Beam Therapeutics Announces Business and Pipeline Progress and Reports Fourth Quarter and Full Year 2020 Financial Results

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

Acquisition of Guide Therapeutics Supports Targeting of Diverse Tissues for In Vivo Delivery of Gene Editing

Team Bolstered by Appointment of Amy Simon, M.D., as Chief Medical Officer and Kate Walsh to Board of Directors

Balance Sheet Strengthened by $260 Million Common Stock Investment

CAMBRIDGE, Mass., March 15, 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 fourth quarter and full year 2020 financial results.

“Beam has made significant progress in the last 12 months,” said John Evans, chief executive officer of Beam. “Since our IPO in February 2020, we named three development candidates from our portfolio; initiated IND-enabling studies for BEAM-101; presented data highlighting our broad set of gene editing technologies; expanded access to novel delivery modalities through the acquisition of Guide Therapeutics; secured and initiated work on our build-to-suit manufacturing facility; enhanced our leadership team with the addition of Amy Simon as chief medical officer; expanded our board with the appointment of Kate Walsh; and further strengthened our capital position and operating runway. In the remainder of 2021, we plan to submit our first Investigational New Drug application for BEAM-101, initiate IND-enabling studies for BEAM-102 and BEAM-201, and nominate our first development candidate from our liver portfolio, positioning Beam to become a clinical stage company with an expansive pipeline moving quickly behind our lead assets. We are well positioned today to advance our platform and pipeline, and remain focused on achieving our vision of providing life-long cures for patients suffering from serious diseases.”

Recent Business Highlights

- **Acquisition of Guide Therapeutics (GuideTx) Expands Novel Base Editing Capabilities:** In February 2021, Beam acquired GuideTx, a developer of nonviral drug delivery vehicles for genetic medicines, further expanding the potential reach of Beam’s genetic medicines into target tissues beyond the liver. GuideTx’s technology enables the screening of hundreds of nanoparticles in a single experiment, potentially generating *in vivo* drug delivery data at significantly greater rates compared to traditional screens. Beam believes that the proprietary screening method will enable identification of lipid nanoparticles with novel biodistribution and high selectivity for target cells. Using this platform and a proprietary library of cationic lipids, GuideTx has identified a broad array of novel lipid nanoparticle formulations.

- **Balance Sheet Enhanced with $260 Million Private Placement:** In January 2021, Beam sold 2,795,700 shares of its common stock to certain institutional investors in a private placement. Aggregate gross proceeds from the offering were approximately $260 million, before deducting fees to the placement agents and other offering expenses payable by the Company.

- **Leadership Team Strengthened by the Addition of Amy Simon, M.D., as Chief Medical Officer:** In March 2021, Beam appointed Amy Simon, M.D., as chief medical officer. Dr. Simon joins Beam from Alnylam Pharmaceuticals, where she spent over a decade in various roles with increasing responsibility for the clinical development of numerous RNAi-based medicines, most recently serving as vice president, clinical development and the lead clinician developing GIVLAARI® (givosiran) for acute hepatic porphyria, which was approved by FDA in 2019. Dr. Simon holds a B.A. in history and science from Harvard University and received her M.D. from Tufts University School of Medicine.

- **Leading Healthcare Executive, Kate Walsh, Appointed to Board of Directors:** In January 2021, Kate Walsh, president and chief executive officer of the Boston Medical Center (BMC) health system, joined Beam’s board of directors. BMC is a private, not-for-profit, academic medical center with a community-based focus and is the primary teaching affiliate of Boston University School of Medicine.

Base Editing Progress

- **Updated Data from Novel Sickle Cell Disease Approaches Presented at 62nd American Society of Hematology Annual Meeting and Exposition (ASH 2020):** In December 2020, Beam presented updated data from the company’s complementary base editing approaches to treat hemoglobinopathies, BEAM-101 and BEAM-102, during poster sessions

at ASH 2020. The BEAM-101 data presented demonstrated precise base editing with no off-target editing observed, supporting the company's planned IND submission in the second half of 2021. Additionally, *in vivo* data from the company's Makassar base editing program demonstrated long-term engraftment and editing retention at 16 weeks, with BEAM-102 achieving greater than 90% bi- and mono-allelic Makassar editing in SCD CD34+ HSPCs *in vitro*.

