Beam Therapeutics Announces Updated Preclinical Data Highlighting Optimized LNP Delivery Approaches for In Vivo Base Editing to the Liver and Other Tissues

September 23, 2021

CAMBRIDGE, Mass., Sept. 23, 2021 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced new preclinical data highlighting advancements with the company's approach to developing novel lipid nanoparticle (LNP) formulations for *in vivo* liver editing. In addition, the company reported initial *in vivo* data demonstrating its delivery capabilities to tissues outside of the liver. The data will be presented today, September 23, 2021, from 11:15 – 11:45 a.m. ET at the TIDES USA Oligonucleotide & Peptide Therapeutics Conference (TIDES 2021) in a presentation titled "*Optimization of LNP for in vivo base editing*."

"We are committed to advancing innovative genetic medicines to reach as many patients as possible, and our progress toward that is exemplified by the data reported today with our novel delivery technologies," said Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam. "Continued optimization of our LNPs has led to a substantial increase in liver editing potency to what we believe could be a clinically relevant dose for our lead LNP program, which we plan to finalize later this year. Importantly, data from our studies in non-human primates (NHPs) showed that these formulations were well tolerated and had good early storage stability in ongoing studies. We've also generated encouraging data using our novel LNP formulations gained through our acquisition of Guide Therapeutics, which could have meaningful application for delivery of our base editors to tissues beyond the liver, broadening the potential reach of our genetic medicines."

Using an mRNA-encoding adenine base editor (ABE) and guide RNA, Beam evaluated various LNP formulations and mRNA production processes to optimize its LNPs for improved *in vivo* liver editing and to avoid immune stimulation in NHPs. The findings highlight key improvements in Beam's LNP delivery system for the liver, including:

- Increased editing potency in NHPs, demonstrating up to 60% editing at 1.0 mg/kg;
- Well-tolerated formulations in NHPs treated with up to 1.5 mg/kg LNP, with minimal to mild increases in transient liver enzyme elevations that were resolved by day 15 post-treatment; and
- Stable formulations with potency maintained at -20 and -80 ºC out to three months.

In addition, Beam reported an update on its proprietary approach to developing LNPs to deliver base editors to tissues beyond the liver, including hematopoietic stem and progenitor cells (HSPCs). Leveraging its DNA barcoding technology, Beam identified a family of LNPs for delivery of base editors to HSPCs in mice, with administration at 1.0 mg/kg leading to 40% expression of mRNA cargo in cells. Beam is evaluating this delivery approach for potential application in hemoglobinopathies and other genetic blood disorders.

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: our planned base editing data presentations at upcoming scientific conferences; our belief that we may have identified a clinically relevant dose for our lead LNP program; our ability to deliver base editors to tissues beyond the liver; and the therapeutic applications and potential of our technology, including 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 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 (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.

Contacts:

Investors:
Chelcie Lister
THRUST Strategic Communications
chelcie@thrustsc.com

Media:
Dan Budwick
1AB
dan@1abmedia.com