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# Abeona Therapeutics Announces Presentation on Pivotal Transpher A Study of ABO-102 in MPS IIIA at the 14th ICIEM Conference

NEW YORK and CLEVELAND, Nov. 17, 2021 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that information about the company's ABO-102 pivotal Transpher A study in Sanfilippo syndrome type A (MPS IIIA) will be presented at the 14<sup>th</sup> International Congress of Inborn Errors of Metabolism (ICIEM). The meeting will take place virtually and at the Hilton Sydney in Sydney, Australia on November 21-23, 2021.

The oral presentation titled, "Interim results of Transpher A, a multicenter, single-dose, phase 1/2 clinical trial of ABO-102 gene therapy for Sanfilippo syndrome type A (mucopolysaccharidosis IIIA)," will be presented by Dr. Nicholas Smith, Department of Neurology and Clinical Neurophysiology, Women's and Children's Hospital. The presentation includes new data characterizing the positive correlational relationship between the Bayley Scales of Infant and Toddler Development (BSITD) and the Mullen Scales of Early Learning (MSEL), standardized measures of non-verbal cognitive functioning for young children, and previously announced efficacy and safety data on Abeona's AAV-based gene therapy, ABO-102. The presentation will take place during a session on Tuesday, November 23, 2021 from 11:45 am-1:15 pm Australian Eastern Daylight Time (AEDT).

## **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; novel investigational AAV-based gene therapies ABO-102 in the pivotal Transpher A study for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 in the Phase 1/2 Transpher B study for Sanfilippo syndrome type B (MPS IIIB). 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](http://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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