

August 9, 2018



Abeona Therapeutics Reports Second Quarter 2018 Financial Results and Business Highlights

Investor Conference Call on Friday, August 10th at 10:00 am ET

NEW YORK and CLEVELAND, Aug. 09, 2018 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel cell and gene therapies for life-threatening rare genetic diseases, today announced financial results for the second quarter of 2018. The Company will host a call to update investors on recent clinical developments and quarter financial results on Friday, August 10th at 10:00 am (Eastern). Interested parties are invited to participate in the call by dialing 877-407-9210 (toll-free domestic) or 201-689-8049 (International) or via webcast <http://www.investorcalendar.com/event/35041>.

“The second quarter was marked by continued progress in our clinical, pre-clinical and corporate initiatives,” stated Carsten Thiel, Ph.D., Abeona’s CEO. “The strength of our lead programs, which continue to demonstrate robust and durable clinical effects, is underscored by the achievement of additional regulatory designations and the recent appointment of key executives and Board members. Notably, the opening of our in-house GMP manufacturing facility in Cleveland reinforces Abeona’s ongoing commitment to transforming patients’ lives and bolsters our position for commercial readiness.”

2nd Quarter Summary Financial Results:

- Cash position: Cash, cash equivalents and marketable securities as of June 30, 2018 were \$120 million, compared to \$132 million as of March 31, 2018.
- Revenues: Revenues were \$819 thousand for the second quarter of 2018, compared to \$217 thousand in the second quarter of 2017. A portion of the increased quarterly revenues consisted of the recognition of Foundation grants that were announced during the fourth quarter of 2017. A portion of the grants were received in the second quarter of 2018, and the amount recognized is matched against corresponding expenditures for drug manufacture and clinical readiness. Additional revenues consisted of royalties from marketed products, primarily MuGard®.
- Loss per share: Loss per share was \$0.25 for the second quarter of 2018, compared to a loss per share of \$0.21 in the comparable period in 2017.

Abeona Recent Highlights:

- July 26, 2018: Announced Leadership Appointments with Max Colao as Chief Commercial Officer

- May 31, 2018: Announced Opening of Commercial Gene & Cell Therapy Manufacturing Facility in Ohio
- May 18, 2018: Reported Update on MPS IIIA Gene Therapy Trial at the 21st Annual ASGCT Meeting
 - ABO-102 18-month efficacy and safety data continue to demonstrate time- and dose-dependent reductions in underlying disease pathology, including decreased CSF and urine GAGs and improved liver volumes
 - 11 subjects enrolled through > 4,200 days cumulative follow up
- May 17, 2018: Reported Update from EB-101 Gene Therapy in Epidermolysis Bullosa at 21st Annual ASGCT Meeting
 - Phase 1/2 study update results confirm EB-101 is safe and well-tolerated, with durable efficacy throughout various timepoints post-administration
 - Collagen VII (C7) expression: C7 and morphologically normal NC2 reactive anchoring fibrils were observed as early as one month in EB-101 treated wounds and have remained up to three years post-administration.
 - Wound healing, defined as >50% closure after EB-101 administration, was observed in:
 - 100% (42/42 treated wounds, n=7 subjects) at 3 months;
 - 90% (38/42 treated wounds, n=7 subjects) at 6 months;
 - 67% (24/36 treated wounds, n=6 subjects) at 12 months;
 - 88% (21/24 treated wounds, n=4 subjects) at 24 months; and
 - 100% (6/6 treated wounds, n=1 subject) at 36 months post-administration.
- May 14, 2018: Announced Appointment of Stefano Buono and Richard Van Duyne to its Board of Directors
- April 23, 2018: Announced FDA Grants RMAT Designation to ABO-102 Gene Therapy in MPS IIIA
- April 20, 2018: Announced EMA Grants Orphan Drug Designation in the European Union for ABO-202 Gene Therapy Program in Batten Disease
- April 2, 2018: Announced Appointment of Carsten Thiel, Ph.D., as Chief Executive Officer

“As we advance towards becoming a key player in the development of novel breakthrough gene and cell therapies for rare genetic diseases, 2018 continues to be a year of execution for Abeona,” stated Steven H. Rouhandeh, Abeona’s Executive Chairman.

About Abeona: Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing cell and gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-102 (AAV-SGSH), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type A (MPS IIIA) and ABO-101 (AAV-NAGLU), an adeno-associated virus (AAV) based gene therapy for Sanfilippo syndrome type B (MPS IIIB). Abeona is also developing ABO-201 (AAV-CLN3) gene therapy for CLN3 disease, ABO-202 (AAV-CLN1) for treatment of CLN1 disease, EB-201 for epidermolysis bullosa (EB), 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 is developing a proprietary vector platform,

AIM™, for next generation product candidates. For more information, visit www.abeonatherapeutics.com.

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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. These statements include statements regarding our advancement towards becoming a key player in the development of novel breakthrough gene and cell therapies for rare genetic diseases and that 2018 continues to be a year of execution. We have attempted to identify forward looking statements by such terminology as “may,” “will,” “anticipate,” “believe,” “estimate,” “expect,” “intend,” 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 continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, 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; our belief that initial signals of biopotency and clinical activity, which suggest that ABO-102 successfully reached target tissues throughout the body, including the central nervous system and the increased reductions in CNS GAG support our approach for intravenous delivery for subjects with Sanfilippo syndromes, risks associated with data analysis and reporting, and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and quarterly reports on Form 10-Q 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.



Source: Abeona Therapeutics Inc.