Beam Therapeutics Provides Business and Pipeline Updates and Reports Third Quarter 2021 Financial Results

November 8, 2021

BEAM-101 IND Cleared by FDA for Evaluation as a Treatment for Sickle Cell Disease

BEAM-102 IND-Enabling Studies Also Underway

CAMBRIDGE, Mass., Nov. 08, 2021 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported recent business and pipeline updates, as well as third quarter 2021 financial results.

As part of today's update, Beam announced that its Investigational New Drug (IND) application for BEAM-101 for the treatment of sickle cell disease was cleared by the U.S. Food and Drug Administration (FDA). BEAM-101, the company's lead *ex vivo* base editing product candidate, is a patient-specific, autologous hematopoietic investigational cell therapy which incorporates base edits that mimic single nucleotide polymorphisms seen in individuals with hereditary persistence of fetal hemoglobin (HPFH) to potentially alleviate the effects of mutations causing sickle cell disease or beta-thalassemia. This is the first open IND for base editing technology, a next-generation form of CRISPR capable of making single base changes without creating double strand breaks in the DNA. Beam is preparing to initiate a Phase 1/2 clinical trial designed to assess the safety and efficacy of BEAM-101 for the treatment of sickle cell disease, which Beam refers to as the BEACON-101 trial.

"We are thrilled to share that the FDA has cleared our first IND. BEAM-101 has the potential to offer a one-time treatment for patients with sickle cell disease, and this clearance enables the important transition from a preclinical to a clinical-stage company, bringing us closer to our ultimate goal of helping patients," said John Evans, chief executive officer of Beam. "As leaders in the field of base editing, this milestone underscores the expertise of our team and the significant potential of our technology. We are grateful to the team members who have dedicated countless hours to this effort, and we look forward to this program's continued evaluation in the clinic. As we look toward 2022, we believe we are well-positioned both financially and organizationally to execute on our vision."

"In addition to the clearance of our IND for BEAM-101, we are pleased to announce that we have initiated IND-enabling studies for BEAM-102, our Makassar base editing approach for the treatment of sickle cell disease," said Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam. "We have also made continued progress on BEAM-201, our multiplexed base editing approach for the treatment of relapsed/refractory T-cell acute lymphoblastic leukemia and T-cell lymphoblastic leukemia as well as continued advancement across our robust portfolio, as showcased by a number of recent and upcoming data presentations demonstrating our leadership in base editing as well as optimized delivery technologies for our programs. Importantly, we also expect to nominate our first development candidate for *in vivo* base editing in the liver using LNP delivery for the treatment of patients with glycogen storage disease type Ia (R83C mutation) by the end of the year. We've made extraordinary progress as a company this year and remain committed to advancing novel science so that we may reach as many patients as possible."

Upcoming Base Editing Data Presentations

- **Preclinical data highlighting potential of CAR T multiplex base editing approach targeting CD5 at 2021 SITC Annual Meeting.** At the Society for Immunotherapy of Cancer's (SITC) 36th Annual Meeting, Beam will present preclinical data from its multiplex edited allogeneic CAR T research targeting CD5-positive hematologic malignancies in a poster titled "CD5 knockout enhances the potency of multiplex base-edited allogeneic anti-CD5 CAR T-cell therapy for the treatment of T-cell malignancies."
- **Multiple abstracts accepted for presentation at the 63rd American Society of Hematology Annual Meeting & Exposition (ASH).** At this year's ASH meeting, Beam will present preclinical data highlighting its base editing approach to address sickle cell disease, including new preclinical data for BEAM-102, and data highlighting Beam's *in vivo* high-throughput lipid nanoparticle (LNP) screening approach to identify novel LNPs that can deliver base editors to tissues beyond the liver, such as hematopoietic stem cells. In addition, Beam will present an overview on the application of base editing for the treatment of beta-hemoglobinopathies and other genetic blood disorders during a scientific program session. Details of the presentations can be found here.

Recent Base Editing Progress & Data Updates

- **BEAM-102 IND-enabling studies initiated.** Beam has initiated IND-enabling studies for BEAM-102 for the treatment of sickle cell disease. BEAM-102 aims to directly correct the causative mutation in sickle cell disease by recreating a naturally-occurring normal human hemoglobin variant, HbG Makassar.
- **Preclinical data highlighting base editing approach to address GSDIa presented at European Society of Gene & Cell Therapy.** Beam recently presented preclinical data demonstrating the ability of its liver-targeted base editing approach to directly correct R83C, one of the primary disease-causing mutations of glycogen storage disease type Ia (GSDIa), and the elimination of the disease phenotype in a novel *in vivo* model.

- **Optimized LNP delivery approaches for *in vivo* base editing to the liver and other tissues presented at TIDES 2021.** Beam announced new preclinical data highlighting advancements with its approach to developing novel LNP formulations for increased *in vivo* liver editing potency, with high levels of editing at what Beam believes could be a clinically-relevant dose level of 1.0 mg/kg. Beam remains on track to nominate its first Development Candidate for *in vivo* base editing using LNP delivery by the end of 2021. Also at TIDES, Beam reported an update on its proprietary approach to developing LNPs to deliver base editors to tissues beyond the liver, including hematopoietic stem and progenitor cells.
- **Preclinical data highlighting potential of base editors to target disease drivers of chronic hepatitis B infection presented at 2021 International HBV Meeting.** Beam recently presented data demonstrating the potential of its cytosine base editors to reduce viral markers, including hepatitis B surface antigen (HBsAg) expression, and prevent viral rebound of hepatitis B virus (HBV) in *in vitro* models.

