



Vertex and Arbor Biotechnologies Establish Collaboration to Discover Novel Proteins to Advance Discovery of Gene-Editing Therapies

January 3, 2019

-Multi-year research collaboration funded by Vertex using Arbor's proprietary research platform to enhance efforts in developing gene-editing therapies in five diseases-

-Arbor to receive up-front cash payment and convertible note investment, research funding, and potential for additional milestones and royalty payments-

BOSTON & CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 3, 2019-- Vertex Pharmaceuticals Incorporated (NASDAQ:VRTX) and Arbor Biotechnologies (Arbor) today announced that the two companies have entered into a strategic research collaboration focused on the discovery of novel proteins including DNA endonucleases to advance the development of new gene-editing therapies for cystic fibrosis and four other diseases to be selected later. Arbor has developed a protein biodiscovery platform that includes a comprehensive set of technologies and techniques, integrating machine learning, genome sequencing, gene synthesis, and high-throughput screening that will augment Vertex's efforts to develop gene-editing approaches for the treatment of serious diseases.

"Our strategy of developing transformative medicines for serious diseases is based on addressing causal human biology with innovative therapeutics," said David Altshuler, M.D., Ph.D., Executive Vice President, Global Research and Chief Scientific Officer of Vertex. "Arbor's proprietary high-throughput screening platform will enhance our ongoing efforts to develop innovative gene-editing therapies."

"Vertex has a proven track record in discovering and developing innovative medicines and is an ideal partner for Arbor," said David Walt, Ph.D, Arbor Co-Founder. "We believe that by using our powerful biodiscovery platform we will be able to identify complementary and next generation tools to enhance Vertex's pipeline of transformative medicines and new gene-editing therapies."

About the Collaboration

Under the terms of the collaboration, scientists from Vertex and Arbor will work closely together to discover novel proteins and tools that Vertex may use in the development of new medicines. The focus of the collaboration is to discover novel programmable DNA endonucleases or nickases with high fidelity and catalytic activity as well as novel transduction approaches. Discovery activities will be conducted primarily by Arbor and funded by Vertex.

Vertex will provide Arbor with an up-front cash commitment, fund research activities, and will pay research, development, regulatory, and commercial milestones, as well as royalties on future net sales.

Vertex will also make an investment in Arbor in the form of a convertible note and obtain an observer seat on the Arbor Board of Directors.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases. In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases.

Founded in 1989 in Cambridge, Mass., Vertex's headquarters is now located in Boston's Innovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including being named to *Science* magazine's Top Employers in the life sciences ranking for nine years in a row.

For additional information and the latest updates from the company, please visit www.vrtx.com.

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. Altshuler's statements in the second paragraph of the press release, Dr. Walt'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.

About Arbor Biotechnologies

Arbor Biotechnologies is an early-stage company pushing the boundaries of biodiscovery. Based in Cambridge, MA, Arbor is unlocking nature's genetic diversity to create transformative products in human health and sustainability. Arbor's proprietary platform integrates a computational search engine with high-throughput experimental capabilities to enable both rapid protein characterization and product generation.

For more information, visit www.arbor.bio.

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