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Merck KGaA, Darmstadt, Germany Licenses Four Oncology Research and Development Programs from Vertex

-Merck KGaA, Darmstadt, Germany licenses two promising clinical-stage programs targeting DNA damage and repair, and two novel pre-clinical programs-

-Vertex receives upfront payment of \$230 million plus royalties on future sales-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that it has entered into a licensing agreement with Merck KGaA, Darmstadt, Germany for the worldwide development and commercialization of four promising research and development programs that represent novel approaches to the treatment of cancer. As part of the agreement, Merck KGaA, Darmstadt, Germany will license two clinical-stage programs targeting DNA damage and repair, along with two additional novel pre-clinical programs. Vertex will receive an upfront payment of \$230 million, in addition to royalties on future net sales. Merck KGaA, Darmstadt, Germany will assume full responsibility for the development and commercialization of all the programs.

"The Vertex R&D team has produced a portfolio of first-in-class compounds with the potential to enhance the therapy of multiple cancers," said Jeffrey Leiden, M.D., Ph.D, Chairman, President and CEO of Vertex. "We are pleased to partner with Merck KGaA, Darmstadt, Germany, a leader in oncology with exciting complementary assets that will help fully realize the value of these unique compounds and accelerate the programs' potential benefits for patients."

"With this strategic deal, we significantly strengthen our oncology pipeline in two attractive areas where we have leading competence, DNA damage and repair and immuno-oncology - areas which also have promising therapeutic synergy," said Belen Garijo, CEO Healthcare and Member of the Executive Board of Merck KGaA, Darmstadt, Germany. "This deal underscores our commitment to accelerate innovation for cancer patients, and we are excited for the opportunity to build on Vertex's rigorous science and advance these leading programs."

The two clinical-stage programs represent first-in-class approaches to inhibit the DNA repair pathways that are fundamental to the survival and proliferation of certain cancers:

- ┆ An ataxia telangiectasia and Rad3 related (ATR) protein kinase inhibitor program comprised of two compounds, VX-970 and VX-803. VX-970 is being investigated broadly through 10 ongoing Phase 1 and Phase 2 trials across a variety of tumors and patient subtypes expected to be responsive to ATR inhibition based on biomarker data. Preliminary VX-970 clinical data were presented at the 2016 American Society of Clinical Oncology (ASCO) Annual Meeting and the 2016 American Association for Cancer Research (AACR) Annual Meeting. VX-803 is an orally dosed ATR inhibitor currently in Phase 1 trials evaluating escalating doses of VX-803 alone and in combination with chemotherapy.
- ┆ A DNA-dependent protein kinase (DNA-PK) inhibitor program including the clinical candidate VX-984. A Phase 1 trial is now evaluating escalating doses of VX-984 alone and in combination with pegylated liposomal doxorubicin in subjects with advanced solid tumors. Merck KGaA, Darmstadt, Germany will combine these assets with its existing DNA-PK assets into a single development program.

The pre-clinical programs include one immuno-oncology program against an attractive target with first-in-class potential, and a program against a completely novel target. For both of these programs, Vertex research has demonstrated efficacy in relevant pre-clinical models, including demonstration of combination potential with immune checkpoint inhibition for the immuno-oncology program. Merck KGaA, Darmstadt, Germany will continue to characterize the Vertex compounds in these programs with the goal of taking them forward into the clinic.

The strength of the oncology R&D program at Merck KGaA, Darmstadt, Germany, including a leading presence in immunotherapy and DNA damage and repair, demonstrates how the company is re-imagining the way cancer can be treated.

The collaboration, and the related \$230 million upfront payment, is subject to the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act.

About Vertex

Vertex is a global biotechnology company that aims to discover, develop and commercialize innovative medicines so people with serious diseases can lead better lives. In addition to our clinical development programs focused on cystic fibrosis, Vertex has more than a dozen ongoing research programs aimed at other serious and life-threatening diseases.

Founded in 1989 in Cambridge, Mass., Vertex today has research and development sites and commercial offices in the United States, Europe, Canada and Australia. For seven years in a row, *Science* magazine has named Vertex one of its Top Employers in the life sciences. For additional information and the latest updates from the company, please visit www.vrtx.com.

About Merck KGaA, Darmstadt, Germany

Merck KGaA, Darmstadt, Germany is a leading science and technology company in healthcare, life science and performance materials. Around 50,000 employees work to further develop technologies that improve and enhance life - from biopharmaceutical therapies to treat cancer or multiple sclerosis, cutting-edge systems for scientific research and production, to liquid crystals for smartphones and LCD televisions. In 2015, Merck KGaA, Darmstadt, Germany generated sales of €12.85 billion in 66 countries.

Founded in 1668, Merck KGaA, Darmstadt, Germany is the world's oldest pharmaceutical and chemical company. The founding family remains the majority owner of the publicly listed corporate group. Merck KGaA, Darmstadt, Germany holds the global rights to the Merck KGaA, Darmstadt, Germany name and brand. The only exceptions are the United States and Canada, where the company operates as EMD Serono, MilliporeSigma and EMD Performance Materials.

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, the statements in the second and third paragraph of the press release and statements regarding (i) the upfront payment, which is subject to the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act, and (ii) potential royalty payments. 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 factors that could cause actual events to differ materially from those indicated by such forward-looking statements. These risks and uncertainties include, among other things, the 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.

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