



Beam Therapeutics Reports Additional Data at ASGCT Annual Meeting and First Quarter 2020 Financial Results

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Alpha-1 Antitrypsin Deficiency Program Demonstrates More than Four-Fold Increase in Circulating Levels of Functional Protein Following Durable Direct Correction In Vivo

Novel HbG-Makassar Program for Sickle Cell Disease Demonstrates Direct Correction Levels Greater than 80% with Corresponding HbS Globin Reduction to Less than 20%

Base Editing Program to Recreate Hereditary Persistence of Fetal Hemoglobin Shows Greater than 90% Editing and 65% Increase in Gamma Globin Protein after 16 weeks Engraftment

CAMBRIDGE, Mass., May 12, 2020 (GLOBE NEWSWIRE) -- [Beam Therapeutics Inc.](#) (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported additional data from multiple oral and poster presentations during the ongoing 23rd American Society of Gene and Cell Therapy (ASGCT) Annual Meeting as well as its first quarter 2020 financial results.

"The start to 2020 has been one of focused execution across our business and continued advancement of our proprietary base editing platform and portfolio, even with the uncertainty around the COVID-19 pandemic," said John Evans, chief executive officer of Beam. "We are pleased to have a strong showing during ASGCT, in which we are presenting our first *in vivo* proof of concept for base editing in alpha-1 antitrypsin deficiency as well as two potentially best-in-class approaches for base editing in sickle cell disease, including the direct correction of the HbS point mutation. These data are a testament to the compelling therapeutic potential of base editors and, coupled with our financial strength following our initial public offering, position us to continue our comprehensive investment to develop base editors as a new class of precision genetic medicines for patients."

Updated Data Presented at ASGCT

- **Alpha-1 Antitrypsin Deficiency Program Demonstrates Proof-of-Concept in a Mouse Model:** Beam reported additional data from its direct editing program for Alpha-1 in a poster at ASGCT titled "*Use of Adenine Base Editors to Precisely Correct the Disease-Causing PiZ Mutation in Alpha-1 Antitrypsin Deficiency.*" Updated data demonstrate that using Beam's adenine base editors (ABEs) to directly correct the PiZ mutation resulted in an average of 16.9% correction of beneficial alleles at seven days and 28.8% at three months. This significant increase over the period suggests that corrected hepatocytes may have a proliferative advantage relative to uncorrected cells. In addition, treated mice demonstrate decreased alpha-1 antitrypsin (A1AT) globule burden within the liver and a durable increase in serum A1AT, roughly 4.9-fold higher than in controls, was observed at three months. These data support the potential for base editing to treat both the lung and liver manifestations of Alpha-1.
- **HbG-Makassar Program Demonstrates Significant *In Vitro* Direct Editing for Sickle Cell Disease:** Beam reported additional data from its direct correction program for sickle cell disease in a poster titled "*A Novel Base Editing Approach to Directly Edit the Causative Mutation in Sickle Cell Disease.*" Updated data show conversion of the causative HbS point mutation to HbG-Makassar, a naturally-occurring human variant that does not cause hemoglobin to polymerize or red cells to sickle, at levels greater than 80%. A simultaneous reduction of HbS globin to less than 20% of control levels was observed in edited *in vitro*-differentiated erythroid cells from a homozygous sickle cell disease patient. In addition, more than 70% of erythroid colonies derived from edited patient cells showed biallelic editing, with 20% monoallelic and 2% unedited. Further, when *in vitro*-differentiated erythroid cells were subjected to hypoxia, a very significant reduction in sickling was observed. These data demonstrate therapeutic levels of correction and support advancement of this program to potentially address the underlying genetic cause of sickle cell disease.
- **HPFH Program Demonstrates Therapeutically Relevant Increase in Gamma Globin Protein:** Beam will present findings from its HPFH program in an oral presentation titled, "*Base Editing of Gamma Globin Gene Promoters Generates Durable Expression of Fetal Hemoglobin for the Treatment of Sickle Cell Disease.*" Beam's HPFH program aims to recreate naturally-occurring single base changes in the gamma globin gene promoters (HBG1 and HBG2) that disrupt repressor binding and lead to increased expression of gamma globin, which is half of the fetal hemoglobin (HbF) tetramer. Beam observed more than 90% editing and a more than 65% increase in gamma globin levels compared to less than 1.5% in unedited cells in mice at 16 weeks post-transplant. Beam was able to replicate these findings with a second donor at 18 weeks post-transplant. In addition, the data showed successful editing of CD34+ cells from a homozygous sickle patient, demonstrating greater than 60% increase in gamma globin levels with a concomitant decrease to less than 40% in sickle beta globin levels *in vitro*. The data demonstrate that *ex vivo* delivery of ABEs achieved precise editing, resulting in long-term engraftment and therapeutically relevant increases in target gene expression.

Business Continuity Plans

- Beam continues to execute its business objectives for 2020, while closely monitoring the impact of the evolving COVID-19 pandemic. The company continues to operate under a remote model while advancing critical research and development activities to support an initial wave of Investigational New Drug (IND) applications beginning in 2021.

First Quarter 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$253.4 million as of March 31, 2020, which includes \$188.3 million in net proceeds from the company's initial public offering completed in February 2020.
- **Research & Development (R&D) Expenses:** R&D expenses were \$21.5 million for the quarter ended March 31, 2020, compared to \$9.2 million for the quarter ended March 31, 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 \$6.8 million for the quarter ended March 31, 2020, compared to \$3.9 million for the quarter ended March 31, 2019. This increase was primarily a result of increased personnel related costs due to an increase in general and administrative employees and other administrative expenses.
- **Net Loss:** Net loss attributable to common stockholders was \$31.7 million, or \$1.03 per share, for the quarter ended March 31, 2020, compared to \$16.6 million for the quarter ended March 31, 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 our plans for scientific publications, 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 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)

	March 31, 2020	December 31, 2019
Cash, cash equivalents, and marketable securities	\$ 253,442	\$ 91,848
Total assets	322,638	156,099
Redeemable convertible preferred stock	—	302,049
Total stockholders' equity (deficit)	261,394	(201,104)

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

	Three Months Ended March 31,	
	2020	2019
License revenue	\$ 6	\$ —
Operating expenses:		
Research and development	21,549	9,179
General and administrative	6,812	3,929
Total operating expenses	28,361	13,108
Loss from operations	(28,355)	(13,108)
Other income (expense):		
Change in fair value of derivative liabilities	(2,700)	(1,000)
Interest and other income, net	597	498
Total other expense	(2,103)	(502)
Net loss	\$ (30,458)	\$ (13,610)
Accretion of redeemable convertible preferred stock to redemption value, including dividends on preferred stock	(1,277)	(2,963)
Net loss attributable to common stockholders	\$ (31,735)	\$ (16,573)
Net loss per common share attributable to common stockholders, basic and diluted	\$ (1.03)	\$ (2.86)
Weighted-average common shares used in net loss per share attributable to common stockholders, basic and diluted	30,725,077	5,795,481