Beam Therapeutics Announces First Development Candidates for Sickle Cell Disease and Reports Second Quarter 2020 Results

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BEAM-101 and BEAM-102 Named as Development Candidates Targeting Distinct Approaches to Treating Sickle Cell Disease

Lease Agreement Signed to Build In-house Manufacturing Facility Dedicated to Producing Base Editing Therapeutics

Broad Partnering Strategy Continues to Advance and Expand the Therapeutic Application of Novel Base Editing Technologies

CAMBRIDGE, Mass., Aug. 12, 2020 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported pipeline updates, recent business highlights and second quarter 2020 financial results.

"Throughout the first half of 2020, we made significant progress across all aspects of our business, culminating into naming the first two base editing development candidates from our portfolio. As we move closer to the clinic, we have also made the important strategic decision to establish a build-to-suit manufacturing facility, which will significantly enhance our capability to manufacture a wide range of base editing medicines," said John Evans, chief executive officer of Beam. "In addition, we continue to execute our strategy of establishing innovative partnerships to access new capabilities and to accelerate the development of base editors as a new class of precision genetic medicines for patients. Amidst the evolving COVID-19 situation, our team is performing well, and we remain on track to initiate IND-enabling studies in 2020 and file at least one Investigational New Drug application in 2021."

Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam added, "Achieving our first development candidates with base editing is one of the most important milestones for our company yet. BEAM-101 and BEAM-102 are highly differentiated editing programs that may enable a one-time treatment option for patients with sickle cell disease. Both candidates are supported by promising preclinical data, and we are working to advance them to the next stage of development to assess the impact they could have in treating this devastating disease."

Base Editing Progress

- **First Base Editing Development Candidates Named for Treatment of Sickle Cell Disease:** Beam is pursuing two differentiated base editing approaches to treat hemoglobinopathies and recently named the first two development candidates from its portfolio. Promising preclinical data from these two complementary editing programs were presented at the 23rd American Society of Gene and Cell Therapy (ASGCT) Virtual Annual Meeting.
  - BEAM-101 is an adenine base editor (ABE) that reproduces single base changes observed in individuals with hereditary persistence of fetal hemoglobin, or HPFH, in which elevated levels of fetal hemoglobin protect these individuals from the effects of sickle cell disease or beta-thalassemia.
  - BEAM-102 is an ABE that directly corrects the causative mutation in sickle cell disease, converting it into a naturally-occurring human hemoglobin variant, Hb-G Makassar. Individuals with the Makassar variant have normal hematologic parameters and no evidence of hemoglobin polymerization or sickling of red blood cells.

- **Preclinical Non-Human Primate Data Validating Beam's ABE Technology Presented by Verve Therapeutics:** Verve Therapeutics presented preclinical proof-of-concept data in non-human primates, utilizing ABE technology licensed from Beam, that demonstrate the successful use of base editing to turn off a gene in the liver and thereby lower blood levels of either LDL cholesterol or triglyceride-rich lipoproteins. Beam and Verve previously announced a strategic collaboration, under which Verve has exclusive access to Beam's base editing, gene editing and delivery technologies for human therapeutic applications against certain cardiovascular targets. After the completion of Phase 1 studies, Beam has the ability to participate in future development and commercialization, and share 50 percent of U.S. profits and losses, for any product directed against these targets.

Recent Business Highlights

- **Lease Agreement for Beam's In-House Manufacturing Facility to Support Future Product Development:** On August 11, 2020, Beam entered into a lease agreement with Alexandria Real Estate Equities, Inc. to build a 100,000 square foot current Good Manufacturing Practice (cGMP) compliant manufacturing facility in Research Triangle Park, North Carolina that will support a broad range of clinical programs. Beam will invest up to $83 million over a five-year period and anticipates that the facility will be operational by the first quarter of 2023. The project will be facilitated, in part, by a Job Development Investment Grant (JDIG) approved by the North Carolina Economic Investment Committee, which authorizes potential reimbursements to Beam based on new tax revenues generated through the project. The facility will be designed

to support manufacturing for the company's *ex vivo* cell therapy programs in hematology and oncology and *in vivo* non-viral delivery programs for liver diseases, with flexibility to support manufacturing of its viral delivery programs, and ultimately, scale-up to support potential commercial supply.

- **Collaboration Established with Magenta Therapeutics to Evaluate MGTA-117 as a Conditioning Regimen for Base Editing Therapies:** In June 2020**,** Beam and Magenta Therapeutics announced a non-exclusive research and clinical collaboration agreement to evaluate the potential utility of MGTA-117, Magenta's targeted antibody-drug conjugate, for conditioning of patients with sickle cell disease and beta-thalassemia receiving Beam's base editing therapies.

