

November 10, 2016



# Abeona Therapeutics Announces Third Quarter 2016 Financial Results and Recent Clinical Highlights

**Investor Conference Call to be held Friday, November 18 at 10:30 am ET**

NEW YORK, NY and CLEVELAND, OH -- (Marketwired) -- 11/10/16 -- Abeona Therapeutics Inc. (NASDAQ: ABEO) a clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, today announced financial results for the third quarter. The Company will provide investors an update on recent and ongoing business activities and an overview of its 3Q16 financials on, Friday, November 18 at 10:30 am (Eastern). Interested parties are invited to participate in the call by dialing 877-269-7756 (toll free domestic) or 201-689-7817 (international).

"During the third quarter of 2016, we have made significant progress advancing our clinical gene therapy programs addressing serious genetic diseases and showcased the strengths of our clinical and regulatory capabilities. Notably, we demonstrated promising safety and bio-potency in the low-dose cohort for ABO-102 and received Fast Track designation for the program and Orphan Drug Designation in the European Union where we are expanding clinical studies," stated Timothy J. Miller, Ph.D. "In addition, our strategic work in adding the AIM™ vector platform, an AAV capsid portfolio from University of North Carolina at Chapel Hill, positions us well for advancing next generation proprietary AAV gene therapies."

## ***Third Quarter Summary Financial Results:***

- **Cash position:** Cash, cash equivalents and marketable securities as of September 30, 2016 were \$31.2 million, compared to \$34.3 million as of June 30, 2016. Net cash used in operating activities in the Nine Months Ended September 30, 2016 was \$9.6 million as compared to \$7.5 million in the same period in 2015, an increase of \$2.1 million.
- **Offering:** On November 1, 2016, we closed an underwritten public offering of 6,000,000 shares of common stock, at a public offering price of \$7.00 per share. The gross proceeds to the Company were \$42,000,000 million, before deducting the underwriting discounts and commissions and estimated offering expenses payable by the Company.
- **Revenues:** Revenues were \$184 thousand for the third quarter of 2016, compared to \$285 thousand in the third quarter of 2015. Revenues consisted of a combination of royalties from marketed products, primarily MuGard®, and recognition of deferred revenues related to upfront payments from early license agreements.
- **Loss per share:** Loss per share was \$0.08 for the third quarter of 2016, compared to a loss per share of \$0.19 in the comparable period in 2015.

"The third fiscal quarter of 2016 brought significant advancements in our goal of building a

strong leadership position in the development of innovative therapies for rare diseases," stated Steven H. Rouhandeh, Executive Chairman. "We are excited about the initial biopotency signals seen in our ABO-102 Phase 1/2 clinical trial for Sanfilippo syndrome Type A (MPS IIIA), initiation of the EB-101 Phase 2 study for epidermolysis bullosa (EB), and the completion of a \$42 million financing which provides us with ample runway to continue creating significant value for the Company and its shareholders."

### ***Abeona Recent Highlights:***

- November 1, 2016: Abeona announced closing of its \$42 million underwritten offering of common stock
- October 25, 2016: Abeona announced receipt of Fast Track designation from the FDA for ABO-102
- October 18, 2016: [Abeona received Orphan Drug Designation in the European Union for ABO-102 gene therapy in Sanfilippo syndrome Type A](#)
- October 14, 2016: Abeona presented top-line data of low-dose cohort for ABO-102 in Phase 1/2 clinical trial for MPS IIIA patients at Orphan Drugs and Rare Disease Congress October 19-20th in London, UK
  - ABO-102 reduced GAG (heparan sulfate) in urine 57.6% +/- 8.2%
  - ABO-102 reduced GAG (heparan sulfate) in the CSF 25.6% +/- 0.8%
  - Reduction in liver volume of 17.7% +/- 1.9%
  - Reduction in spleen volume of 17.6% +/- 7.1%
- October 7, 2016: Abeona announced JAMA publication of preclinical data supporting clinical translation of Juvenile Batten Disease gene therapy
- October 5, 2016: Abeona announced Data Safety Monitoring Board approved ABO-102 dose escalation for second cohort in a Phase 1/2 Clinical Trial for Sanfilippo syndrome Type A
- September 26, 2016: Abeona enrolled first patient in Phase 2 for EB-101, gene therapy clinical trial for epidermolysis bullosa
- September 21, 2016: Abeona announced the exclusive worldwide license of the AIM™ AAV capsid portfolio for next generation gene therapies from University of North Carolina at Chapel Hill
- September 8, 2016: Abeona enrolled 5<sup>th</sup> patient in Phase 1 gene therapy clinical trial for epidermolysis bullosa

**About Abeona:** Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and plasma-based therapies for life-threatening rare genetic diseases. Abeona's lead programs are ABO- 102 (AAV-SGSH) and ABO-101 (AAV-NAGLU), adeno-associated virus (AAV) based gene therapies for Sanfilippo syndrome (MPS IIIA and IIIB, respectively). Abeona is also developing EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), EB-201 for epidermolysis bullosa (EB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (INCL), and ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona has a plasma-based protein therapy pipeline, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD, using its 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 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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