May 26, 2021

Abeona Therapeutics Issues Letter to Stockholders

NEW YORK and CLEVELAND, May 26, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today issued the following letter to stockholders in conjunction with the 2021 Abeona Therapeutics Annual Meeting of Stockholders to be held today, May 26, 2021:

Dear Fellow Stockholders,

We at Abeona remain committed to bringing urgently needed treatments to patients with recessive dystrophic epidermolysis bullosa (RDEB), and Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB, respectively).

I am incredibly proud of the tremendous progress Abeona achieved in 2020 and to start 2021, which is a testament to the talent, unwavering commitment, and ability to execute of our employees, who have worked tirelessly to achieve important milestones even in the face of the COVID-19 pandemic. I want to especially thank the patients, caregivers, investigators, and patient advocacy partners who have continued in their steadfast dedication to our clinical trials despite the challenges of the pandemic.

Importantly, we have recently strengthened our leadership team and board of directors by adding relevant experience to support our focus on driving future growth, enhancing corporate governance, and creating shareholder value. In addition to my appointment as chief executive officer and board member, we appointed four new independent members to our board of directors, and yesterday we announced the key hire of Senior Vice President, Head of Research & Clinical Development. Our strengthened board and management team represent a group of highly qualified and diverse executives who bring fresh perspectives, relevant operational expertise with life sciences companies and leadership experience to Abeona.

EB-101 (Autologous, Gene-Corrected Cell Therapy)
Our most significant clinical milestone of 2020 was the initiation of the Phase 3 VIITAL™ study of EB-101, our gene-corrected cell therapy for patients afflicted with RDEB. EB-101 has the potential to be a viable treatment for all RDEB wounds, and in particular, the larger and/or chronic wounds that carry the highest burden, including the need for frequent dressing changes, pain, pruritus, and risks of developing infections or skin cancer.

EB-101 has already shown durable healing of large, chronic RDEB wounds and associated pain reduction in a phase 1/2 clinical trial. Data from a phase 1/2a clinical trial presented at the 2020 Society for Pediatric Dermatology Annual Meeting showed that wound healing of 50% or greater following EB-101 treatment in patients with RDEB was associated with no pain at treated sites at three-, four- and five-years after treatment, compared with presence

of pain in 53% of these wound sites before treatment.

The VIITAL™ study is being conducted with investigators at Stanford University Medical Center, a world-renowned epidermolysis bullosa (EB) treatment center. We expect to activate a second site in mid-2021 at a large academic EB medical center in the northeastern U.S. to provide a convenient treatment location for study participants on the East Coast, and to expand experience of health care professionals with our EB-101 product to support a potential commercial launch. We expect to complete patient enrollment in the VIITAL™ study in 2021 and anticipate top-line data in mid-2022 followed by a Biologics License Application (BLA) filing if the data is positive.

In addition to our clinical progress, we also had positive dialogue with the U.S. Food and Drug Administration (FDA) around the endpoints for the VIITAL™ study. I am happy to share with you that we had a successful Type B meeting with the FDA in late 2020. The result of the meeting was very positive, and our interaction resulted in alignment with the FDA on our proposed co-primary endpoints of a) proportion of EB-101 treated wounds with >50% healing from baseline at 24 weeks and b) improvement in pain at 24 weeks as assessed by the Wong-Baker pain scale at time of dressing change versus an untreated control wound. We believe these are clinically meaningful endpoints, and we are thankful to the FDA for their collaboration.

ABO-102 and ABO-101 (AAV-based Gene Therapies)
Turning to our investigational adeno-associated virus or AAV-based gene therapy clinical candidates, we are developing ABO-102 for the treatment of MPS IIIA and ABO-101 for the treatment of MPS IIIB. Children born with MPS IIIA and MPS IIIB experience, starting at a very young age, progressive neurodevelopmental decline and loss of motor function that is life-threatening.

