Vertex Expands into New Disease Areas and Enhances Gene Editing Capabilities Through Expanded Collaboration with CRISPR Therapeutics and Acquisition of Exonics Therapeutics

June 6, 2019

-Provides Vertex with leading gene editing capabilities to develop novel therapies for Duchenne Muscular Dystrophy and Myotonic Dystrophy Type 1-

-CRISPR to receive an upfront payment of $175 million, with potential for additional milestone and royalty payments-

-Exonics to be acquired for an upfront payment of $245 million, with potential for additional milestone payments-

-John T. Gray, Ph.D. appointed Vertex Senior Vice President, Genetic Therapies-

BOSTON & WATERTOWN, Mass. & ZUG, Switzerland--(BUSINESS WIRE)--Jun. 6, 2019-- Vertex Pharmaceuticals Incorporated (NASDAQ: VRTX) today announced that the company is enhancing its gene editing capabilities to develop novel therapies for Duchenne Muscular Dystrophy (DMD) and Myotonic Dystrophy Type 1 (DM1) by expanding its collaboration with CRISPR Therapeutics and acquiring Exonics Therapeutics.

Vertex and CRISPR Therapeutics (NASDAQ: CRSP) have expanded their collaboration and entered into an exclusive licensing agreement to discover and develop gene editing therapies for the treatment of DMD and DM1.

Vertex and Exonics Therapeutics have entered into a definitive agreement under which Vertex will acquire privately held Exonics, a company focused on creating transformative gene editing therapies to repair mutations that cause DMD and other severe neuromuscular diseases.

“Through the expanded collaboration with CRISPR and the acquisition of Exonics, we are bringing together the intellectual property, technologies, and scientific expertise needed to establish a leading gene editing platform for DMD and DM1. These transactions are highly aligned with our strategy of investing in scientific innovation to create transformative medicines for people with serious diseases,” said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. “We are continuing to build a toolbox of small molecule and nucleic acid technologies and capabilities that will allow us to drive scientific innovation to produce transformative medicines for a broad portfolio of diseases.”

“This agreement with Vertex reflects the strong collaboration we have built together in other programs and underscores Vertex’s commitment to gene editing,” said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. “We continue to make significant advancements in enabling in vivo approaches for gene editing and are excited about the possibility of developing potentially curative therapies for DMD and DM1 together with Vertex.”

“DMD and DM1 are devastating muscle diseases with no curative therapies available,” said Eric Olson, Ph.D., Founder and Chief Science Advisor of Exonics, and Professor and Chair of the Department of Molecular Biology at UT Southwestern Medical Center. “Vertex has a proven track record of developing important therapies for serious diseases and we are excited to combine our efforts to potentially develop a safe and efficacious one-time treatment for severe neuromuscular diseases.”

“The Duchenne community needs novel approaches to treat and cure this devastating disease and Exonics’ technology has the potential to dramatically improve the lives of Duchenne patients,” said Debra Miller, CEO and Founder of Cure Duchenne that provided the initial seed funding for Exonics. “We are delighted that the talented scientists at Vertex and Exonics will work together to advance these promising gene editing treatments for Duchenne and other neuromuscular diseases.”

About Vertex’s Collaboration with CRISPR Therapeutics
Under the terms of this strategic collaboration and license agreement, Vertex will pay $175 million upfront for the exclusive worldwide rights to CRISPR Therapeutics’ existing and future intellectual property including foundational CRISPR/Cas9 technology, novel endonucleases, single and double cut guide RNAs, and AAV vectors for DMD and DM1 gene editing products.

For the DMD program, Vertex is responsible for all research, development, manufacturing, and commercialization activities and all related costs. For the DM1 program, Vertex and CRISPR will share research costs for specified guide RNA research to be conducted by CRISPR, and Vertex is responsible for all other research, development, manufacturing, and commercialization costs.

CRISPR Therapeutics is eligible to receive payments of up to $1 billion inclusive of the upfront and potential future payments

based upon the successful achievement of specified research, development, regulatory, and commercial milestones for the DMD and DM1 programs. In addition, Vertex will pay tiered royalties on future net sales on any products that may result from this collaboration. At IND filing, CRISPR has the option to forego the DM1 milestones and royalties to co-develop and co-commercialize all DM1 products globally.

The closing of this transaction will be subject to certain conditions, including the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act. The companies anticipate the transaction will close in the third quarter of 2019.

About Vertex's Acquisition of Exonics Therapeutics
Exonics Therapeutics is developing gene editing therapies to treat patients with DMD and other severe genetic neuromuscular diseases and brings to Vertex intellectual property, technology, and scientific expertise in gene editing therapies for these serious diseases. In multiple small and large animal DMD preclinical models, Exonics has used SingleCut CRISPR to genetically repair and restore dystrophin, the key protein missing in children with DMD.

Exonics' technology is licensed from UT Southwestern Medical Center and is based on the research of Dr. Olson. Dr. Olson will continue in his role as Exonics' chief science advisor and provide oversight and guidance on the research and development of transformative gene editing therapies.

Under the terms of the acquisition, Vertex will acquire all outstanding shares of Exonics, which will become a separate wholly-owned subsidiary of Vertex. Exonics equity holders are eligible to receive payments of approximately $1 billion, including $245 million upfront and potential future payments based primarily upon the successful achievement of specified development and regulatory milestones for the DMD and DM1 programs.

The closing of this transaction will be subject to certain conditions, including the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act and other customary conditions. The companies anticipate the acquisition will close in the third quarter of 2019.

About Vertex's Scientific Leadership Expansion
Vertex today announced the appointment of Dr. John T. Gray as Senior Vice President, Genetic Therapies effective June 17, 2019.

