Beam Therapeutics Publishes Data Highlighting Ability to Rationally Design Base Editors for Precise Editing

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Data Published in The CRISPR Journal *Describe Inlaid Base Editors (IBEs) that Enhance Editing Efficiency and Capability, an Advancement of the Company's Pioneering Work in Developing Base Editors*

Data Demonstrates Utility of IBEs in BEAM-102, Enabling Conversion of Sickle Cell Hemoglobin Allele to the Naturally Occurring, Benign HbG-Makassar Variant in Patient-derived Hematopoietic Stem Cells

CAMBRIDGE, Mass., April 20, 2021 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced the publication of work describing inlaid base editors (IBEs) in *The CRISPR Journal*. IBEs are architectural variants of base editors that have attributes of enhanced specificity and altered activity windows relative to foundational base editors. The work highlights Beam's application of its IBEs for BEAM-102, one of its base editing programs in development for the treatment of sickle cell disease (SCD).

IBEs are an important innovation enabled by Beam's extensive know-how and understanding of structural biology and protein-guided DNA-targeting, which adds to the company's ever-expanding genome editing toolbox. Notably, the predictable, shifted editing window enabled by IBEs aids in further addressing disease-causing mutations normally out of reach with canonical base editors, with high efficiency and minimized off-target effects on the genome.

"Beam is a pioneer in the field of base editing, and this work is a testament to the leadership of our team of scientists in expanding the role that base editing medicines can play in the treatment of a wide variety of diseases," said Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam. "Direct correction of the sickle-causing mutation with traditional gene editing technologies has been limited by low efficiency. The therapeutic potential of our rationally designed IBEs is exciting. Not only have these differentiated base editors demonstrated the potential to efficiently convert the disease-causing sickle hemoglobin allele into a normal variant; they open the doors to potentially target a broad range of other genetic diseases."

Beam's base editors use a DNA-targeted deaminase to create a precise chemical modification of a target DNA base with high precision and limited off-target consequences. IBEs expand the breadth of potential base editing targets by extending the range of editing windows that can be created for any given CRISPR-Cas protein used to target the DNA. By inserting the deaminase into the CRISPR protein at various strategic positions, repositioning the deaminase's active site, IBEs enable editing outside the traditional editing window. The broad modularity of these proprietary base editor designs was demonstrated using both the adenine base editor (ABE) and the cytosine base editor (CBE).

Potential Utility of Beam's IBEs in BEAM-102
BEAM-102 aims to treat SCD by directly editing the causative sickle hemoglobin (HbS) point mutation to recreate a naturally occurring normal human hemoglobin variant, HbG-Makassar. The Makassar variant has been reported to have the same function as the wild-type (HbA) allele and does not cause SCD. Findings in the paper show that Beam's IBE resulted in highly efficient editing levels of over 70% in CD34+ cells from both sickle trait (HbAS) and sickle cell disease (HbSS) individuals. Additionally, the IBE demonstrated significantly reduced guide-independent off-target editing *in vitro*.

"Beam was launched in 2017 by world-leading innovators in gene editing who discovered that base editing is capable of making single-base changes with high efficiency and precise control of editing outcomes," said John Evans, chief executive officer of Beam Therapeutics. "With the development of our IBEs, we have once again set a precedent for the expansion and advancement of base editing as a new generation of gene editing technology. In addition, the patent applications on this work complement and extend Beam's comprehensive patent portfolio in the field of base editing. We are dedicated to fully realizing the potential of this technology for patients as we advance our portfolio, with plans to submit what we expect will be the first IND application for a base editor program in the second half of 2021."

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 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 life-long cures to patients suffering from serious diseases.

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 expected timing of filing our first investigational new drug application, our ability to advance programs to the clinic; and the therapeutic applications and potential of our technology, including our IBEs and our ability to develop life-long, curative, precision genetic medicines for patients through base editing. 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, 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 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 heading “Risk Factors” in our Annual Report on Form 10-K for the year ended December 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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