- **Strategic Alliance with Boston Children's Hospital to Accelerate Translational and Clinical Research in Gene Editing for Complex Conditions:** Beam has entered into a strategic alliance agreement with Boston Children's Hospital designed to facilitate the development of disease-specific therapies using Beam's base editing technology. Under the terms of the agreement, Beam will sponsor multiple research programs at Boston Children's Hospital to advance translation of Beam's pipeline across certain therapeutic areas of interest, including programs in sickle cell disease and pediatric leukemias and exploration of new disease areas. Boston Children's Hospital will also serve as a clinical site in the future to advance bench-to-bedside translation of Beam's pipeline.

- **Research Collaboration Established with Institute of Molecular and Clinical Ophthalmology Basel (IOB) to Develop Gene Editing Programs for Ocular Diseases:** Beam has established a research collaboration agreement with the IOB, under which the partners will leverage IOB's expertise in the field of ophthalmology and Beam's base editing technology to advance programs directed to the treatment of certain ocular diseases, including Stargardt disease. Through this collaboration, IOB will leverage insights from ProgStar, an international collaboration studying the natural history study of Stargardt disease progression and helping to determine clinical outcome measures that could be used in clinical trials of future therapies. Additionally, IOB has developed technology that images the retina and choroid with better quality and dimension, as well as living models of the retina, which can be used to study the therapeutic impact base editing could have on ocular diseases.

Second Quarter 2020 Financial Results

- **Cash Position**: Cash, cash equivalents and marketable securities were $228.0 million as of June 30, 2020.

- **Research & Development (R&D) Expenses**: R&D expenses were $19.4 million for the quarter ended June 30, 2020, compared to $12.7 million for the quarter ended June 30, 2019. This increase was primarily due to the growth in the number of research and development employees and their related activities, as well as the expense allocated to R&D related to Beam's leased facilities.

- **General &Administrative (G&A) Expenses**: G&A expenses were $6.9 million for the quarter ended June 30, 2020, compared to $5.0 million for the quarter ended June 30, 2019. This increase was primarily a result of increased personnel related costs due to an increase in general and administrative employees and the cost of being a public company.

- **Net Loss:** Net loss attributable to common stockholders was $34.2 million, or $0.69 per share, for the quarter ended June 30, 2020, compared to $21.1 million for the quarter ended June 30, 2019.

About Beam Therapeutics

Beam Therapeutics (Nasdaq: BEAM) is a biotechnology company developing precision genetic medicines through the use of base editing. Beam's proprietary base editors create 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 life-long cures to patients suffering from serious diseases. For more information, visit www.beamtx.com.

Forward-Looking Statements

This press release contains forward-looking statements. Investors are cautioned not to place undue reliance on these forward-looking statements, including statements about the expected timing of filing INDs applications, the therapeutic applications of our technology and our ability to develop base editors as a new class of precision genetic medicines for patients. Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement. Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the success in development and potential commercialization of our product candidates; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; whether preclinical testing of our product candidates and preliminary or interim data from preclinical and clinical trials will be predictive of the results or success of ongoing or later clinical trials; that enrollment of clinical trials may take longer than expected; that our product candidates will experience manufacturing or supply interruptions or failures; that we will be unable to successfully initiate or complete the preclinical and clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; and the other risks and uncertainties identified under the heading "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2019, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2020 and in any subsequent filings with the Securities and Exchange Commission.

These forward-looking statements (except as otherwise noted) 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
(in thousands)

	June 30, 2020	December 31, 2019
Cash, cash equivalents, and marketable securities	$ 227,950	$ 91,848
Total assets	299,975	156,099
Redeemable convertible preferred stock	—	302,049
Total stockholders' equity (deficit)	230,822	(201,104)

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
License revenue	$ 6	$ 6	$ 12	$ 6
Operating expenses:				
Research and development	19,354	12,680	40,903	21,859
General and administrative	6,937	4,977	13,749	8,906
Total operating expenses	26,291	17,657	54,652	30,765
Loss from operations	(26,285)	(17,651)	(54,640)	(30,759)
Other income (expense):				
Change in fair value of derivative liabilities	(8,700)	(1,000)	(11,400)	(2,000)
Interest and other income, net	767	790	1,364	1,288
Total other expense	(7,933)	(210)	(10,036)	(712)
Net loss	$ (34,218)	$ (17,861)	$ (64,676)	$ (31,471)
Accretion of redeemable convertible preferred stock to redemption value, including dividends on preferred stock	—	(3,226)	(1,277)	(6,189)
Net loss attributable to common stockholders	$ (34,218)	$ (21,087)	$ (65,953)	$ (37,660)
Net loss per common share attributable to common stockholders, basic and diluted	$ (0.69)	$ (3.38)	$ (1.65)	$ (6.26)
Weighted-average common shares used in net loss per share attributable to common stockholders, basic and diluted	49,430,138	6,238,798	40,077,788	6,018,364