November 17, 2015

Abeona Therapeutics Reports Financial Results and Highlights for Third Quarter 2015

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 11/17/15 -- Abeona Therapeutics Inc. (NASDAQ: ABEO), a biopharmaceutical company focused on developing and delivering gene therapy and plasma-based products for severe and life-threatening rare diseases, announced its financial results for the third quarter ended September 30, 2015.

"The Company continued to make progress on its stated plans to develop and deliver its gene therapy and plasma-based products for severe, life-threatening rare diseases," stated Steven H. Rouhandeh, Executive Chairman. "Additionally, we took steps to strengthen our balance sheet with further investments from key institutional investors and insiders, and strengthened our intellectual property position through an additional license agreement and internal programs."

Tim Miller, Ph.D., Abeona's President and CEO, stated, "During the third quarter, we continued to build out our management team with key clinical and regulatory hires, advanced our novel AAV gene therapies for Sanfilippo Syndrome A and B towards human clinical trials, and progressed with our academic collaborators our novel gene therapies for the treatment of juvenile Batten disease and Fanconi anemia. Our SDF process scale up is proceeding to plan, and we are engaged in the regulatory process to confirm the clinical plans for our proprietary alpha-1 protease inhibitor, SDF Alpha. We look forward to providing updates of our progress."

Financial and Corporate Highlights

- At September 30, 2015, Abeona's cash and cash equivalents totaled $43.3 million, compared to $30.4 million at June 30, 2015. Quarterly net loss was $6.0 million and $12.2 million, or $0.19 and $0.47 per share, for the three months and nine months ended September 30, 2015 respectively. Net cash used in operating activities in the nine months ended September 30, 2015, was $7.5 million, adjusted for non-cash stock-based compensation expenses.
- The Company has made several key additional hires to further build out its management team. Key hires include Kaye Spratt, Ph.D, Vice-President of Regulatory and Quality Assurance; Allan Valmonte, MBA, Director of Clinical Operations, and Michelle Berg, Vice-President, Patient Advocacy.
    - *S. Kaye Spratt, Ph.D.*, Vice President of Regulatory and Quality: Dr. Spratt has experience defining nonclinical development plans and strategies to US regulatory authorities. With more than 20 years of scientific research, product development and translational expertise, Dr. Spratt was Director of Preclinical and Quality Control at Sangamo BioSciences with increasing responsibilities from

1997 until 2015 and was responsible for leading a team of scientists developing novel cell and gene therapy products utilizing the Sangamo Zinc Finger Therapeutic strategy platform. Prior to joining Sangamo, Dr. Spratt was a Senior Scientist and Project Leader at Somatix Therapy Corporation. Dr. Spratt has over 40 publications in gene therapy. Dr. Spratt earned her Ph.D. in Microbiology with a focus in molecular biology in infectious diseases from Meharry Medical College. She also holds a B.S. in Biology from Langston University.
    - *Michelle Berg,* Vice President, Patient Advocacy: Prior to joining Abeona, she was Vice President, Client Relations at Aldevron, LLC, a leading global contract manufacturing organization specializing in biological reagents for R&D through clinical trial use. She brings over 17 years of experience in the gene and cell therapy, vaccine, gene editing and molecular diagnostic fields. Ms. Berg has a B.S. in Biotechnology from North Dakota State University.
    - *Allan Valmonte,* Director, Clinical Operations: Allan has directed domestic and international clinical Phase 1 - 4 programs in multiple indications. Through his career, Mr. Valmonte held clinical operations positions at Hyperion, Cell Genesys, Abgenix, Millenium Pharmaceuticals, Oxigene, Galena, and Xoma. He holds an MBA from St. Joseph's University and a B.A. from California State University.
- On July 7, 2015, the Company announced preliminary results of its SDF plasma protein programs, confirming that multiple batches of its two-step salt precipitation process yields resultant fractions with significantly enhanced levels of alpha-1 protease inhibitor and immunoglobulins (IVIG) relative to the industry-standard Cohn process.
- On July 31, 2015 the Company closed a $15.5 million direct placement of registered common stock with institutional investors, including Soros Fund Management and Perceptive Life Science Fund, and two members of the Board of Directors. The financing is comprised of 2.83 million shares of common stock at a price of $5.50 per share.
- On October 6, 2015, the Company announced a license agreement with Stanford University for an AAV (adeno-associated virus) delivery vector for the treatment of Fanconi anemia and rare blood disease platform. This license augments a previously announced license agreement with the University of Minnesota for ABO-301 (AAV-FANCC) to treat patients with Fanconi anemia (FA) disorder and other rare blood diseases
- On July 1, 2015 Abeona announced additional financing of $4.6 million through warrant exercises of our publicly traded warrants (NASDAQ: ABEOW).

About Abeona: Abeona Therapeutics, Inc. develops and delivers gene therapy and plasma-based products for severe and life-threatening rare diseases. Abeona's lead programs are AB0-101 (AAV9 NAGLU) and ABO-102 (scAAV9 SGSH), adeno-associated virus (AAV)-based gene therapies for Sanfilippo syndrome (MPS IIIB and IIIA) in collaboration with patient advocate groups, researchers and clinicians. We are also developing ABO-201 (scAAV9 CLN3) gene therapy for juvenile Batten disease (JBD); and ABO-301 (AAV FANCC) for Fanconi anemia (FA) disorder using a novel CRISPR/Cas9-based gene editing approach to gene therapy program for rare blood diseases. In addition, we are also developing rare plasma protein therapies including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD using our proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com.

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 that involve risks and uncertainties. These statements include, without limitation, our plans for the use of proceeds of the financings, our plans to begin enrolling patients in clinical trials for the treatment of Sanfilippo syndrome, development and internationalization of other clinical programs, management plans for the Company, and general business outlook. These statements are subject to numerous risks and uncertainties, including but not limited to continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the impact of competition; the ability to develop our products and technologies; the ability to achieve or obtain necessary regulatory approvals; the impact of changes in the financial markets and global economic conditions; and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

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