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# Vertex Pharmaceuticals Announces that VX-680 Demonstrates Effect on Clinically Relevant Biomarker in Phase I Cancer Study 

## - Company achieves two milestones in broad collaboration with Merck to develop VX-680 and Aurora kinase inhibitors -

Cambridge, MA, December 7, 2005 - Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced clinical and research progress in its worldwide collaboration with Merck \& Co., Inc. to develop Aurora kinase inhibitors for the treatment of cancer. In a Phase I clinical study in patients with solid tumor cancers, dosing with VX-680 (MK-0457) demonstrated activity on a clinically relevant biomarker, triggering achievement of a milestone under the contract. VX-680 is a small molecule inhibitor of Aurora kinases and the lead development compound in the collaboration. In addition, Merck has selected a follow-on compound for development from a joint Aurora kinase research program that is part of the collaboration.

In conjunction with this progress, Vertex will receive two milestone payments from Merck totaling $\$ 19.5$ million. Vertex expects to recognize revenue of approximately $\$ 7.0$ million of the $\$ 19.5$ million in milestone payments in the fourth quarter of 2005 , and the balance in 2006.
"We are pleased with our progress in the research and development of Aurora kinase inhibitors, which may represent an exciting new approach to the treatment of cancer," said Stephen H. Friend, M.D., Ph.D., Executive Vice President, Advanced Technology \& Oncology at Merck Research Laboratories. "We initiated a number of clinical trials for VX-680 in 2005, and the biomarker activity observed in the Phase I trial in solid tumors is encouraging. We look forward to continued progress in the coming year's program as we look to further define the clinical activity of VX-680 in several potential oncology indications."
"The rapid achievement of these milestones is an important part of our goals for the VX-680 program and in realizing our 2005 corporate objectives," said Joshua Boger, Ph.D., Chairman, President and Chief Executive Officer of Vertex Pharmaceuticals. "Working together with Merck, we have successfully executed on all key clinical and research activities in the program this year. The progress announced today affirms our view that VX-680 and Aurora kinase inhibitors have the potential to play an important role in cancer therapy."

## VX-680 Clinical Studies

Merck is presently conducting three clinical studies of VX-680 in patients with hematologic cancers, recurrent or nonresponsive solid tumors, or cancers for which standard therapy does not currently exist. In these studies, the safety and tolerability of VX-680 are being evaluated when administered as either a 24 -hour continuous infusion or as a 5 -day continuous infusion. Presentation of clinical data from these studies at one or more scientific conferences is anticipated in 2006.

## Background: Aurora Kinases and the VX-680 Collaboration

Cancer cells typically contain mutations in a number of genes, which ultimately result in uncontrolled cell growth and tumor metastasis. As enzymes specific for and essential to cell growth and division, Aurora kinases hold the potential to be important control points for slowing the growth and spread of tumors. Aurora kinases ( $\mathrm{A}, \mathrm{B}$ and C ) comprise a family of serine-threonine kinases that are believed to play multiple roles in the development and progression of cancer by acting as regulators of cell proliferation, by transforming normal cells into cancer cells and by down-regulating p53, one of the body's natural tumor suppressors. Aurora kinases are known to be over-expressed in many tumor types, including colon cancer, breast cancer, ovarian cancer and other cancers as well as in lymphoma and leukemias. Amplification of Aurora genes is associated with progression of colorectal cancer and poor prognosis in certain types of breast cancer.

In June 2004, Vertex entered into a global collaboration with Merck to develop and commercialize VX-680. Along with clinical development, Vertex and Merck are conducting a joint research program to characterize VX-680's activity across a broad range of cancer types and have identified an additional drug candidate targeting the Aurora kinases.

## 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 companies. Vertex's product pipeline is principally focused on viral diseases, inflammation, autoimmune diseases and cancer. Vertex co-promotes the HIV protease inhibitor, Lexiva, with

GlaxoSmithKline.
Lexiva is a registered trademark of the GlaxoSmithKline group of companies.

## Vertex Forward-Looking Statement

This press release may contain forward-looking statements, including statements that (i) Vertex expects to receive $\$ 19.5$ million in milestone payments from Merck and expects to recognize revenue of approximately $\$ 7.0$ million of the $\$ 19.5$ million in the fourth quarter of 2005, and the balance in 2006; (ii) the progress announced today affirms the view that VX-680 and Aurora kinase inhibitors have the potential to play an important role in cancer therapy; and (iii) presentation of clinical data from the Phase I studies is anticipated in 2006. 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 the actual results of studies to vary materially. Those risks and uncertainties include the risk that non-clinical or early clinical results from studies of compounds targeting Aurora kinases may not be predictive of human clinical results, or later stage clinical results, in the treatment of cancer, that development of VX-680 may not be pursued due to clinical, technical or financial issues, that the rate of patient enrollment may limit expected study timelines and follow-on studies, that Merck may choose not to develop VX-680 or any other Aurora kinase inhibitor discovered by Vertex for commercial or scientific reasons, and other risks listed under Risk Factors in Vertex's Form 10-K filed with the Securities and Exchange Commission on March 16, 2005.

## Vertex Contacts:

Lynne H. Brum, VP, Corporate Communications and Financial Planning, (617) 444-6614
Michael Partridge, Director, Corporate Communications, (617) 444-6108
Lora Pike, Manager, Investor Relations, (617) 444-6755
Zachry Barber, Specialist, Media Relations, (617) 444-6470

