

August 17, 2015



# Abeona Therapeutics Announces Recent Highlights and Second Quarter 2015 Conference Call

NEW YORK, NY -- (Marketwired) -- 08/17/15 -- 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, will provide a business update for investors on a conference call, Thursday, August, 20<sup>th</sup>, at 10:00 AM (ET).

Tim Miller Ph.D., President and CEO and Jeffrey Davis, Chief Operating Officer, together with other executives, will conduct the call. Interested parties are invited to participate in the call by dialing: Toll Free Number: 877-269-7756 or International Toll Number: 201-689-7817. The call will consist of an overview of the Company's first quarter Form 10-Q, and a discussion of business highlights and activities of the recently announced transactions. Immediately thereafter, there will be a question and answer period open to all registered analysts and investors. A recorded replay of the call will be available at the following toll free number: 877-660-6853 or international toll number 201-612-7415, and choose Conference ID 13617494.

"The second quarter of 2015 was very significant in the development of Abeona Therapeutics as a leader in the rare disease, gene therapy and plasma protein space," stated Steven H. Rouhandeh, Executive Chairman. "In addition to changing our name to Abeona Therapeutics to reflect our rare disease focus, we successfully completed multiple financing transactions with leading healthcare institutional investors that strengthened the balance sheet and provided significant runway to get our programs into the clinic over the coming quarters."

"In the quarter, in addition to advancing preclinical development of Abeona's Therapeutics leading gene therapy programs in Sanfilippo Syndromes type A and B, we in-licensed a complementary AAV gene therapy program in juvenile Batten disease, and in-licensed an exciting new technology platform in CRISPR/Cas9 gene editing in rare blood diseases and its lead product candidate in Fanconi anemia. We look forward to keeping you apprised of our progress," stated Tim Miller, President & CEO.

## ***Abeona Recent Highlights***

- 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 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 1, 2015 Abeona announced additional financing of \$4.6 million through warrant exercises of our publicly traded warrants (NASDAQ: ABEOW).
- On June 19, 2015 The Company announced we had changed our name to Abeona Therapeutics Inc. from PlasmaTech Biopharmaceuticals, Inc.
- On June 15, 2015, Abeona licensed exclusive worldwide rights to an AAV gene therapy and intellectual property from the University of Minnesota to treat patients with Fanconi anemia (FA) disorder and other rare blood diseases using the CRISPR/Cas9 technology platform for undisclosed terms.
- On June 8, 2015, The Company licensed exclusive worldwide rights to an AAV gene therapy for the treatment of juvenile Batten disease (JBD) from UNeMed Corporation, the technology transfer and commercialization office for the University of Nebraska Medical Center in Omaha, Nebraska for undisclosed terms.
- On May 11, 2015 The Company closed a \$10 million private placement of common stock with an accredited institutional healthcare investor.
- On May 5, 2015, the Company announced it had signed definitive agreements to acquire Abeona Therapeutics LLC, a leader in systemic AAV9 gene therapies for Sanfilippo Syndrome A and B. The Merger closed on May 15, 2015.
- On April 23, 2015 we closed a \$7 million private placement of common stock with two leading institutional healthcare investors.

**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 ABO-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, anticipated to commence clinical trials in 2015. We are also developing ABO-201 (scAAV9 CLN3) gene therapy for juvenile Batten disease (JBD); and ABO-301 (AAV LK19 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](http://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 financing, 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, the anticipated closing of the transaction, 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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