# Beam Therapeutics Announces Upcoming Preclinical Data Presentations at the American Society of Hematology Annual Meeting

November 4, 2021

*Preclinical Data Demonstrates that BEAM-102 Makassar Base Editing Converts Sickle Hemoglobin to Functional Hemoglobin for Potential Treatment of Sickle Cell Disease*

*New* In Vivo *Data Highlight Novel LNP Screening Technology to Identify LNPs for Delivery of Base Editors to Tissues Beyond the Liver, Including to Blood Stem Cells*

CAMBRIDGE, Mass., Nov. 04, 2021 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced that preclinical data highlighting the company's Makassar base editing approach for the potential treatment of sickle cell disease (SCD) and its proprietary lipid nanoparticle (LNP) screening capabilities will be presented during two poster sessions at the 63rd American Society of Hematology (ASH) Annual Meeting. 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 titled, "Gene Editing 2.0: Advances in Gene Editing to Treat Genetic Blood Disorders." The ASH Annual Meeting is being held December 11-14, 2021, virtually and in Atlanta.

"We look forward to presenting these new data at ASH, which further support our leadership position in the field of base editing and the significant opportunity we have to potentially make a difference for patients in need of meaningful treatment options," said Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam. "The additional preclinical data being presented from our Makassar program continue to validate the potential of our base editing approach to treating SCD, which is designed to correct sickle globin to a normally functioning hemoglobin variant. In addition, the ability of our *in vivo* high-throughput LNP screening technology to identify novel LNPs capable of delivering our editors to stem cells is exciting and supports a promising delivery platform that could eventually eliminate the need for transplant in sickle cell disease and other genetic blood disorders. Taken together, these data, coupled with our ongoing work around non-genotoxic conditioning, give us hope to be able to provide a more holistic treatment approach for patients with SCD and beyond."

Details of the poster presentations are as follows:

**Poster Title:** *Conversion of HbS to Hb G-Makassar by Adenine Base Editing is Compatible with Normal Hemoglobin Function*
**Session Name:** 113. Hemoglobinopathies, Excluding Thalassemia: Basic and Translational: Poster I
**Date:** Saturday, December 11, 2021
**Presentation Time:** 5:30p.m - 7:30p.m.
**Location:** Georgia World Congress Center, Hall B5
**Abstract Summary:** Beam is advancing BEAM-102, its Makassar base editing approach, as a potential treatment for SCD that is constructed to directly edit the causative HbS point mutation to recreate a naturally occurring normal human hemoglobin variant, HbG-Makassar. The Makassar variant has been reported to be a functional beta-hemoglobin that is not pathogenic. In preclinical studies, Beam sought to further characterize Makassar hemoglobin, evaluating its size, molecular weight, oligomerization and polymerization potential, oxygen binding properties, as well as solving its crystal structure. Altogether, Beam's biophysical and biochemical characterization shows that Makassar globin behaves as a functional hemoglobin and represents a promising investigational approach for the treatment of sickle cell disease.

**Poster Title:** *Screening of Chemically Distinct Lipid Nanoparticles In Vivo Using DNA Barcoding Technology Towards Effectively Delivering Messenger RNA to Hematopoietic Stem and Progenitor Cells*
**Session Name:** 801. Gene Therapies: Poster II
**Date:** Sunday, December 12, 2021
**Presentation Time:** 6:00p.m. - 8:00p.m.
**Location:** Georgia World Congress Center, Hall B5
**Abstract Summary:** Beam is developing an approach to directly deliver base editors to hematopoietic stem and progenitor cells (HSPCs) *in vivo* through non-viral delivery methods, such as LNPs for the potential delivery of base editors as a treatment for a range of hemoglobinopathies. To identify optimal LNPs for delivery to HSPCs, leveraging its proprietary DNA barcoding technology, Beam screened more than 1,000 LNPs and identified LNP-HSC1 as the most potent. LNP-HSC1 was validated *in vivo*, leading to durable, dose-dependent mRNA transfection in HSPCs of more than 40%, which was maintained for 10 weeks post-delivery. Further, LNP-HSC1 efficiently transfected mice at doses ranging from 0.3mg/kg-1.0mg/kg, and in non-human primates, a dose-dependent increase in mRNA in bone marrow-derived CD34+ HSPCs was observed.

**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.

**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: our planned base editing and LNP screening data presentations at an upcoming scientific conference; the therapeutic applications and potential of our technology, including with respect to SCD and LNP screening; and 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, 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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