



Beam Therapeutics Provides Business and Pipeline Updates and Reports Second Quarter 2021 Financial Results

August 10, 2021

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

Continued Progress Across Base Editing Portfolio, Including Initiation of IND-Enabling Studies for BEAM-201

CAMBRIDGE, Mass., Aug. 10, 2021 (GLOBE NEWSWIRE) -- [Beam Therapeutics Inc.](#) (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported recent business and pipeline updates, as well as second quarter 2021 financial results.

"We have made meaningful progress in advancing our base editing programs in the first half of the year, and importantly, remain on track to submit our first investigational new drug (IND) for BEAM-101 in the second half of this year," said John Evans, chief executive officer of Beam. "With the initiation of IND-enabling studies for BEAM-201, we are now bringing the versatility and precision of base editing to a second therapeutic area, targeting the high unmet need in T-cell cancers with the first quad-edited cell therapy. We've also continued to expand our innovative collaborator network, most recently through our partnership with Apellis to apply base editing to the more biologically complex disease area of the complement pathway. With a strong balance sheet, we are well positioned to advance our robust pipeline of novel base editing programs through IND filings and into the clinic as we strive to provide potentially life-long cures for patients suffering from serious diseases."

Base Editing Pipeline Progress

- **IND-Enabling Studies for BEAM-201 Initiated:** Beam has initiated IND-enabling studies for BEAM-201 for the treatment of relapsed/refractory T-cell acute lymphoblastic leukemia (T-ALL). BEAM-201 is a potent and specific allogeneic CAR-T targeting CD7+ malignancies, which uses multiplex base editing to simultaneously make four knockout edits at over 96% efficiency for each gene. Relapsed/refractory T-ALL is a severe disease affecting children and adults with a five-year overall survival rate of less than 25%.
- **Additional Upcoming Milestones in *Ex Vivo* and *In Vivo* Base Editing Pipeline On Track:** Beam anticipates filing an IND for BEAM-101, which reproduces single base changes seen in individuals with Hereditary Persistence of Fetal Hemoglobin, or HPFH, to potentially protect them from the effects of mutations causing sickle cell disease (SCD), and initiating IND-enabling studies for BEAM-102, which directly corrects the causative mutation in SCD by recreating a naturally-occurring normal human hemoglobin variant, both in the second half of 2021. Beam is also on track to nominate its first Development Candidate for *in vivo* base editing of the liver using LNP delivery by the end of 2021.
- **Data Highlighting Base Editing Approach for the Treatment of Alpha-1 Antitrypsin Deficiency Published in *Molecular Therapy*:** In July 2021, data from a preclinical study led by the Center for Regenerative Medicine at Boston Medical Center and Boston University evaluating Beam's base editors in induced pluripotent stem cells (iPS cells) from patients with alpha-1 antitrypsin deficiency (AATD) were [published in *Molecular Therapy*](#). As part of the study, the researchers utilized patient-derived liver cells that mimic the biology of liver hepatocytes, the main producers of alpha-1 antitrypsin protein in the body, that likewise perform important metabolic, endocrine and secretory functions. The data showed that Beam's base editors corrected the mutation in the gene responsible for AATD and reduced the effects of the disease in the hepatocytes, demonstrating successful base editing in human cells.

Upcoming Base Editing Data Presentations

- **Data from Sickle Cell Disease Program to be Presented at Cold Spring Harbor Laboratory's Genome Engineering: CRISPR Frontiers:** Beam plans to present preclinical data highlighting its approach to using adenine base editing (ABE) for the treatment of sickle cell disease, including updated safety data during an oral session at the Cold Spring Harbor Laboratory's Genome Engineering: CRISPR Frontiers Conference. Details of the presentation are as follows:

Title: Adenine base editing strategy for the treatment of sickle cell disease by elimination of the pathogenic globin protein

Session: Disease/Therapeutic

Date: Friday, August 20, 2021, 2:30 -5:30 p.m. ET

- **Updated Data on Optimization of LNP Delivery Approach to be Presented at TIDES 2021:** Beam plans to present preclinical data highlighting work to optimize its lipid nanoparticles (LNPs) for the delivery of mRNA encoding a base editor

and guide RNA to hepatocytes in the liver during an oral session at the TIDES USA Oligonucleotide & Peptide Therapeutics Conference. Details of the presentation are as follows:

