to $8.4 million for the fourth quarter of 2020 and $29.6 million for the full year ended December 31, 2020.
- **Net Loss:** Net loss attributable to common stockholders was $64.7 million, or $0.95 per share, for the fourth quarter of 2021 and $370.6 million, or $5.77 per share, for the year ended December 31, 2021, compared to $95.5 million, or $1.69 per share, for the fourth quarter of 2020 and $195.9 million, or $4.19 per share, for the full year ended December 31, 2020.

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 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, and LNPs, including our ability to deliver base editors to target organs in and beyond the liver; our ability to develop improved conditioning regimens in connection with our long-term strategy for base editing programs in sickle cell disease; the planned initiation and design of our BEACON-101 clinical trial, including the timing of enrolling the first subject in the trial; the potential benefits of our collaboration with Pfizer, any future payments we may receive under our research collaboration agreement with Pfizer; 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; 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)

	December 31, 2021	December 31, 2020
Cash, cash equivalents, and marketable securities	$ 965,647	$ 299,671
Total assets	1,474,453	451,677
Total liabilities	647,715	206,116
Total stockholders’ equity	826,738	245,561

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

	Three Months Ended December 31,		Year Ended December 31,	
	2021	2020	2021	2020
License and collaboration revenue	$ 51,069	$ 6	$ 51,844	$ 24
Operating expenses:				
Research and development	96,781	32,451	387,087	103,179
General and administrative	17,772	8,354	57,222	29,605
Total operating expenses	114,553	40,805	444,309	132,784
Loss from operations	(63,484)	(40,799)	(392,465)	(132,760)
Other income (expense):				
Change in fair value of derivative liabilities	7,400	(54,700)	(1,000)	(63,400)
Change in fair value of non-controlling equity investments	(4,270)	-	17,690	517
Change in fair value of contingent consideration liabilities	(4,407)	-	5,146	-
Interest and other income (expense), net	54	35	(9)	1,051
Total other income (expense)	(1,223)	(54,665)	21,827	(61,832)
Net loss	$ (64,707)	$ (95,464)	$ (370,638)	$ (194,592)
Accretion of redeemable convertible preferred stock to redemption value, including dividends on preferred stock	-	-	-	(1,277)
Net loss attributable to common stockholders	$ (64,707)	$ (95,464)	$ (370,638)	$ (195,869)
Net loss per common share attributable to common stockholders, basic and diluted	$ (0.95)	$ (1.69)	$ (5.77)	$ (4.19)

Weighted-average common shares used in net loss per share attributable to common stockholders, basic and diluted	67,988,717	56,544,620	64,227,676	46,733,221