May 6, 2020

Abeona Therapeutics Reports First Quarter Financial Results and Business Updates

First patient treated in ongoing pivotal Phase 3 VIITAL™ study evaluating EB-101 for RDEB

Additional patients dosed in the Phase 1/2 gene therapy studies for MPS IIIA and MPS IIIB

Positive interim data from MPS III gene therapy programs presented at WORLDSymposium™; updated results to be presented at ASGCT 2020

Appointed two industry leaders to Board of Directors and strengthened management team

Company to host investor conference call Thursday, May 7, 2020 at 8:30 a.m. ET

NEW YORK and CLEVELAND, May 06, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced first quarter 2020 financial results, which will be discussed on a conference call scheduled for Thursday, May 7, 2020 at 8:30 a.m. ET. Interested parties are invited to participate in the call by dialing 844-455-1352 (U.S. toll-free) or 509-844-0155 (international), and reference conference ID 5352629, or via webcast at https://investors.abeonatherapeutics.com/ir-calendar.

“During these challenging times, our priority remains to ensure the safety of our employees and patients, while supporting continuity of our business and clinical operations,” said João Siffert, M.D., Chief Executive Officer of Abeona. “We have made considerable progress in our clinical programs in the first quarter of 2020, and are working with our investigators to minimize the impact of the COVID-19 pandemic. It is our intention to restore full patient access to our clinical programs as soon as possible. Our gene and cell therapies in development aim at addressing urgent unmet needs, and have the potential to provide durable benefit to patients who have no approved treatments.”

First Quarter and Recent Highlights

- First patient treated in pivotal Phase 3 VIITAL™ study evaluating EB-101 for recessive dystrophic epidermolysis bullosa (RDEB). An additional 10 patients have been prescreened for this study.
- Additional patients treated in dose cohort 3 of the Transpher A study and the Transpher B study.
- Presented positive interim data from the Transpher A study of ABO-102 at WORLD*Symposium*™ demonstrating improved neurocognitive skills 18 months to two years post-treatment in MPS IIIA patients younger than 30 months, sustained, dose-related biomarker improvements, and a favorable safety profile.

- Presented positive interim data from the Transpher B study of ABO-101 at WORLD*Symposium*™ demonstrating initial improvement in multiple disease-specific biomarkers, denoting clear biologic effects, and a favorable safety profile among MPS IIIB patients.
- Updated interim results from the Transpher A and Transpher B studies to be presented during the American Society of Gene & Cell Therapy (ASGCT) 23rd Annual Meeting, which will take place online May 12-15, 2020.
- Two U.S. patents issued for adeno-associated virus (AAV) capsids exclusively licensed by Abeona from the University of North Carolina ("UNC"), generated using UNC's AIM™ vector platform.
- Announced key appointments of industry leaders to its Board of Directors in April. Dr. Brian J. G. Pereira was appointed as Executive Chairman and Ms. Shawn Tomasello as an Independent Board Member. Dr. Pereira is a seasoned biopharmaceutical and healthcare leader with experience in financing and growing companies, including the clinical development and commercialization of innovative drug products. Ms. Tomasello has substantial commercial and strategic experience, including serving as Chief Commercial Officer at cell therapy pioneer Kite Pharma, which was acquired by Gilead Sciences.
- Strengthened its leadership team with the appointments of Gregory Gin as Vice President, Investor Relations and Dr. Dan Rudin as Vice President, Clinical Development, focusing on the EB-101 program. Mr. Gin brings more than 25 years of investor relations, communications, and capital markets experience with small- and mid-cap biotechnology and specialty pharmaceutical companies developing novel treatments for orphan diseases and areas of high unmet medical need. Dr. Rudin has substantial research and development experience gained in industry and academia with focus on rare diseases, including lysosomal storage diseases. He has led several programs through the lifecycle of clinical development supporting multiple product approvals.

Dr. Siffert continued, "We look forward to working with our new Executive Chairman, Dr. Brian Pereira and Independent Board Member, Ms. Shawn Tomasello. Both bring invaluable experience guiding biotech companies from clinical development through commercial launch. In addition, with the appointments of Greg and Dan, we have strengthened our leadership in investor relations and clinical development, respectively."

COVID-19 Impact Mitigation

The ongoing COVID-19 pandemic has caused meaningful disruptions to the global healthcare system, including the conduct of clinical trials as healthcare institutions shift their focus and resources to treating COVID-19 patients. In response to the unprecedented challenges related to the COVID-19 pandemic, Abeona has taken several measures to protect and support the health of its employees and their families, healthcare partners and patients participating in its clinical trials. At the same time, the Company has implemented measures to maintain continuity of its operations and to preserve financial flexibility for the future.

First Quarter Financial Results

Cash, cash equivalents and marketable securities as of March 31, 2020, were $116 million compared to $129 million as of December 31, 2019. The decrease in cash of $13 million was

driven by R&D expenses across our programs along with supporting administrative costs.

The net loss was $0.52 per share for the first quarter of 2020, compared to $0.39 per share in the comparable period in 2019. The increase in the net loss per share results primarily from the non-cash impairment charge on the termination of the REGENXBIO license of $32.9 million, or $0.36 per share.

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. The Company's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa, as well as ABO-102 and ABO-101, novel AAV-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively. The Company's portfolio of AAV-based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates, including Regenerative Medicine Advanced Therapy designation for two candidates (EB-101 and ABO-102). www.abeonatherapeutics.com

Forward-Looking Statements
This press release contains certain statements that are forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. These statements include statements about the Company's clinical trials and its products and product candidates, future regulatory interactions with regulatory authorities, as well as the Company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," "will," "believe," "estimate," "expect," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, numerous risks and uncertainties, including but not limited to the potential impacts of the COVID-19 pandemic on our business, operations, and financial condition, continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies, the impact of competition, the ability to secure licenses for any technology that may be necessary to commercialize our products, the ability to achieve or obtain necessary regulatory approvals, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and quarterly reports on Form 10-Q and other periodic reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to update them to reflect events or circumstances occurring after the date of this presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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Source: Abeona Therapeutics Inc.