



Vertex and Verve Therapeutics Establish Collaboration to Discover and Develop an In Vivo Gene Editing Program for Liver Disease

July 20, 2020

Verve to Receive \$25 Million Upfront Payment and \$35 Million Equity Investment, as Well as Potential Milestones and Royalties

BOSTON and CAMBRIDGE, Mass., July 20, 2022 (GLOBE NEWSWIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and Verve Therapeutics, Inc. (Nasdaq: VERV) today announced an exclusive, four-year global research collaboration focused on discovering and developing an *in vivo* gene editing program for a single undisclosed liver disease.

Under the terms of the collaboration, Verve will advance the discovery, research and certain preclinical development of a novel *in vivo* gene editing program for the target of interest, with all program costs funded by Vertex. Vertex will be responsible for subsequent development, manufacturing and commercialization of any program stemming from Verve's research efforts.

"This partnership with Vertex enables an important step forward for Verve as we build out our leading gene editing capabilities and pipeline of *in vivo* gene editing medicines to address serious diseases," said Sekar Kathiresan, M.D., Co-Founder and Chief Executive Officer of Verve. "This agreement validates the pioneering work at Verve to develop liver-directed gene editing medicines and expands the reach of our capabilities and breadth of our pipeline. We are thrilled to partner with Vertex to advance this important research and development program."

"Vertex is committed to discovering and developing transformative medicines for people with serious diseases," said David Altshuler, M.D., Ph.D., Executive Vice President, Global Research and Chief Scientific Officer of Vertex. "We are impressed by the progress Verve has made and look forward to combining the expertise in gene editing and drug development of our two companies to serve more patients in need."

Transaction Terms

Under the terms of the agreement, Verve will receive an upfront payment of \$60 million, including a \$35 million equity investment in Verve. Verve is also eligible to receive up to \$66 million in success payments, up to \$340 million in development and commercial milestones, and tiered royalties on future net sales for any products that may result from this collaboration agreement.

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, cell and genetic therapies in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, pain, type 1 diabetes, alpha-1 antitrypsin deficiency and Duchenne muscular dystrophy.

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 12 consecutive years on Science magazine's Top Employers list and one of the 2021 Seramount (formerly Working Mother Media) 100 Best Companies. 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 Verve Therapeutics

Verve Therapeutics, Inc. (Nasdaq: VERV) is a clinical-stage genetic medicines company pioneering a new approach to the care of cardiovascular disease, transforming treatment from chronic management to single-course gene editing medicines. The company's initial two programs target *PCSK9* and *ANGPTL3*, genes that have been extensively validated as targets for lowering blood lipids such as low-density lipoprotein cholesterol (LDL-C), a root cause of cardiovascular disease. Verve's lead product candidate, VERVE-101, is designed to permanently turn off the *PCSK9* gene in the liver in order to disrupt blood *PCSK9* protein production and thereby durably reduce blood LDL-C levels, with the goal of reducing a patient's risk for cardiovascular disease. VERVE-101 is being developed initially for the treatment of patients with heterozygous familial hypercholesterolemia, a potentially fatal genetic heart disease. For more information, please visit www.VerveTx.com.

Vertex 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 Drs. Sekar Kathiresan and David Altshuler in this press release, statements about the potential benefits and results that may be achieved through Vertex's collaboration with Verve, including Verve's gene editing capabilities and delivery technology, statements regarding upfront and milestone payments and potential royalties on future sales, and expectations about the ability to ultimately bring therapies to patients.

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 Verve may not be achieved on the anticipated timeline, or at all, 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 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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Verve Forward-Looking Statements

This press release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 that involve substantial risks and uncertainties, including statements regarding the potential benefits and results that may be achieved through the collaboration with Vertex, are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with the company’s limited operating history; the timing of and the company’s ability to submit applications for, its product candidates; advance its product candidates in clinical trials; initiate and enroll clinical trials on the timeline expected or at all; correctly estimate the potential patient population and/or market for the company’s product candidates; replicate in clinical trials positive results found in preclinical studies and/or earlier-stage clinical trials of VERVE-101 and its other product candidates; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; obtain, maintain or protect intellectual property rights related to its product candidates; manage expenses; and raise the substantial additional capital needed to achieve its business objectives. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the company’s actual results to differ from those contained in the forward-looking statements, see the “Risk Factors” section, as well as discussions of potential risks, uncertainties and other important factors, in the company’s most recent filings with the Securities and Exchange Commission and in other filings that the company makes with the Securities and Exchange Commission in the future. In addition, the forward-looking statements included in this press release represent the company’s views as of the date hereof and should not be relied upon as representing the company’s views as of any date subsequent to the date hereof. The company anticipates that subsequent events and developments will cause the company’s views to change. However, while the company may elect to update these forward-looking statements at some point in the future, the company specifically disclaims any obligation to do so.

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