



AskBio Partners with TPG and Vida Ventures

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Leading gene therapy company announces \$235 million investment to accelerate growth

Research Triangle Park, NC; Fort Worth, Texas; San Francisco; and Boston – April 11, 2019 – Asklepios BioPharmaceutical, Inc (AskBio), a developer and manufacturer of Adeno-Associated Virus (AAV) gene therapy therapeutics for underserved patient populations with rare and generally untreatable genetic disorders, announced today \$235 million in funding to augment and accelerate its business. TPG Capital and Vida Ventures are investing \$225 million for a minority stake in the company. As part of the transaction, AskBio's founders and board members are co-investing \$10 million. The investment provides AskBio with additional capital to help advance and expand clinical trials, enhance its manufacturing capabilities and capacity, and drive long-term growth. Additional terms of the transaction were not disclosed.

AskBio was founded in 2001 by Dr. Jude Samulski, Ph.D., the first scientist to clone AAV, along with Dr. Xiao Xiao, who was the first to develop a miniaturized dystrophin gene enabling the advancement of gene therapy for Duchenne Muscular Dystrophy, and company CEO Sheila Mikhail, an accomplished life sciences executive. Formed in the Gene Therapy Center at the University of North Carolina at Chapel Hill, AskBio offers proprietary AAV technology, best-in-class manufacturing capabilities, and a robust pipeline of in-house clinical programs. An early innovator in the space, the company holds over 500 patents in areas such as AAV production, chimeric vectors, and self-complementary DNA. AskBio's engineered viral vectors are able to target particular tissues, de-target other tissues, and minimize the effects of neutralizing antibodies.

"It's humbling to imagine that my Ph.D. project several decades ago has ushered in a new generation of transformative therapies that will impact so many lives," said Dr. Jude Samulski, Ph.D., President and Chief Scientific Officer of AskBio. "I am excited to partner with TPG and Vida to catalyze the next generation of gene therapy innovation and translate discovery into life changing therapeutics."

"With the funding from TPG and Vida, we will be able to accelerate our development of a broad range of transformative therapies for those affected by serious and oftentimes incurable genetic diseases," said Sheila Mikhail, CEO and Co-Founder of AskBio. "We look forward to advancing our approaches for repeat administration and avoidance of neutralizing antibodies into the clinic to maximize the number of patients who benefit from AAV therapies."

Gene therapy has been one of the most active areas of pharmaceutical R&D, and in recent years, AAV has emerged as an efficient, safe, and versatile vehicle for delivering corrective genes inside of the body. AskBio has successfully partnered with companies on two of its programs: Bamboo Therapeutics, a therapy targeting Duchenne Muscular Dystrophy and other rare diseases, and Chatham Therapeutics, which focuses on Hemophilia A and B. The platforms were sold to Pfizer and Baxter (programs now owned by Takeda), respectively.

"We are thrilled to partner with Jude, Sheila, and the entire AskBio team to support the development of therapies for patients suffering from historically untreatable diseases," said John Schilling, M.D., Partner at TPG Capital. "AskBio is a very special company, operating in one of the most innovative and strategically important sectors of the pharmaceutical industry today. In addition to its leading clinical programs in several therapeutic areas, AskBio has a broad base of technologies that we believe can support quantum leaps in therapies for many partners in the market. We are honored to have the opportunity to work with AskBio in the next leg of this journey."

AskBio recently advanced a therapeutic for Pompe Disease into the clinic based on the work by Dwight Koeberl, M.D., Ph.D., and Priya Kishnani, M.D., M.B.B.S., at Duke University. The company is also developing therapeutics for heart failure, Limb Girdle 2i, Myotonic Dystrophy, and several other muscular and neurological disorders. AskBio's AAV manufacturing process, Pro10, currently yields 1018 in 200-liter bioreactors and is recognized within the industry as the gold standard in scale manufacturing. Pro10 is used by Pfizer, Takeda, and Viralgen Vector Core SA. Recently, AskBio formed a partnership with Touchlight Genetics Ltd. for the commercialization of a safer, cheaper, and more efficient alternative to plasmid DNA.

"At Vida, we are driven to find innovative companies with disruptive technologies that have the potential to transform how we combat disease," said Fred Cohen, M.D., Ph.D., Co-Founder and Senior Managing Director at Vida Ventures. "Jude's acclaimed research in AAV and scientific vision for AskBio, along with the leadership of Sheila and the research expertise of the larger team, exemplify what's possible when passionate scientists and advocates demand more than conventional therapies."

Some of TPG Capital's past and current investments in the pharmaceutical industry include Par Pharmaceutical (now known as Endo International), Adare Pharmaceuticals, Amneal Pharmaceuticals, Aptalis, EnvisionRx, Quintiles, and IMS (together, now known as IQVIA). Last year, TPG Capital and Vida partnered to invest in Allogene Therapeutics, Inc (NASDAQ: ALLO). Vida's recent biotechnology investments include Kronos Bio, Praxis Precision Medicines, Oyster Point Pharma, and Pionyr Immunotherapeutics.

Piper Jaffray served as financial advisor to AskBio in connection with this transaction.

About AskBio

Asklepios BioPharmaceutical, Inc. (AskBio) is a privately held, gene therapy platform company dedicated to improving the lives of children and adults with rare genetic disorders. AskBio's gene therapy platform includes an industry-leading proprietary cell line manufacturing process known as Pro10 and an extensive AAV capsid library. The company has generated hundreds of proprietary third generation gene vectors that have entered clinical testing, and maintains a portfolio of clinical programs across a range of indications including Pompe, Limb Girdle Muscular Dystrophy, Cystic Fibrosis, Myotonic Dystrophy, Huntington's, Hemophilia (Chatham Therapeutic/Shire) and Duchenne Muscular Dystrophy (Bamboo Therapeutics/Pfizer). For more information, visit www.askbio.com.

About TPG

TPG is a leading global alternative asset firm founded in 1992 with more than \$104 billion of assets under management and offices in Austin, Beijing, Boston, Dallas, Fort Worth, Hong Kong, Houston, London, Luxembourg, Melbourne, Moscow, Mumbai, New York, San Francisco, Seoul, and Singapore. TPG's investment platforms are across a wide range of asset classes, including private equity, growth venture, real estate, credit, and public equity. TPG aims to build dynamic products and options for its investors while also instituting discipline and operational excellence across the investment strategy and performance of its portfolio. For more information, visit www.tpg.com

About Vida Ventures

Vida Ventures is a next-generation life sciences investment firm founded in 2017 by a group of scientists, physicians, entrepreneurs and investors passionate about building and funding breakthroughs in biomedicine. Together they form an independent, bold investment group bound together by a simple word – life. Its mission is to bring science to life and advance transformative biomedical innovations that have the potential to make a meaningful difference for patients. Vida has a bicoastal presence and currently manages approximately \$355 million. For more information on Vida Ventures, please visit www.vidaventures.com, on LinkedIn or follow on Twitter @Vida_Ventures.

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