



January 12, 2004

## **Vertex Pharmaceuticals Highlights 2003 Accomplishments and 2004 Outlook at 22nd Annual JP Morgan Healthcare Conference**

**Cambridge, MA, January 12, 2004** -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today presented its 2003 accomplishments and 2004 outlook at the 22nd Annual JPMorgan Healthcare Conference in San Francisco. The Company also provided preliminary results from the Phase II study of pralnacasan in osteoarthritis. The update was presented by Vicki Sato, Ph.D., President of Vertex Pharmaceuticals. A live webcast of the presentation will be available on Vertex's website, [www.vrtx.com](http://www.vrtx.com), at 9:30 am PT, January 12, 2004. An archived webcast of the presentation will be available on Vertex's website through January 30, 2004.

"In 2003, Vertex made important advances in its business," stated Dr. Sato. "We launched the HIV protease inhibitor Lexiva (TM) in the U.S. with our partner GlaxoSmithKline, strengthening our commercial presence in antivirals. We also expanded our commitment to developing new treatments for viral disease by selecting the oral HCV therapy merimepodib as a lead product candidate for full development and commercialization by Vertex. Supported by our strong financial profile and continued progress in other important areas of research and development, we have brought Vertex closer to our goal of becoming a major drug company."

"Disciplined execution in clinical development in 2003 is enabling Vertex to focus its research and development investment in 2004 in its antiviral and inflammation product portfolios," continued Dr. Sato. "We look forward to the continued advancement of Vertex-driven and partner-driven products in 2004 and believe that our clinical progress will ultimately drive a transition to profitability and sustained profit growth."

### **2004 Corporate Goals:**

Vertex provided an overview of milestones for 2004 that will enable the Company to advance its business. Specific milestones included:

- **Advance HIV franchise with partner GlaxoSmithKline:**
  - Launch the new HIV protease inhibitor Lexiva(TM) in Europe
  - Begin Phase II development of investigational HIV protease inhibitor VX-385
- **Advance proprietary HCV pipeline:**
  - Initiate pivotal development of oral HCV therapy merimepodib
  - Conduct Phase I development of oral HCV protease inhibitor VX-950
  - Advance second-generation HCV compounds into preclinical development
- **Advance inflammation pipeline:**
  - Complete pilot Phase II trial of p38 MAP kinase inhibitor VX-702 for acute coronary syndromes (ACS)
  - Initiate Phase II development of oral ICE inhibitor VX-765
  - Complete toxicology evaluation for pralnacasan in partnership with Aventis
- **Invest in discovery organization**
  - Select new drug development candidates in kinase and voltage-gated sodium ion channel research programs
- **Establish new pharmaceutical collaborations.**

### **Pralnacasan Phase II Osteoarthritis Study: Preliminary Results**

Aventis and Vertex today announced preliminary results from a Phase II study of pralnacasan in patients with osteoarthritis (OA) that was completed in 2003. The Phase II study, involving 522 patients with knee OA treated with one of three dosages of pralnacasan or placebo for 12 weeks, was designed to provide a preliminary evaluation of the safety and clinical activity of pralnacasan in OA patients. Aventis and Vertex reported that pralnacasan was well tolerated across all three dosage groups. There was improvement (29-35%) in all four treatment groups in the primary endpoint, total WOMAC scores, during the 12 weeks of study. (The WOMAC is "Western Ontario and McMaster Universities" scale for measuring signs and symptoms in OA studies.) However, there were no statistically significant differences in the change in total WOMAC score, between placebo treatment and any of the pralnacasan treatment groups. In the Phase II OA study reported today, Aventis and Vertex reported statistically significant changes in some urine and serum markers of bone and cartilage turnover. However, interpretation of these results in the context of modifying the progression of OA requires additional scientific understanding, which may require further clinical validation.

The decision by Aventis and Vertex to proceed to longer-duration studies in OA and other inflammatory diseases will be part of

a program review in the coming months, and will be based on the results of ongoing and planned toxicology studies and additional analysis of the current study.

"Pralnacasan has been the subject of an extensive Phase II clinical program, reflecting the promise of oral ICE inhibitors as a breakthrough strategy to treat a range of inflammatory diseases," said Vicki Sato, President of Vertex. "We will continue to work diligently in 2004 with Aventis to evaluate the clinical and toxicology results to determine the appropriate path forward."

### **Financial Outlook**

Vertex today reiterated its guidance for a full-year 2003 loss, before charges and gains, of less than \$180 million.

Vertex plans to provide 2004 financial guidance when it releases its year-end 2003 financial results on Wednesday, February 11, 2004. In conjunction with this announcement, the Company will host a conference call at 5:00 pm (ET), February 11, 2004. The conference call also will be webcast. The webcast will be available to all interested parties through Vertex's website, [www.vrtx.com](http://www.vrtx.com). To access the webcast, go to the investor center and select "conference calls." The call will also be available via telephone at 800-374-0296.

"We are committed to a disciplined financial strategy that will support our short and long-term objectives," stated Ian Smith, Senior Vice President and CFO of Vertex Pharmaceuticals. "During 2004, we will invest in our proprietary products that are selected for late-stage development, we will adjust our R&D investment based on the progression of our early-stage drug candidates, and we will continue to seek pharmaceutical collaborations that are aligned with our short and long-term objectives."

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

### **Non-GAAP Financial Measures**

In this press release, Vertex provides guidance for a 2003 loss, excluding certain charges and gains, of less than \$180 million, which is a non-GAAP financial measure. This guidance is provided as a complement to results provided in accordance with GAAP because management believes these non-GAAP financial measures help indicate underlying trends in the Company's business, and uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally, to manage the Company's business and to evaluate its performance.

This press release may contain forward-looking statements, including statements that (i) VX-385 will enter Phase II development in 2004; (ii) Vertex-driven and partner-driven product candidates will continue to advance in 2004, and clinical progress will ultimately enable Vertex to transition to profitability and sustained profit growth; (iii) Lexiva will be approved and launched in Europe in 2004; (iv) pivotal development of merimepodib will begin in 2004; (v) Phase I development of VX-950 will begin in 2004; (vi) Vertex will advance second-generation HCV compounds in 2004; (vii) Vertex will complete a pilot Phase II trial of VX-702 for acute coronary syndromes; (viii) Vertex will initiate Phase II development of VX-765 in 2004; (ix) Vertex and Aventis will resolve the non-clinical toxicology finding with pralnacasan; (x) Vertex will advance new drug candidates from its ion channel and kinase research programs in 2004; (xi) Vertex will sign new collaborations in 2004; (xii) Vertex will achieve the financial guidance set forth above for 2003. 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 one or more of Vertex's drug candidates 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; (ii) partnerships or collaborations for the future development of some or all of Vertex's drug candidates may not be available on terms satisfactory to Vertex, if at all; (iii) Vertex will be unable to realize its financial objectives 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 31, 2003.

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

### **Webcast:**

Vertex Pharmaceuticals will webcast its corporate presentation at the 22nd Annual JPMorgan Healthcare Conference on January 12, 2004 at 9:30 am PT. A link to the webcast will be available via the Internet at Vertex's website, [www.vrtx.com](http://www.vrtx.com), in the Investor Center.

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