We were very excited to share new interim data from the Transpher A study at the 17th Annual WORLDSymposium™ in February 2021, which continues to show the potential for ABO-102 to help preserve neurocognitive development in patients with MPS IIIA when they are treated at a young age. In the Transpher A study, we demonstrated that in the three youngest patients treated with ABO-102 at ages 27 months, 19 months, and 12 months, neurocognitive development was preserved within the normal range of non-afflicted children at 2.5 to 3 years after treatment with ABO-102. It is important to note that these three patients were between the ages of 3.5 years to 5+ years at their latest respective follow-up visit. This is a critical timepoint based on the natural history of disease progression since almost all patients with MPS IIIA have already started to experience neurocognitive decline without viable treatment options. We believe ABO-102 has the potential to be a life-altering treatment option for children with MPS IIIA.

We look forward to meeting with the FDA in June 2021 to discuss whether the Transpher A dataset could form the basis for a BLA submission with natural history data as a comparator to assess efficacy for ABO-102. Recall that we previously met with the EMA in 2020 and received feedback on a viable path toward a marketing authorization for ABO-102 in the EU. We plan to evaluate next steps with the EMA after the FDA meeting and after we refine our plan forward based on the FDA's feedback.

We were also pleased to announce new results from the Transpher B study at the WORLDSymposium™ in February 2021 that provided additional insights into ABO-101's

biologic effect in patients with MPS IIIB. The updated results showed that treatment with ABO-101 was associated with dose-dependent and sustained improvement in disease-specific biomarkers, denoting clear biologic effects. We look forward to continued follow-up to assess ABO-101's potential to preserve neurocognitive development in patients with MPS IIIB beginning later this year.

Gene and Cell Therapy Manufacturing
We have a Current Good Manufacturing Practice (cGMP) facility located in Cleveland, Ohio, which has enabled us to enhance supply chain control, establish quality control testing, increase supply capacity, reduce production costs, and increase manufacturing efficiency for clinical trials and commercial grade product supply in the event our therapies receive marketing approval.

Since the beginning of 2020, we have continued to advance our in-house manufacturing capabilities for EB-101. A key component of the EB-101 drug product manufacturing process is the retroviral vector that delivers the functional copy of the Collagen VII Alpha 1 gene into skin graft cells. In 2020, we internally developed the cGMP manufacturing process for the retroviral vector and have produced three cGMP lots for analytical and clinical comparability. In addition, we developed a cGMP master cell bank and a working cell bank to support the cGMP production of the retroviral vector. In 2020, we also initiated AAV process development activities to enable in-house manufacturing of commercial supply of ABO-102 and ABO-101.

Strategic Partnerships
As part of our strategy to focus resources on our key clinical programs, we entered into two strategic partnerships in 2020 with Taysha Gene Therapies for ABO-202 for CLN1 disease (also known as infantile Batten disease) and for an AAV-based gene therapy for Rett syndrome. The partnerships are intended to facilitate development by Taysha of earlier stage, non-core assets for patients with high unmet need, while providing the opportunity for Abeona to share in the future success of these programs. The partnerships also provided near-term proceeds that can support the ongoing development of our core clinical programs.

2021 Strategic Goals
Looking ahead to the balance of 2021, we are focused on completing enrollment in the EB-101 Phase 3 pivotal VIITAL™ study, gaining clarity on a regulatory path for ABO-102 in MPS IIIA, producing the first lot of Abeona-produced clinical grade product for ABO-102, and reporting additional neurocognitive data from the Transpher A study and additional clinical updates from the Transpher B study. As part of our strategy to expand our clinical pipeline, we are conducting preclinical research assessing AAV capsids in six undisclosed eye disorders, and we are advancing toward IND-enabling studies in 2022.

In conclusion, I would like to thank our stockholders for supporting our efforts and sharing in our lofty goals. Our accomplishments over the past year and into our fast start of 2021 fill us with great optimism for Abeona's future, and give us positive momentum as we focus on execution, achieving key milestones, and continuing to propel our clinical and early-stage programs forward to patients.

Sincerely,

Michael Amoroso

Chief Executive Officer
Abeona Therapeutics Inc.

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. Abeona's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, 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, in Phase 1/2 development. The Company's portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical needs. Abeona's novel, next-generation AIM™ capsids have shown potential to improve tropism profiles for a variety of devastating diseases. Abeona's fully functional, gene and cell therapy cGMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and commercial production of AAV-based gene therapies. For more information, visit 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. 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 disclosed in the Company's most recent Annual Report on Form 10-K and subsequent quarterly reports on Form 10-Q and other periodic reports filed 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 press release, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

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