Vertex and Arbor Biotechnologies Establish New Partnership to Develop Novel ex vivo Engineered Cell Therapies

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Vertex to use Arbor's CRISPR gene-editing technology to enhance efforts in developing novel cell therapies for the treatment of serious diseases

CAMBRIDGE, MA – August 24, 2021 – Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and Arbor Biotechnologies (Arbor) today announced a new collaboration to enhance efforts in developing *ex vivo* engineered cell therapies, using Arbor's proprietary CRISPR gene-editing technology for select diseases.

The new agreement between Arbor and Vertex builds upon the companies' first partnership established in 2018. Under this new partnership, Vertex will receive rights to use Arbor's technology to research and develop *ex vivo* engineered cell therapies towards Vertex's goal of generating fully differentiated, insulin-producing hypoimmune islet cells for the treatment of type 1 diabetes, for next-generation approaches in sickle cell disease and beta thalassemia, and for the treatment of other diseases.

"This new collaboration further expands our toolkit in cell and genetic therapies and, specifically, our work to discover and develop cell therapies for the treatment of multiple serious diseases," said Bastiano Sanna, Ph.D., Executive Vice President and Chief of Cell and Genetic Therapies at Vertex. "We are excited to bring Arbor's technology together with Vertex's ongoing programs and capabilities in diabetes, hemoglobinopathies and other diseases to create improved cell replacement therapies for broad populations of patients."

"Arbor and Vertex share a common goal to improve the lives of people with life-threatening diseases through the discovery and development of innovative therapies, and Vertex has proven to be an ideal partner in that effort," said Devyn Smith, Ph.D., CEO of Arbor. "This agreement demonstrates the versatility and strength of Arbor's platform and reinforces our strategic vision of expanding our engineered cell therapy capabilities through partnerships with leading organizations in our industry, while we focus our internal portfolio efforts on genetic medicines."

About the Collaboration

Under the terms of the agreement, Arbor will receive an upfront cash payment and is eligible to receive up to $1.2 billion in potential payments based upon the successful achievement of specified research, development, regulatory and commercial milestones across up to seven potential programs. In addition, Vertex will pay tiered royalties on future net sales on any products that may result from this collaboration. Vertex will also make an investment in Arbor in the form of a convertible note.

About Vertex

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 cell and genetic 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. 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 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign. 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.

About Arbor

Arbor Biotechnologies is an early-stage life sciences company discovering and developing the next generation of genetic medicines. Co-founded by Feng Zhang and David Walt, Arbor uses its proprietary discovery engine to discover unique and wholly owned CRISPR-based genetic modifiers with differentiated genetic editing capabilities. This portfolio of gene editors can be custom tailored to address the underlying pathology of genetic diseases. Arbor's pipeline of genetic medicines is focused on bringing curative therapies to all patients with genetic disease. Following its strategic partnership with Vertex Pharmaceuticals to

accelerate the path to the clinic for Arbor's technologies, Arbor recently announced an agreement with Lonza. These partnerships further validate the breadth of applications of Arbor's gene editing platform. For more information about Arbor's technologies and innovations, visit arbor.bio.

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, statements by Bastiano Sanna, Ph.D., and Devyn Smith, Ph.D., in this press release, statements about the potential benefits and results that may be achieved through Vertex's collaboration with Arbor, statements regarding the future activities of the parties pursuant to the collaboration, statements regarding upfront and milestone payments, potential royalties on future sales and Vertex's investment in Arbor in the form of a convertible note. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that the anticipated benefits and potential of Vertex's collaboration with Arbor may not be achieved, that data may not support further development of the therapies subject to the collaboration due to safety, efficacy or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's annual report and subsequent quarterly reports filed with the Securities and Exchange Commission (SEC) and available through Vertex's website at www.vrtx.com and on the SEC's website at www.sec.gov. You should not place undue reliance on these statements. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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