Beam Therapeutics Announces Multiple Scientific Advances in its Base Editing Technology Platforms

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New Research Describes Novel Base Editors with Enhanced Editing Efficiency, Specificity, and Targeting Range

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 11, 2020-- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced continued progress in the evolution of its novel adenine base editor (ABE) and cytosine base editor (CBE) technologies, as reviewed in three recent publications:

- Keynote presentation at The 1st International Conference on Base Editing – Enzymes and Applications titled "ABE8: Superior adenine base editors with expanded targeting range, higher activity, and therapeutic application" presented by Nicole Gaudelli, Ph.D., senior scientist and head of DNA Editing Platform at Beam.
- *Nature Biotechnology*paper titled "Evaluation and Minimization of Cas9-Independent Off-Target DNA Editing by Cytosine Base Editors" by co-lead authors Jordan L. Doman and Aditya Raguram, with corresponding author David R. Liu, Ph.D., professor at Broad Institute and Harvard University, Howard Hughes Medical Institute investigator, and co-founder of Beam.
- *Nature Biotechnology*paper titled "Continuous Evolution of SpCas9 Variants Compatible with Non-G PAMs" by co-lead authors Shannon M. Miller and Tina Wang, Ph.D., also with Dr. Liu as a corresponding author.

"Base editing is an emerging and highly differentiated new editing technology, and Beam is committed to further enhancing our base editing platform to potentially enable a wide range of therapeutic applications," said Giuseppe Ciaramella, Ph.D., president and chief scientific officer. "These recent advancements from Beam scientists and our academic partners highlight our continuing ability to optimize base editors. We believe base editors have transformational potential for the field of precision genetic medicines."

Beam's base editors represent a potentially new class of precision genetic medicines that enable precise, predictable and efficient single base changes in the genome. ABEs and CBEs can make precisely-targeted A-to-G and C-to-T DNA base changes, respectively, with high efficiency and without the introduction of double-stranded breaks in the DNA, which can result in unwanted DNA insertions, deletions and rearrangements.

In the first publication, Dr. Gaudelli's presentation highlighted the continued evolution of Beam's adenine base editors (ABEs). Dr. Gaudelli used a bacterial selection strategy to identify ABEs with enhanced properties in mammalian cells. These proprietary "ABE8" variants demonstrated increased therapeutic potential relative to previously-published versions, including increased on-target activity while maintaining undetectable levels of off-target editing.

In the second publication, Dr. Liu's *Nature Biotechnology* paper describes novel cytosine base editors (CBEs), which diminish or eliminate the low levels of off-target editing observed with the first published version of CBEs (known as BE3). Off-target editing rates observed with BE3 are very low and are within mutation rates that naturally occur with no consequence in somatic cells. Dr. Liu's laboratory identified certain novel mutations in the deaminase domain that yielded base editors that retained high on-target editing while eliminating detectable off-target editing.

Finally, an additional publication from Dr. Liu's laboratory, also in *Nature Biotechnology*, outlined novel variants of base editors that are able to bind a wider range of genetic targets, expanding their therapeutic potential. These editors feature modifications that allow them to bind to different "PAM" sequences in the genome. PAM sequences are short sequences of DNA bases that define where the targeting domain of the base editor can bind. The newly developed variants could potentially target up to 95% of all point mutations correctable with base editors and were shown to be compatible with both ABEs and CBEs.

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 focused on 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 ability of base editors to target up to 95% of all point mutations correctable with base editors. 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 identified under the heading "Risk Factors" and elsewhere in the final prospectus dated February 5, 2020 related to our initial public offering, which are available on the SEC's website at www.sec.gov. Additional information will be made available by our quarterly reports on Form 10-Q and other filings that we make from time to time with the SEC. 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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