

## Abeona Therapeutics Announces Appointment of Juan Ruiz as Chief Medical Officer

NEW YORK and CLEVELAND, July 25, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq:ABEO), a leading clinical-stage biopharmaceutical company focused on developing novel gene therapies for life-threatening rare diseases, announced today that Juan Ruiz, M.D., Ph.D., MBA has joined the company as Chief Medical Officer. He will be responsible for leading all clinical development, medical affairs and related functions and will report directly to Timothy J. Miller, Ph.D., President and CEO.



Juan Ruiz, Chief Medical Officer, Abeona Therapeutics

A photo accompanying this announcement is available at <a href="https://www.globenewswire.com/NewsRoom/AttachmentNg/54ff84e1-4ff4-4192-a0eb-92df1dd362c2">https://www.globenewswire.com/NewsRoom/AttachmentNg/54ff84e1-4ff4-4192-a0eb-92df1dd362c2</a>

Dr. Juan Ruiz is well regarded for his extensive research in molecular biology and gene therapy. He has previously held global leadership positions at Lykera Biomed and Digna Biotech, where he spent over 13 years leading teams dedicated to developing gene therapy translational medicine programs. During his tenure as Chief Medical Officer of Digna Biotech, Dr. Ruiz's proven track record demonstrated a "start to finish" scope, from proof-of-concept studies and regulatory IND and IMPD submissions, to manufacturing, clinical trial design and execution, and partnering and licensing activities. In addition, he has expertise in

the manufacturing and quality control of the adenoviral vectors that were tested in his clinical trials.

"Juan is a proven leader in gene therapy clinical development, with both academic and industry experience, which will be invaluable as we advance our MPS III and RDEB programs into pivotal trials," said Timothy J. Miller, Ph.D., Abeona President and CEO. "His training in medicine and ability to translate scientific discoveries from concept to clinical trial will add depth to an experienced management team and advisors."

Dr. Ruiz has been instrumental in designing Patient Reported Outcomes (PRO) in the USA to assess skin outcome measures in patients with Systemic Sclerosis for use as primary endpoints in Phase IIb trials. Additional work includes multiple regulatory designation achievements and successful Scientific Advice and Protocol Assistance meetings with the FDA and EMA. He is the author of over forty peer-reviewed journal articles and book chapters and an inventor on multiple issued patents. Dr. Ruiz holds a M.D. with a specialty in Hepatology and a Ph.D. in Molecular Biology, both from the University of Navarra, and completed his post-doctoral fellowship at the University of Connecticut. He holds a MBA from IESE Business School, also at the University of Navarra.

"We stand at a transformative moment in gene therapy and the development of rare disease therapies," commented Dr. Ruiz. "Gene therapy has shown the ability to correct the underlying pathology associated with lysosomal storage diseases and skin disorders, and Abeona is harnessing the power of this technology for the potential to improve patient lives in multiple ongoing clinical trials. With its robust pipeline, novel AIM™ vector platform and strong gene therapy know-how, Abeona is well positioned to continue making its innovative mark in the rare disease industry."

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

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Source: Abeona Therapeutics Inc