- **First Data Highlighting Base Editing Program for Glycogen Storage Disease Type Ia Presented at American Association for the Study of Liver Diseases' (AASLD) The Liver Meeting Digital Experience™:** In November 2020, Beam presented the first data highlighting its novel base-editing strategy for correcting disease-causing mutations underlying Glycogen Storage Disease Type Ia (GSDIa) during a poster session at AASLD, demonstrating *in vivo* correction of the R83C and Q347X mutations by ABEs in the livers of two strains of transgenic mice, each carrying one of the two G6PC mutations. Next-generation sequencing data from whole liver extracts revealed significant correction for both R83C and Q347X, with nearly 40% and approximately 70% A-to-G conversion efficiency, respectively, of each mutation back to the normal gene sequence. These significant levels of mutation correction greatly surpassed those expected to restore glucose homeostasis, and functional studies are ongoing to correlate pathophysiology to extent of mutation correction by base-editing. Further, these levels of *in vivo* correction for GSDIa by base-editing were achieved without detectable creation of double-stranded breaks. In total, these data support base-editing technology as a promising approach for precise correction of causative mutations in GSDIa.

Fourth Quarter and Full Year 2020 Financial Results

- **Cash Position**: Cash, cash equivalents and marketable securities were $299.7 million as of December 31, 2020, compared to $91.8 million as of December 31, 2019. This cash balance does not include the proceeds from the January 2021 private placement.
- **Research & Development (R&D) Expenses**: R&D expenses were $32.5 million for the fourth quarter of 2020 and $103.2 million for the full year ended December 31, 2020, compared to $20.2 million for the fourth quarter of 2019 and $54.6 million for the full year ended December 31, 2019.
- **General & Administrative (G&A) Expenses**: G&A expenses were $8.4 million for the fourth quarter of 2020 and $29.6 million for the full year ended December 31, 2020, compared to $6.2 million for the fourth quarter of 2019 and $20.6 million for the full year ended December 31, 2019.
- **Net Loss:** Net loss attributable to common stockholders was $95.5 million, or $1.69 per share, for the fourth quarter of 2020 and $195.9 million, or $4.19 per share, for the full year ended December 31, 2020, compared to $31.1 million, or $4.35 per share, for the fourth quarter of 2019 and $91.0 million, or $14.05 per share, for the full year ended December 31, 2019.

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, nominating our first development candidate from our liver portfolio, and initiating IND-enabling studies for BEAM-102 and BEAM-201; our ability to advance programs to the clinic; our ability to expand the reach of gene editing, including as a result of our acquisition of Guide Therapeutics; the strategic and other potential benefits of the Guide Therapeutics acquisition; the sufficiency of our cash position; 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; our ability to successfully integrate Guide Therapeutics' operations and employees and to realize the anticipated benefits of the acquisition; 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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Condensed Consolidated Balance Sheet Data
(in thousands)

	December 31,	
	2020	**2019**
Cash, cash equivalents and marketable securities	$ 299,671	$ 91,848
Total assets	451,677	156,099
Redeemable convertible preferred stock	-	302,049
Total stockholders' equity (deficit)	245,561	(201,104)

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

	Three months ended December 31,		Year ended December 31,	
	2020	**2019**	**2020**	**2019**
License revenue	$ 6	$ 6	$ 24	$ 18
Operating expenses:				
Research and development	32,451	20,217	103,179	54,619
General and administrative	8,354	6,160	29,605	20,553
Total operating expenses	40,805	26,377	132,784	75,172
Loss from operations	(40,799)	(26,371)	(132,760)	(75,154)
Other income (expense):				
Change in fair value of derivative liabilities	(54,700)	(1,800)	(63,400)	(5,400)
Interest and other income (expense), net	35	321	1,568	2,228
Total other income (expense)	(54,665)	(1,479)	(61,832))	(3,172)
Net loss	$ (95,464)	$ (27,850)	$ (194,592)	$ (78,326)
Accretion of redeemable convertible preferred stock to redemption value, including dividends on preferred stock	-	(3,263)	(1,277)	(12,714)
Net loss attributable to common stockholders	$ (95,464)	$ (31,113)	$ (195,869)	$ (91,040)
Net loss per common share attributable to common stockholders, basic and diluted	$ (1.69)	$ (4.35)	$ (4.19)	$ (14.05)
Weighted-average common shares used in net loss per share attributable to common stockholders, basic and diluted	56,544,620	7,149,568	46,733,221	6,479,591