

Vertex Pharmaceuticals and Affinia Therapeutics Establish Multi-Year Collaboration to Discover and Develop Novel AAV Capsids for Genetic Therapies

Affinia Therapeutics' proprietary AAV vector technology to be used in Vertex's genetic therapy efforts with focus on Duchenne muscular dystrophy, myotonic dystrophy type 1 and cystic fibrosis

BOSTON & WALTHAM, Mass. – (BUSINESS WIRE) – April 27, 2020 – Vertex Pharmaceuticals Incorporated (Nasdaq:VRTX) and Affinia Therapeutics announced today that the two companies have entered into a strategic research collaboration to engineer novel adeno-associated virus (AAV) capsids to deliver transformative genetic therapies to people with serious diseases. Affinia Therapeutics' proprietary AAVSmartLibrary and associated technology provides capsids for improved tissue tropism, manufacturability and pre-existing immunity. The collaboration will leverage Affinia Therapeutics' capsid engineering expertise and Vertex's scientific, clinical and regulatory capabilities to accelerate the development of genetic therapies for people affected by Duchenne muscular dystrophy (DMD), myotonic dystrophy type 1 (DM1) and cystic fibrosis (CF).

"This collaboration with Affinia Therapeutics will enhance our existing capabilities in discovering and developing transformative therapies for people with serious diseases," said Bastiano Sanna, Executive Vice President and Chief of Cell and Genetic Therapies at Vertex. "Affinia Therapeutics' innovative approach to the discovery and design of AAV capsids brings yet another tool to our Vertex Cell and Genetic Therapies toolkit, and we're excited to partner with them to bring together their technology platform with our research and development expertise."

"At Affinia Therapeutics, we're setting a new standard in genetic therapy by leveraging our platform to methodically engineer novel AAV vectors that have unique therapeutic properties," said Rick Modi, Chief Executive Officer. "Vertex is an established leader in developing transformative medicines for genetic diseases and renowned for its scientific rigor. We are thankful for the scientific validation this partnership brings and look forward to working closely with them to advance life-changing, differentiated genetic therapies and make a meaningful difference to those affected by these diseases."

About the Collaboration

Under the terms of the agreement, Affinia Therapeutics will apply its vector design and engineering technologies to develop novel capsids with improved properties. The agreement provides Vertex an exclusive license under Affinia Therapeutics' proprietary technology and intellectual property (IP) in DMD and DM1 with an exclusive option to license rights for CF and an additional undisclosed disease. The scope of the agreement covers all genetic therapy modalities in these diseases. Affinia Therapeutics will be eligible to receive over \$1.6 billion in upfront and development, regulatory and commercial milestones, including \$80 million in upfront payments and research milestones that will be paid during the research term, plus tiered royalties on future net global sales on any products that result from the collaboration. Affinia Therapeutics will be responsible for the discovery of capsids that meet certain pre-determined criteria. Vertex will be responsible for and will fund the design and manufacturing of genetic therapies incorporating the selected capsids, preclinical and clinical development efforts, and commercialization of any approved products in the licensed diseases.

About Affinia Therapeutics

At Affinia Therapeutics, our purpose is to develop gene therapies that can have a transformative impact on people affected by devastating genetic diseases. Our proprietary platform enables us to methodically engineer novel AAV vectors and gene therapies that have remarkable tissue targeting and other properties. We are building world-class capabilities to discover, develop, manufacture and commercialize gene therapy products with an initial focus on muscle and central nervous system (CNS) diseases with significant unmet need. www.affiniatx.com.

About Vertex Pharmaceuticals

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) – a rare, life-threatening genetic disease – and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust pipeline of investigational small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency and APOL1-mediated kidney diseases. In addition, Vertex has a rapidly expanding pipeline of genetic and cell therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes mellitus.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London, UK. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 10 consecutive years on Science magazine's Top Employers list and top five on the 2019 Best Employers for Diversity list by Forbes. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, Dr. Sanna's statements in the second paragraph of the press release, Mr. Modi's statements in the third paragraph of the press release, and statements regarding future activities of the parties pursuant to the collaboration. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent Vertex's beliefs only as of the date of this press release and there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, Vertex may not realize the anticipated benefits of the collaboration, and the other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

(VRTX-GEN)

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