With more than twenty-five years in academic and industry settings, John is a proven leader in the genetic therapies space with experience in both the scientific and manufacturing sides of product development. His capabilities and expertise are an ideal fit with Vertex's strategy to bring the multi-faceted components of genetic therapies together and to advance the science of genetic therapies, such that the Vertex genetic therapies platform offers the best of all this emerging approach has to offer.

John previously served as Chief Scientific Officer and Senior Vice President at Audentes Therapeutics where he and his team focused on rAAV gene therapy for neuromuscular and liver disorders. John has a B.A. in Biochemistry from the University of California, Berkeley and a Ph.D. in Biochemistry from the University of Colorado, Boulder and was a postdoctoral Scientist at the Howard Hughes Medical Institute and Pfizer Central Research.

About Vertex's New Genetic Therapies Research Site
Vertex will establish a new genetic therapies research site in the Boston area where research programs, as well as vector development and clinical manufacturing for genetic therapies, including DMD and DM1 programs, will be conducted.

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'sInnovation 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.

Vertex 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. Leiden's statements in the fourth paragraph of the press release, Dr. Kulkarni's statements in the fifth paragraph of the press release, Dr. Olson's statements in the sixth paragraph of the press release, Ms. Miller's statements in the seventh paragraph of the press release and statements regarding the timing of the potential closing of the transaction, future activities of the parties pursuant to the collaboration and the potential benefits of the acquisition. 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, both transactions are subject to certain conditions, including the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act, Vertex may not realize the potential benefits of the collaboration or acquisition, 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 CRISPR Therapeutics
CRISPR Therapeutics is a leading gene editing company focused on developing transformative gene-based medicines for serious diseases using its proprietary CRISPR/Cas9 platform. CRISPR/Cas9 is a revolutionary gene editing technology that allows for precise, directed changes to genomic DNA. CRISPR Therapeutics has established a portfolio of therapeutic programs across a broad range of disease areas including hemoglobinopathies, oncology, regenerative medicine and rare diseases. To accelerate and expand its efforts, CRISPR Therapeutics has established strategic collaborations with leading companies including Bayer AG, Vertex Pharmaceuticals and ViaCyte, Inc.CRISPR Therapeutics AG is headquartered in Zug, Switzerland, with its wholly-owned U.S. subsidiary, CRISPR Therapeutics, Inc., and R&D operations based in Cambridge, Massachusetts, and business offices in London, United Kingdom. For more information, please visit www.crisprtx.com.

CRISPR Forward-Looking Statement
This press release may contain a number of “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including statements regarding CRISPR Therapeutics’ expectations about any or all of the following: (i) clinical trials (including, without limitation, the timing of filing of clinical trial applications and INDs, any approvals thereof and the timing of commencement of clinical trials), development timelines and discussions with regulatory authorities related to product candidates under development by CRISPR Therapeutics and its collaborators; (ii) the number of patients that will be evaluated, the anticipated date by which enrollment will be completed and the data that will be generated by ongoing and planned clinical trials, and the ability to use that data for the design and initiation of further clinical trials; (iii) the scope and timing of ongoing and potential future clinical trials; (iv) the intellectual property coverage and positions of CRISPR Therapeutics, its licensors and third parties; (v) the sufficiency of CRISPR Therapeutics’ cash resources; and (vi) the therapeutic value, development, and commercial potential of CRISPR/Cas9 gene editing technologies and therapies. Without limiting the foregoing, the words “believes,” “anticipates,” “plans,” “expects” and similar expressions are intended to identify forward-looking statements. You are cautioned that forward-looking statements are inherently uncertain. Although CRISPR Therapeutics believes that such statements are based on reasonable assumptions within the bounds of its knowledge of its business and operations, forward-looking statements are neither promises nor guarantees and they are necessarily subject to a high degree of uncertainty and risk. Actual performance and results may differ materially from those projected or suggested in the forward-looking statements due to various risks and uncertainties. These risks and uncertainties include, among others: the outcomes for each CRISPR Therapeutics’ planned clinical trials and studies may not be favorable; that one or more of CRISPR Therapeutics’ internal or external product candidate programs will not proceed as planned for technical, scientific or commercial reasons; that future competitive or other market factors may adversely affect the commercial potential for CRISPR Therapeutics’ product candidates; uncertainties inherent in the initiation and completion of preclinical studies for CRISPR Therapeutics’ product candidates; availability and timing of results from preclinical studies; whether results from a preclinical trial will be predictive of future results of the future trials; uncertainties about regulatory approvals to conduct trials or to market products; uncertainties regarding the intellectual property protection for CRISPR Therapeutics’ technology and intellectual property belonging to third parties; and those risks and uncertainties described under the heading "Risk Factors" in CRISPR Therapeutics’ most recent annual report on Form 10-K, and in any other subsequent filings made by CRISPR Therapeutics with the U.S. Securities and Exchange Commission, which are available on the SEC's website at www.sec.gov. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date they are made. CRISPR Therapeutics disclaims any obligation or undertaking to update or revise any forward-looking statements contained in this press release, other than to the extent required by law.

About Exonics Therapeutics
Exonics Therapeutics is developing gene editing therapies to treat patients with Duchenne muscular dystrophy and other severe genetic neuromuscular diseases. In multiple Duchenne preclinical models, Exonics has used SingleCut CRISPR to genetically repair and restore dystrophin, the key protein missing in children with Duchenne. Exonics is initially focused on repairing mutations that cause Duchenne in order to develop a therapy to treat many children with the devastating disease, for which there is no cure. Exonics’ technology is licensed from UT Southwestern Medical Center and is based on the research of Eric Olson, Ph.D., Exonics’ founder and chief science advisor. Exonics is located in Watertown, Mass. For more information, please visit www.exonicstx.com.

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