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# Vertex Pharmaceuticals Licenses VX-944 to Avalon Pharmaceuticals for Development and Commercialization in the Treatment of Cancer

Cambridge, MA, and Germantown, MD, February 15, 2005 -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and Avalon Pharmaceuticals Incorporated today announced that they have entered into a licensing agreement for the development of the investigational agent VX-944 in oncology indications. Under the terms of the agreement, Avalon will hold exclusive rights to develop and commercialize VX-944 worldwide for the treatment of various cancers. Avalon, a privately held company with a therapeutic focus in cancer, will make up to \$73 million in up-front license fees and milestone payments to Vertex based on the successful development and commercialization of VX-944 in oncology. Upon commercialization, Vertex will receive royalties on product sales. Vertex retains certain rights to co-promote VX-944 in U.S. and European markets.

VX-944, a small molecule IMPDH inhibitor discovered by Vertex Pharmaceuticals, has completed a single-dose, Phase I study in healthy volunteers. Recent reports in medical literature and presentations at scientific conferences provide a strong clinical rationale for the development of IMPDH inhibitors generally, and VX-944 specifically, in hematologic malignancies. Results presented at the Annual Meeting of the American Society of Hematology (ASH) in December 2004 indicated that VX-944 inhibited the in vitro proliferation of lymphoid and myeloid cells, the principal cells involved in the most common types of human leukemias. In addition, VX-944 was shown to significantly prolong survival in a model of aggressive mouse leukemia. Based on these results, Avalon plans to initiate a clinical trial with VX-944 in a hematologic cancer indication in the second half of 2005. In addition, Avalon expects to use its chemical genomics platform to evaluate VX-944's potential in the treatment of solid tumors.

"Avalon is pleased to collaborate with Vertex, a proven leader in the discovery and development of small molecule therapeutics," said Kenneth C. Carter, Ph.D., President and Chief Executive Officer of Avalon Pharmaceuticals. "The AvalonRx (TM) drug discovery and analysis engine has enabled us to build a pipeline of novel oncology targets and therapeutic compounds, and this collaboration accelerates our downstream integration toward the development and commercialization of new cancer treatments. Further using our platforms, we will build on Vertex's studies and develop biomarkers for stratification and monitoring for efficacy in patients."

"Based on its unique anti-proliferative mechanism, VX-944 holds strong potential in the treatment of hematologic cancers, as well as other tumor types," said Joshua Boger, Ph.D., Chairman and CEO of Vertex. "This licensing agreement with Avalon places VX-944 in the hands of a committed partner. We expect that Avalon will rapidly advance this compound from early development to clinical proof-of-concept, fulfilling an important strategic objective for the VX-944 program."

### **About Avalon**

Avalon Pharmaceuticals utilizes unique and proprietary forward chemical genomics-based methods to accelerate the discovery of novel targets and drugs. Avalon's unique capabilities in cytogenetics and gene expression analysis allow Avalon to rapidly discover and validate novel oncology targets. For the discovery of small molecules, Avalon utilizes a comprehensive systems approach, enabling more informed decisions at earlier stages in the drug discovery process. The AvalonRx(TM) drug discovery platform allows for the rapid identification of lead compounds through an enriched understanding of the mechanism of action within diseased cells, leading to accelerated medicinal chemistry driven lead optimization programs and clinical development. This revolutionary systems approach is applicable across multiple disease areas. In December 2004, Avalon was recognized by Red Herring as one of the top 100 innovator companies. Currently, Avalon is focusing its efforts to produce the next generation of cancer medicines. For more information please visit <a href="https://www.avalonrx.com">www.avalonrx.com</a>.

### **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(R), with GlaxoSmithKline.

This press release may contain forward-looking statements, including statements that Vertex expects that (i) Avalon will make up-front, milestone and royalty payments as set forth above; (ii) Avalon will initiate a clinical trial of VX-944 in the second half of 2005; (iii) Avalon will use its functional genomics platform to evaluate VX-944's potential in the treatment of solid tumors; and (iv) Avalon will rapidly advance VX-944 through early development to clinical proof-of-concept. 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 (i) clinical trials for VX-944 may not proceed as planned due to technical, scientific, or patient enrollment issues, final results from clinical trials will not reflect positive interim results, or clinical trial results may not be available when expected, or expected regulatory filings may not occur or may be delayed due to adverse clinical or non-clinical trial developments, any or all of which may adversely affect the financial success of the Company, and (ii) Vertex will be unable to realize revenues from this collaboration due to a variety of financial, technical or partnership considerations, and other risks listed under Risk Factors in Vertex's Form 10-K filed with the Securities and Exchange Commission on March 15, 2004 and amended on September 8, 2004.

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

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