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## **Vertex Pharmaceuticals Reports Data Suggesting Potential for Oral Combination Approaches in Treatment of HCV**

Tucson, AZ, May 4, 2004 -- New preclinical data for two proprietary investigational antiviral therapies for hepatitis C virus (HCV) infection being developed by Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) suggest the potential for oral combination approaches in the treatment of hepatitis C virus (HCV) infection. In a poster presentation at the International Conference for Antiviral Research (ICAR) in Tucson, Arizona, Vertex researchers demonstrated that merimepodib, Vertex's lead oral HCV therapy, and VX-950, an investigational HCV protease inhibitor, exhibited enhanced antiviral activity when dosed in combination in HCV replicon cells. These results highlight the potential for future clinical approaches in HCV evaluating different combinations of antiviral agents, including oral-only combinations.

Vertex scientists conducted a series of experiments using a two-day HCV replicon assay, which mimics the intracellular replication of HCV, and evaluated the antiviral effect of several drug-drug combinations in various concentrations. All combinations of merimepodib (MMPD) and VX-950 resulted in greater reduction of HCV RNA than either agent alone, and computational analysis determined that the antiviral effects of MMPD and VX-950 were additive to moderately synergistic. At the highest concentrations tested, the combination of MMPD and VX-950 in replicon cells led to a 2 log reduction in HCV RNA in two days. Researchers also observed antiviral synergy in two triple combinations consisting of MMPD + interferon alpha + ribavirin and MMPD + interferon alpha + VX-950 in two-day replicon assays.

"In clinical practice, new treatment options and combination approaches have the potential to provide increased antiviral activity as well as enhanced tolerability, which are key unmet needs in HCV treatment," stated John Alam, M.D., Senior Vice President for Drug Evaluation and Approval at Vertex. "The combined antiviral activity of Vertex's investigational agents, as measured in the laboratory, provides insight into novel clinical strategies which Vertex may be able to pursue to enhance clinical care in the treatment of HCV."

### **Clinical Need and Market Opportunity in HCV Infection**

Chronic hepatitis C virus (HCV) infection is a serious public health concern affecting approximately 2.7 million people in the United States. HCV causes inflammation of the liver, which may lead to fibrosis and cirrhosis, liver cancer, and ultimately, liver failure. Cirrhosis of the liver resulting from chronic HCV infection is the leading indication for liver transplantation in the U.S. Due to the asymptomatic nature of HCV infection, it often goes undetected for up to 20 years following initial infection. Worldwide, the disease strikes as many as 185 million people. Each year, 8,000 to 10,000 people in the U.S. die from complications of HCV.

The current standard of care in HCV treatment is a treatment combination of pegylated interferon (peg-IFN), an injectable agent, and ribavirin. This combination therapy provides a sustained viral response for only 40 to 50 percent of patients chronically infected with genotype 1 HCV, the most difficult viral strain to treat and the most common form in the U.S. Additionally, peg-IFN and ribavirin combination therapy are associated with treatment-limiting side effects, including depression, fatigue, flu-like symptoms, and hemolytic anemia.

### **About Merimepodib and VX-950**

Merimepodib is Vertex's lead oral therapy for the treatment of HCV infection. Vertex anticipates initiating a Phase IIb clinical study in 2004 of merimepodib in patients who are non-responsive to prior treatment with peg-IFN + ribavirin. VX-950 is Vertex's lead oral HCV protease inhibitor and one of the most advanced of a new class of antivirals in development for HCV. Vertex anticipates that it will initiate a Phase I clinical trial of VX-950 in healthy volunteers in the second quarter of 2004. Positive results from the first Phase I study will pave the way for the first evaluation of VX-950's activity in HCV-infected subjects.

### **About Vertex**

Vertex Pharmaceuticals Incorporated is a global biotechnology company committed to the discovery and development of breakthrough small molecule drugs for serious diseases. The Company's strategy is to commercialize its products both independently and in collaboration with major pharmaceutical partners. Vertex's product pipeline is principally focused on viral diseases, inflammation, autoimmune diseases and cancer.

Vertex co-promotes the new HIV protease inhibitor, Lexiva(TM), with GlaxoSmithKline.

This press release may contain forward-looking statements, including statements that (i) merimepodib and VX-950 hold

promise as part of combination therapy for HCV patients who have limited treatment options and represents an attractive commercial opportunity for Vertex; and (ii) combination therapy may result in increased antiviral activity and enhanced tolerability; and (iii) further clinical study of merimepodib and VX-950 will be initiated in 2004. While management makes its best efforts to be accurate in making forward-looking statements, such statements are subject to risks and uncertainties that could cause Vertex's actual results to vary materially. These risks and uncertainties include, among other things, the risks that clinical trials for merimepodib or VX-950 may not proceed as planned due to technical, scientific, or patient enrollment issues, that actual clinical studies of VX-950 and merimepodib in combination will not reflect the results obtained through in vitro testing, and that clinical results may not demonstrate the value of combination therapies for HCV patients generally, and other risks listed under Risk Factors in Vertex's form 10-K filed with the Securities and Exchange Commission on March 15, 2004.

Lexiva(TM) is a registered trademark of the GlaxoSmithKline group of companies.

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