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Vertex Licenses VX-787 to Janssen Pharmaceuticals for the Treatment of Influenza

-VX-787 is an investigational medicine discovered by Vertex that is designed to directly inhibit replication of the influenza virus-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that it has entered into a licensing agreement with Janssen Pharmaceuticals, Inc. for the worldwide development and commercialization of VX-787, a novel medicine discovered by Vertex for the treatment of influenza. As part of the agreement, Vertex will receive an up-front payment of \$30 million from Janssen and has the potential to receive additional development and commercial milestone payments as well as royalties on future product sales. Vertex completed a Phase 2a study of VX-787 in 2013 that showed statistically significant improvements in viral and clinical measurements of influenza infection. VX-787 is designed to directly inhibit replication of the influenza virus.

"With a deep history in developing new medicines for viral infections and diseases, Janssen is well-positioned to advance the global development of VX-787 for the treatment of influenza," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "This collaboration provides important support for the continued development of VX-787 in influenza and contributes to our financial strength to enable continued investment in our key development programs for cystic fibrosis and in research aimed at discovering new medicines."

About the Collaboration

Under the terms of the collaboration, Janssen will have full global development and commercialization rights to VX-787. Vertex will receive a \$30 million up-front payment from Janssen and could receive additional development and commercial milestone payments as well as royalties on future product sales. The collaboration, and the related \$30 million up-front payment, is subject to the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act.

About VX-787

VX-787 is an investigational medicine that is designed to directly inhibit replication of influenza A, including recent H1 (pandemic) and H5 (avian) influenza strains, based on *in-vitro* data. VX-787's mechanism represents a new class of potential medicines for the treatment of influenza, distinct from neuraminidase inhibitors, the current standard of care for the treatment of influenza. VX-787 is intended to provide a rapid onset of action and an expanded treatment window.

In a Phase 2a influenza challenge study, statistically significant improvements in viral and clinical measurements of influenza infection were observed after treatment with VX-787. The study met its primary endpoint and showed a statistically significant decrease in the amount of virus in nasal secretions (viral shedding) over the seven-day study period. In addition, at the highest dosing regimen evaluated in the study, there was a statistically significant reduction in the severity and duration of influenza-like symptoms. In this study, VX-787 was generally well-tolerated, with no adverse events leading to discontinuation. Those who took part in the study volunteered to be experimentally exposed to an attenuated form of live H3N2 influenza A virus. H3N2 is a common type of influenza virus and was the most common type observed in the 2012/2013 influenza season in the United States.

VX-787 was discovered by Vertex scientists.

About Influenza

Often called "the flu," seasonal influenza is caused by influenza viruses, which infect the respiratory tract.¹ The flu can result in seasonal epidemics² and can produce severe disease and high mortality in certain populations, such as the elderly.³ Each year, on average 5 to 20 percent of the U.S. population gets the flu⁴ resulting in more than 200,000 flu-related hospitalizations and 36,000 deaths.⁵ The overall national economic burden of influenza-attributable illness for adults is \$83.3 billion.⁵ Direct medical costs for influenza in adults totaled \$8.7 billion including \$4.5 billion for adult hospitalizations resulting from influenza-attributable illness.⁵ The treatment of the flu consists of antiviral medications that have been shown in clinical studies to shorten the disease and reduce the severity of symptoms if taken within two days of infection.⁶ There is a significant need for new medicines targeting flu that provide a wider treatment window, greater efficacy and faster onset of action.

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 four 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.

Vertex's press releases are available at 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. Leiden's statements in the second paragraph of the press release and statements regarding (i) the up-front payment, which is subject to the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act, and (ii) potential development and commercial milestone and royalty payments. While Vertex believes the forward-looking statements contained in this press release are accurate, those statements are subject to risks and uncertainties that could cause actual outcomes to vary materially from the outcomes referenced in the 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.

(VRTX-GEN)

¹ Centers for Disease Control and Prevention. Key Facts about Influenza (Flu) & Flu Vaccine. CDC Web site: www.cdc.gov/flu/keyfacts.htm. Accessed February 28, 2013.

² Centers for Disease Control and Prevention. Influenza (Flu) Viruses. CDC Web site: <http://www.cdc.gov/flu/about/viruses/index.htm>. Accessed February 28, 2013.

³ Centers for Disease Control and Prevention. Key Facts about Influenza (Flu) & Flu Vaccine. CDC Web site: www.cdc.gov/flu/keyfacts.htm. Accessed February 28, 2013.

⁴ Centers for Disease Control and Prevention. Season Influenza. CDC Web site: <http://www.cdc.gov/flu/about/qa/disease.htm>. Accessed February 28, 2013.

⁵ Centers for Disease Control and Prevention. Adult Immunization. CDC Web site: <http://www.cdc.gov/workplacehealthpromotion/evaluation/topics/immunization.html>. Accessed February 28, 2013.

⁶ Centers for Disease Control and Prevention. What you should know about flu antiviral drugs. CDC Web site: <http://www.cdc.gov/flu/antivirals/whatyoushould.htm>. Accessed February 28, 2013.

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