

December 22, 2021



Abeona Therapeutics Issues Letter to Shareholders

NEW YORK and CLEVELAND, Dec. 22, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today issued the following letter to shareholders.

Dear fellow shareholders,

We have never been closer to fulfilling our mission of providing novel gene and cell therapies to patients who currently have no approved treatment options as we continue to advance the EB-101 and ABO-102 pivotal studies toward completion to support two U.S. Biologics License Application (BLA) submissions. At the same time, we are continuing to make steady progress with other clinical and preclinical programs.

As we end the calendar year 2021, it is important to reflect on all that we at Abeona have achieved over the last 12 months, and it is even more crucial to look ahead at the opportunities awaiting us in 2022 and beyond. Here is a recap of our recent accomplishments, which have positioned Abeona on course for a potentially transformative year ahead.

EB-101 (Autologous, Gene-Corrected Cell Therapy) for RDEB

We are close to completing patient enrollment for the pivotal Phase 3 VIITAL™ study for our investigational product for recessive dystrophic epidermolysis bullosa (RDEB), EB-101. Under the study protocol, the enrollment target is approximately 35 randomized large chronic wounds and we have treated 80% of that target to date. To increase patient enrollment, we activated UMass Memorial Medical Center as the second clinical trial site in the VIITAL™ study. We remain confident in our ability to complete enrollment in the first quarter 2022, positioning us for topline data in third quarter 2022. We are optimistic about EB-101's potential based on updated Phase 1/2a results presented at various medical congresses. On the manufacturing front, we have continued to prepare our cGMP commercial facility in Cleveland, Ohio for manufacturing EB-101 drug product to support our planned BLA filing. EB-101 study drug product for all our VIITAL study participants has been manufactured at our Cleveland facility and we have now completed submission of Module 3 for chemistry, manufacturing and control describing the in-house production of both retroviral vector and the final drug product to the Investigational New Drug application (IND).

ABO-102 (AAV-based Gene Therapy) for MPS IIIA

We have continued to gain clarity on a regulatory path for investigational ABO-102 as a potential treatment for Sanfilippo syndrome type A (MPS IIIA), having successfully completed a Type B meeting with the FDA where we aligned on the primary endpoint for

registration. We are excited about important clinical data updates from the pivotal Transpher A study presented at multiple medical meetings and congresses on disease-specific biomarkers, preservation of neurocognitive development and anatomical development using brain MRI in children treated early in age at the therapeutic dose. We believe that the totality of data will provide a holistic view of the treatment effect of ABO-102 and contribute to a robust regulatory package in a disease where there are no approved treatments and other investigational products are at a very early stage. In a major step toward becoming non CDMO-dependent, we have completed manufacturing of six GMP lots of ABO-102. Additionally, in preparation for manufacturing commercial supply of ABO-102, we initiated construction of a 12,000 square foot commercial AAV manufacturing facility at our Cleveland site, which will also have the capacity to support additional AAV programs, including our pre-clinical ocular programs.

ABO-101 (AAV-based Gene Therapy) for MPS IIIB

We closed enrollment in our ABO-101 Transpher B study in Sanfilippo syndrome type B (MPS IIIB), and we are now following patients for safety and efficacy. We look forward to seeing two-year neurocognitive data and will subsequently determine next steps for the program.

Preclinical Pipeline

While our clinical programs are currently focused on rare diseases, we intend to address larger areas of unmet medical need in the future, and our preclinical programs are investigating novel AAV capsids in five undisclosed ophthalmic conditions each with estimated U.S. prevalence ranging from 5,000 to 15,000 patients. We have shared data from non-human primates that will help to determine optimal routes of administration and have made significant progress toward measuring efficacy in the preclinical setting. We have also generated appropriate mouse models, produced recombinant capsids, and started dosing mice in proof-of-concept studies that we hope will yield data beginning in mid-2022 to support pre-IND meetings with the FDA in the second half of 2022.

Well-positioned for 2022 Anticipated Milestones

With the settlement of the REGENXBIO dispute and the recent successful completion of a public offering of securities for approximately \$17.5 million in gross proceeds, we expect to begin 2022 with cash and financial resources of more than \$50 million (unaudited). We believe focusing on our milestones is the best way to create value for our shareholders, and our strengthened balance sheet is crucial in positioning us to further execute our operating plan and achieve important anticipated milestones. Our anticipated milestones in 2022 include:

EB-101

- Completion of patient enrollment for the EB-101 Phase 3 VIITAL™ study in the first quarter of 2022
- Top-line VIITAL™ study results in the third quarter of 2022

ABO-102

- Construct and operationalize the new AAV manufacturing facility in 2022
- Top-line results for MPS IIIA Transpher A study between the fourth quarter of 2022 and second quarter of 2023

ABO-101

- Two-year neurocognitive data from MPS IIIB Transpher B study in the second half of 2022

Preclinical Pipeline

- Generate proof-of-concept data assessing AAV capsids in multiple undisclosed eye indications beginning in the first half of 2022
- Pre-IND meeting with FDA in the second half of 2022

In summary, we have confidence in the potential transformative value that Abeona's cell and gene therapies can deliver to patients. We are focused on our road map and making progress through disciplined execution. Our current leadership team is as strong as ever, especially with important recent additions geared to enhance our BLA readiness. I would like to thank our shareholders for supporting our efforts and sharing in our mission to bring gene and cell therapies to people impacted by serious diseases.

Sincerely,

Vish Seshadri, Ph.D., M.B.A.
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 investigational autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel investigational 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 development portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical need. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases. Abeona's fully integrated gene and cell therapy cGMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and planned 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, our ability to execute our operating plan and achieve important anticipated milestones, 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.

Investor and Media Contact:

Greg Gin

VP, Investor Relations and Corporate Communications

Abeona Therapeutics

+1 (646) 813-4709

ggin@abeonatherapeutics.com



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