February 23, 2004

Vertex Researchers Report First Demonstration of Tumor Growth Suppression and Tumor Regression In Vivo with Aurora Kinase Inhibitors

- Study Published in Nature Medicine -

Cambridge, MA, February 23, 2004 -- Today in Nature Medicine, researchers from Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) have demonstrated for the first time that a selective small molecule inhibitor of the Aurora kinases, VX-680, profoundly reduces tumor growth in cancer models. Aurora kinases are known to be overexpressed in many tumor types, including colon cancer, breast cancer, and leukemia.

Research into the function and activity of Aurora kinases over the past several years has suggested that they may 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 downregulating p53, one of the body's natural tumor suppressors.

In the study reported today, the small molecule Aurora kinase inhibitor VX-680 blocked cancer cell proliferation, and also triggered cell death in a broad range of tumor cell types. Data from in vivo xenograft models indicated that VX-680 achieved complete inhibition of tumor growth at well-tolerated doses, and in some instances tumor regression was observed. The report suggests that Aurora kinase inhibition provides a promising, novel approach for the treatment of multiple human malignancies.

" Aurora kinases represent a potentially important class of targets for the future treatment of cancer, and we have now demonstrated for the first time that a small molecule Aurora kinase inhibitor not only blocks tumor cell proliferation but also induces tumor cell death," commented Karen Miller, Ph.D, Director of Biology, Vertex Europe and senior author of the study. "The ability of VX-680 to cause tumor regression is particularly exciting."

" VX-680 has demonstrated promising results in a range of tumor types," said Peter Mueller, Ph.D., Chief Scientific Officer of Vertex. "Inhibition of Aurora kinases represents a novel and highly targeted approach to cancer therapy, and we look forward to the evaluation of Aurora kinase inhibitors in the clinic in 2004."

Aurora Kinases and Cancer
Aurora kinases (also known as BTAK and STK15) are a family of serine-threonine kinases that have been strongly linked to tumorigenesis. Aurora kinases, which play a central role in controlling cell division, are disregulated in many types of human cancer, including leukemia, colon and breast cancer. Overexpression of Aurora kinase has been shown to promote the transformation of normal cells into cancer cells, and decreased Aurora kinase activity is associated with enhanced function of the body's normal tumor suppressor genes. Amplification of Aurora genes is associated with progression of colorectal cancer and poor prognosis in certain types of breast cancer.

Cancer is the second leading cause of death in the United States; more than 1 million solid tumor cancers are diagnosed in the United States annually, with approximately 500,000 deaths. More than 200,000 new cases of breast cancer and 150,000 cases of colorectal cancer are diagnosed in the U.S. each year. The five-year relative survival rates for patients with metastatic breast cancer and patients with colorectal cancer are 21% and 8% respectively. There are more than 30,000 new cases of leukemia in the U.S. every year, and more than 20,000 deaths.

Cancer cells typically contain mutations in a number of genes, which ultimately results 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.

About VX-680
VX-680 was discovered at Vertex as part of a broad research effort targeting the kinase gene family. Vertex researchers published the three-dimensional atomic crystal structure of Aurora-A kinase in 2002, a key scientific advance that enabled the design and optimization of multiple classes of small molecule Aurora kinase inhibitors. VX-680 was advanced to preclinical development in 2002, following evaluation of the compound's activity in cell lines and in animal models of tumor growth. In the study published today, Vertex researchers reported that VX-680 induced tumor regression of 22% in a human pancreatic xenograft model and of 56% in a human colon cancer xenograft model. Vertex has filed an investigational new drug (IND) application with the FDA to support Phase I clinical development of VX-680 in 2004.

Vertex's broad kinase research program is supported by a collaboration with Novartis Pharma AG begun in May 2000 and amended in 2004. Under the terms of the amended agreement, Vertex may opt to continue development of VX-680 under the terms of the original agreement, or to develop and commercialize VX-680 independently.The research was published in the March 2004 issue of the scientific journal Nature Medicine. The authors of the paper, titled "VX-680, a Novel Small Molecule Inhibitor of the Aurora Kinases, Suppresses Tumor Growth In Vivo," are Elizabeth A. Harrington, David Bebbington, Jeff Moore, Richele K. Rasmussen, Abi O. Ajose-Adeogun, Tomoko Nakayama, Joanne A. Graham, Thierry Hercend, Anita Diu-Hercend, Michael Su, Julian M.C. Golec, and Karen M. Miller, all current or former employees of Vertex, and Cecile Demur of INSERM.

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.

Vertex Safe Harbor Statement
This press release may contain forward-looking statements, including statements that targeting Aurora kinases holds the potential to treat multiple human malignancies, and that VX-680 will enter Phase I development 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. Those risks and uncertainties include the risk that preclinical results targeting Aurora kinases may not be predictive of human clinical results, that development of VX-680 may not be pursued due to clinical, technical or financial issues, and other risks listed under Risk Factors in Vertex's Form 10-K filed with the Securities and Exchange Commission on March 31, 2003.

Vertex Contacts:
Lynne H. Brum, VP, Corporate Communications and Financial Planning, (617) 444-6614
Michael Partridge, Director, Corporate Communications, (617) 444-6108