Business Highlights

- **Executed non-exclusive option and license agreement with Sana Biotechnology for Cas12b.** Under the agreement, Beam granted Sana non-exclusive commercial rights to utilize its CRISPR Cas12b system with certain allogeneic T-cell and stem cell-derived programs. The agreement excludes any rights to base editing using Beam's Cas12b system, which rights remain exclusively with Beam. Sana agreed to pay Beam an upfront payment of $50 million, and Beam may also receive certain target option exercise fees, certain milestone payments upon the achievement of certain development and commercial sale milestones, and certain royalties on net sales of royalty-bearing products by Sana.

Third Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $933.4 million as of September 30, 2021, compared to $299.7 million as of December 31, 2020.
- **Research & Development (R&D) Expenses:** R&D expenses were $54.6 million for the third quarter of 2021, compared to $29.8 million for the third quarter of 2020.
- **General & Administrative (G&A) Expenses:** G&A expenses were $15.8 million for the third quarter of 2021, compared to $7.5 million for the third quarter of 2020.
- **Net Loss:** Net loss attributable to common stockholders was $28.1 million, or $0.42 per share, for the third quarter of 2021, compared to $34.5 million, or $0.69 per share, for the third quarter of 2020.

About Sickle Cell Disease and Beta-Thalassemia

Sickle cell disease, a severe inherited blood disease, is caused by a single point mutation, E6V, in the beta globin gene. This mutation causes the mutated form of sickle hemoglobin (HbS) to aggregate into long, rigid molecules that bend red blood cells into a sickle shape under conditions of low oxygen. Sickled cells obstruct blood vessels and die prematurely, ultimately resulting in anemia, severe pain (crises), infections, stroke, organ failure, and early death. Sickle cell disease is the most common inherited blood disorder in the United States, affecting an estimated 100,000 individuals, of which a significant proportion are of African-American descent. Beta-thalassemia is another inherited blood disorder characterized by severe anemia caused by reduced production of functional hemoglobin due to insufficient expression of the beta globin protein. Transfusion-dependent beta-thalassemia (TDBT) is the most severe form of this disease, often requiring multiple transfusions per year. Patients with TDBT suffer from failure to thrive, persistent infections, and life-threatening anemia.

About BEAM-101
BEAM-101 is a patient-specific, autologous hematopoietic cell therapy under investigation for the treatment of sickle cell disease. BEAM-101 incorporates *ex vivo* base edits that mimic single nucleotide polymorphisms seen in individuals with hereditary persistence of fetal hemoglobin (HPFH) to potentially alleviate the effects of mutations causing sickle cell disease or beta-thalassemia by increasing the levels of fetal hemoglobin (HbF).

About Beam Therapeutics
Beam Therapeutics Inc. (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 enables precise, predictable and efficient single base changes, at targeted genomic sequences, without making double-stranded breaks in the DNA. This enables 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 potentially 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 commencement of clinical trials for BEAM-101, including our BEACON-101 trial; our expectation that we will nominate our first development candidate for *in vivo* base editing in the liver using LNP delivery for the treatment of patients with GSDIa disease (R83C mutation) by the end of the year; any future payments we may receive under our agreement with Sana; our planned base editing data presentations at upcoming scientific conferences; and the therapeutic applications and potential of our technology, including 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, 2020, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2021, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, our Quarterly Report on Form 10-Q that we will file for the quarter ended September 30, 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)

	September 30, 2021	December 31, 2020
Cash, cash equivalents, and marketable securities	$ 933,405	$ 299,671
Total assets	1,156,555	451,677
Total liabilities	302,741	206,116
Total stockholders’ equity	853,814	245,561

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

	Three Months Ended September 30,		Six Months Ended September 30,	
	2021	2020	2021	2020
License revenue	$ 763	$ 6	$ 775	$ 18
Operating expenses:				
Research and development	54,623	29,825	290,306	70,728
General and administrative	15,774	7,502	39,450	21,251
Total operating expenses	70,397	37,327	329,756	91,979
Loss from operations	(69,634)	(37,321)	(328,981)	(91,961)
Other income (expense):				
Change in fair value of derivative liabilities	35,800	2,700	(8,400)	(8,700)
Change in fair value of long-term investments	(4,892)	-	21,960	517
Change in fair value of contingent consideration liabilities	10,599	-	9,553	-
Interest and other income (expense), net	9	169	(63)	1,016
Total other income (expense)	41,516	2,869	23,050	(7,167)
Net loss	$ (28,118)	$ (34,452)	$ (305,931)	$ (99,128)
Accretion of redeemable convertible preferred stock to redemption value, including dividends on preferred stock	-	-	-	(1,277)
Net loss attributable to common stockholders	$ (28,118)	$ (34,452)	$ (305,931)	$ (100,405)
Net loss per common share attributable to common stockholders, basic and diluted	$ (0.42)	$ (0.69)	$ (4.86)	$ (2.31)
Weighted-average common shares used in net loss per share attributable to common stockholders, basic and diluted	66,377,611	50,087,747	62,960,219	43,438,919