Title: Optimization of LNP for *in vivo* Base Editing in Liver

Track: mRNA and Genome Editing TRACK: Emerging Genome Editing Technologies

Date/Time: Thursday, September 23, 2021, 11:15 a.m. - 11:45 a.m. ET

Business Highlights

- **Groundbreaking on Internal Manufacturing Facility to Support Future Product Development:** In July 2021, Beam broke ground on its 100,000 square foot current Good Manufacturing Practice (cGMP) compliant manufacturing facility in Research Triangle Park, North Carolina. The facility will be designed to support manufacturing for Beam's *ex vivo* cell therapy programs in hematology and oncology and *in vivo* non-viral delivery programs for liver diseases, with flexibility to support manufacturing of its viral delivery programs, and ultimately, scale-up to support commercial supply. Beam expects the facility to be operational in 2023.
- **Exclusive Research Collaboration Executed with Apellis to Apply Base Editing to Discover Novel Therapies for Complement-Driven Diseases:** In June 2021, Beam and Apellis Pharmaceuticals, Inc. (Apellis) [announced an exclusive five-year research collaboration](#) focused on the use of Beam's proprietary base editing technology to discover new treatments for complement-driven diseases. Under the terms of the collaboration agreement, Beam will apply its base editing technology and conduct preclinical research on up to six base editing programs that target specific genes within the complement system, including C3, in various organs, including the eye, liver and brain. Apellis will have exclusive rights to license each of the six programs and will assume responsibility for subsequent development. Apellis agreed to pay a total of \$75 million in upfront and near-term milestones to Beam. Beam is also eligible to receive development, regulatory and sales milestones, as well as royalty payments on sales of licensed products. Beam may also elect to enter into a 50-50 U.S. co-development and co-commercialization agreement with Apellis with respect to one program licensed under the collaboration.

Second Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$615.3 million as of June 30, 2021, compared to \$299.7 million as of December 31, 2020.
- **Research & Development (R&D) Expenses:** R&D expenses were \$45.6 million for the second quarter of 2021, compared to \$19.4 million for the second quarter of 2020.
- **General & Administrative (G&A) Expenses:** G&A expenses were \$13.4 million for the second quarter of 2021, compared to \$6.9 million for the second quarter of 2020.
- **Net Loss:** Net loss attributable to common stockholders was \$76.3 million, or \$1.23 per share, for the second quarter of 2021, compared to \$34.2 million, or \$0.69 per share, for the second quarter of 2020.

About Beam Therapeutics

Beam Therapeutics Inc. (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 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, for nominating a development candidate for *in vivo* base editing of the liver using LNP delivery, and for our manufacturing facility becoming operational; any future payments we may receive under our collaboration agreement with Apellis; our planned base editing data presentations at upcoming scientific conferences; 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 that we will file 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.

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Condensed Consolidated Balance Sheet Data (unaudited)
(in thousands)

	<u>June 30, 2021</u>	<u>December 31, 2020</u>
Cash, cash equivalents, and marketable securities	\$ 615,279	\$ 299,671
Total assets	893,078	451,677
Total liabilities	344,527	206,116
Total stockholders' equity	548,551	245,561

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

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2021</u>	<u>2020</u>	<u>2021</u>	<u>2020</u>
License revenue	\$ 6	\$ 6	\$ 12	\$ 12
Operating expenses:				
Research and development	45,577	19,354	235,683	40,903
General and administrative	13,403	6,937	23,676	13,749
Total operating expenses	<u>58,980</u>	<u>26,291</u>	<u>259,359</u>	<u>54,652</u>
Loss from operations	(58,974)	(26,285)	(259,347)	(54,640)
Other income (expense):				
Change in fair value of derivative liabilities	(42,300)	(8,700)	(44,200)	(11,400)
Change in fair value of long-term investments	25,814	517	26,852	517
Change in fair value of contingent consideration liabilities	(741)	—	(1,046)	—
Interest and other income (expense), net	(52)	250	(72)	847
Total other income (expense)	<u>(17,279)</u>	<u>(7,933)</u>	<u>(18,466)</u>	<u>(10,036)</u>
Net loss	<u>\$ (76,253)</u>	<u>\$ (34,218)</u>	<u>\$ (277,813)</u>	<u>\$ (64,676)</u>
Accretion of redeemable convertible preferred stock to redemption value, including dividends on preferred stock	—	—	—	(1,277)
Net loss attributable to common stockholders	<u>\$ (76,253)</u>	<u>\$ (34,218)</u>	<u>\$ (277,813)</u>	<u>\$ (65,953)</u>
Net loss per common share attributable to common stockholders, basic and diluted	<u>\$ (1.23)</u>	<u>\$ (0.69)</u>	<u>\$ (4.54)</u>	<u>\$ (1.65)</u>
Weighted-average common shares used in net loss per share attributable to common stockholders, basic and diluted	<u>62,210,239</u>	<u>49,430,138</u>	<u>61,215,705</u>	<u>40,077,788</u>