Beam Therapeutics Presents LNP Formulation Data at ASGCT and Reports First Quarter 2021 Financial Results

May 11, 2021

Data from Beam's Novel LNP-mRNA Formulation Demonstrates In Vivo *Editing in Liver Cells of Non-human Primates Up to 52%*

Company On-track to Submit First IND for BEAM-101 in the Second Half of 2021

CAMBRIDGE, Mass., May 11, 2021 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported recent business highlights and pipeline updates, as well as first quarter 2021 financial results.

"As Beam prepares to enter the clinic, we also continue to extend our leadership position in the field of base editing by expanding our platform and delivery capabilities," said John Evans, chief executive officer of Beam. "The data presented at ASGCT on our proprietary lipid nanoparticle (LNP) formulation show efficient *in vivo* base editing in hepatocytes of non-human primates (NHPs), while our novel analytical assays confirm specificity and efficiency of base editing to directly correct disease-causing mutations. As we look to the rest of 2021, we are on-track with our plans to submit our first Investigational New Drug (IND) application for BEAM-101 and initiate IND-enabling studies for BEAM-102 and BEAM-201, as well as nominate our first development candidate from our liver portfolio. We are well positioned today to advance our base editing platform and pipeline and remain focused on achieving our vision of providing life-long cures for patients suffering from serious diseases."

"We are pleased to share our development of a proprietary LNP formulation for non-viral *in vivo* delivery to the liver at ASGCT," said Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam. "These data demonstrate that our LNPs are well tolerated in NHPs and can achieve levels of editing of hepatocytes that we expect would be therapeutic for many genetic diseases. Initial data also demonstrated the stability of our LNPs at -20 degrees Celsius after 8 weeks, which is important for enabling broad clinical and commercial development. We are continuing to work on optimizing our LNP formulations and are on track to nominate our first development candidate from our liver portfolio in the second half of 2021."

Base Editing Progress

- **Data Demonstrating Optimization of LNP-mRNA Formulation for Liver Editing Presented at the 24th American Society of Gene and Cell Therapy (ASGCT) Annual Meeting:** Beam's approach to developing a novel lipid nanoparticle (LNP) formulation for *in vivo* liver editing will be presented during a poster session titled, "Using Base Editing and LNP Delivery to Correct Disease-Causing Mutations Underlying Genetic Liver Diseases" at ASGCT. Using an mRNA-encoding adenine base editor (ABE) and guide RNA to target the ALAS1 gene, a surrogate payload for genetic liver diseases, Beam evaluated various LNP formulations and mRNA production processes to improve *in vivo* editing in the livers of NHPs from less than 10% initially to 52% at a total RNA dose of 1.5 mg/kg. These formulations were also well tolerated by NHPs at 1.5 mg/kg with mild and transient liver enzyme elevations, and showed promising interim stability, maintaining potency after 8 weeks at -20ºC.

- **Data Highlighting Beam's Approach to Confirming Precision Correction Using Novel Analytical Assays Presented at ASGCT:** Base editing is emerging as a powerful next-generation editing technology; however, developing adequate analytical assays to confirm precise base editing at the protein level is critical and has proven historically challenging. In an oral presentation at ASGCT titled, "*LC-MS Confirmation of Single Amino Acid Correction by Base Editing*," Beam will describe its approach to using liquid chromatography mass spectrometry (LC-MS) for multiple analytical assays to confirm the precise correction of disease-causing mutations at the amino acid level, providing a unique solution for confirmation and quantitation of single amino acid corrections after base editing.

- **Data Highlighting Ability to Rationally Design Base Editors for Precise Editing Published in *The CRISPR Journal:*** In April 2021, work describing Beam's approach to developing inlaid base editors (IBEs) was published in *The CRISPR Journal*. IBEs are architectural variants of base editors that demonstrate 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.

First Quarter 2021 Financial Results

- **Cash Position**: Cash, cash equivalents and marketable securities were $503.5 million as of March 31, 2021, compared to $253.4 million as of March 31, 2020.
- **Research & Development (R&D) Expenses**: R&D expenses were $190.1 million for the first quarter of 2021, compared to

$21.5 million for the first quarter of 2020. R&D expenses for the first quarter of 2021 includes $155.0 million of expense related to in-process research and development acquired from of Guide Therapeutics, Inc.

- **General & Administrative (G&A) Expenses**: G&A expenses were $10.3 million for the first quarter of 2021, compared to $6.8 million for the first quarter of 2020.
- **Net Loss:** Net loss attributable to common stockholders was $201.6 million, or $3.35 per share, for the first quarter of 2021, compared to $31.7 million, or $1.03 per share, for the first quarter of 2020.

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 plans for scientific publications; our plans to enter the clinic; our expected timing for filing an investigational new drug application for BEAM-101, for initiating IND-enabling studies for BEAM-102 and BEAM-201, and for nominating our first development candidate from our liver portfolio; 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 heading "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, 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

Condensed Consolidated Balance Sheet Data
(in thousands)

	March 31, 2021	December 31, 2020
Cash, cash equivalents, and marketable securities	$ 503,479	$ 299,671
Total assets	693,241	451,677
Total liabilities	270,812	206,116
Total stockholders' equity	422,429	245,561

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

	Three Months Ended March 31,	
	2021	2020
License revenue	$ 6	$ 6
Operating expenses:		
Research and development	190,106	21,549
General and administrative	10,273	6,812

Total operating expenses	200,379	28,361
Loss from operations	200,373	(28,355)
Other income (expense):		
Change in fair value of derivative liabilities	(1,900)	(2,700)
Change in fair value of contingent consideration liabilities	(305)	—
Interest and other income, net	1,018	597
Total other income (expense)	(1,187)	(2,103)
Net loss	$ (201,560)	$ (30,458)
Accretion of redeemable convertible preferred stock to redemption value, including dividends on preferred stock	—	(1,277)
Net loss attributable to common stockholders	$ (201,560)	$ (31,735)
Net loss per common share attributable to common stockholders, basic and diluted	$ (3.35)	$ (1.03)
Weighted-average common shares used in net loss per share attributable to common stockholders, basic and diluted	60,210,120	30,725,077