Apic Bio Announces $40M Series A Financing to Advance Novel Gene Therapies for Rare Genetic Disorders

Scientific founders who originated the miRNA mediated "silence and replace" approach take on Alpha-1 and ALS

US patent awarded for its proprietary THRIVE™ platform

Appoints Chief Technology Officer with rAAV gene therapy cGMP manufacturing and virology expertise

CAMBRIDGE MA., January 7, 2019 (BusinessWire) – Apic Bio, Inc., an innovative gene therapy company developing novel treatment options for patients with rare genetic diseases, today announced the completion of a $40 million Series A financing led by Morningside Venture Investments, Ltd. Existing investors, The Alpha-1 Project (TAP) and A1ATD Investors, LLC also participated along with new investor, the ALS Investment Fund.

The new capital will advance Apic's APB-101, APB-102 as well as preclinical Alpha-1 and ALS programs including gene insertion of AAT and targeting of the C9orf72 mutation. Additionally, the new funding will support further discovery efforts leveraging the company's proprietary silence and replace THRIVE™ platform. In connection with the financing, Jason Dinges, Ph.D, J.D. from Morningside joins the board and Mr. Felix von Coerper from the ALS Investment Fund will become a board observer.

"Apic has built a strong rationale for its gene therapy approaches for the treatment of Alpha-1 and ALS. The University of Massachusetts Medical School and its world-renowned scientists and clinicians have developed an outstanding pre-clinical and initial human data package via a compassionate use IND for ALS," said Jason Dinges, Investment Advisor, Morningside Technology Advisory LLC. "There is a critical unmet need for these rare diseases and Morningside is pleased to support advancement of their programs."

"As investors committed to furthering treatment options for patients with ALS, we are excited to add Apic Bio to our portfolio," said Felix von Coerper, Founder SUNU Ventures and fund manager ALS Investment Fund. "We believe the company offers an innovative approach to genetic ALS and we look forward to partnering with Apic to not only make a social impact in the field of ALS but also a solid financial return for our investors."

"Rare diseases with both toxic gain of function and loss of function remain a major area of unmet medical need in the gene therapy field," said John Reilly, CEO and Co-Founder of Apic Bio. "We believe that our breakthrough approach that both silences mutant genes and simultaneously replaces normal, beneficial genes for monogenic diseases, could provide patients with an effective single treatment therapy."

"As 2019 kicks off, we were pleased to announce that US Patent No.10,077,452 "rAAV-Based Compositions and Methods" has been awarded to UMass Medical School and that the University has licensed its exclusive ownership rights in the patent to Apic Bio, which has full rights in the US and major international markets. It solidifies our extraordinary intellectual property position in miRNA mediated silencing and gene replacement technology."

Additionally, Scott Loiler, Ph.D., who was previously the Scientific Director of Vector Development at Nationwide Children's Hospital (NCH), joins the company as CTO. Dr. Loiler is a recognized gene therapy expert who was at the center of multiple translational gene therapy programs during his time at the NCH.

"We are thrilled to have Scott join Apic as he brings deep cGMP manufacturing and regulatory experience which will be key for our IND-enabling toxicology and human clinical studies," said Chris Mueller, Ph.D., Co-Founder of Apic Bio and Associate Professor in the Department of Pediatrics, UMass Medical School. "Scott will not only oversee our current CDMO relatioships, but also will be leading our efforts to fundamentally rethink and develop our own scalable manufacturing platform as we make a longer-term investment in a novel AAV production process."

About Apic Bio
Apic Bio is committed to finding cures for patients with genetic diseases. The company is a spin-off from the University of Massachusetts Medical School (UMMS) and is based upon nearly 30 years of gene therapy research by Apic's scientific founders. Apic is developing best-in-class treatment options for rare, devastating neurological and liver diseases. Its current pipeline focuses on new and effective treatments for Alpha-1 Antitrypsin Deficiency (Alpha-1, or AATD) and genetic Amyotrophic Lateral Sclerosis (ALS.) For more information please visit www.apic-bio.com.

About the THRIVE™ Platform
Numerous diseases are associated with inherited or somatic mutations. In many cases, these mutations can lead to both a toxic-gain-of function and loss-of function as in the case for Alpha-1 antitrypsin deficiency. In other cases, mutations in a single allele lead to dominant toxicity without a clear loss of function but approaches to reduce their toxicity by non-specifically silencing both alleles can unmask or result in a loss of function. In both these scenarios, the THRIVE™ platform provides a superior therapeutic approach than simple gene silencing or gene replacement approaches. The THRIVE™ platform both silences a mutant gene product and replaces a normal gene product in a single "dual function" vector.

About APB-101
APB-101 is a "liver-sparing" gene therapy designed as a one-time treatment for Alpha-1 patients. In pre-clinical studies it has demonstrated the ability to reduce levels of the mutant Alpha-1 protein (Z-AAT) and at the same time program liver cells to produce the correct Alpha-1 protein (M-AAT).

About APB-102
This Apic sponsored program now employs a second-generation vector design with a novel delivery route for the one-time treatment of genetic (SOD1) ALS. This program is based on a compassionate use IND study begun by Robert H. Brown, Jr., DPhil, MD; and Christian Mueller, PhD, in 2017 at the University of Massachusetts Medical School, with collaborators at the Massachusetts General Hospital.

About Morningside
Morningside is a diversified investment group founded in 1986 by the Chan family of Hong Kong. It is engaged primarily in private equity and venture capital investments. The group has investments in North America, Europe, across Asia-Pacific, and since 1992, in Mainland China. Morningside is an active investor in early-stage life science companies formed around new technologies that represent a high degree of novelty over existing technologies. More information is available at www.morningside.com

About ALS Investment Fund
The ALS Investment Fund supports and finances biotech companies developing drugs for Amyotrophic Lateral Sclerosis (ALS). We invest globally but focus on Europe and the US. Being a disease-specific VC fund, we bring in-depth knowledge and a deep network in the space. The portfolio companies we invest in are diversified, with multiple therapies and/or active programs in other, often larger, disease indications, typically in the area of neuro-degeneration. More information is available at www.alsinvestmentfund.com

About the University of Massachusetts Medical School
The University of Massachusetts Medical School has built a reputation as a world-class research institution, consistently producing noteworthy advances in clinical and basic research. The Medical School attracts more than $264 million in research funding annually, 80 percent of which comes from federal funding sources. The mission of the Medical School is to advance the health and well-being of the people of the commonwealth and the world through pioneering education, research, public service and health care delivery with its clinical partner, UMass Memorial Health Care. For more information, visit www.umassmed.edu.

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