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# Vertex Outlines 2009 Business Priorities: Registration Programs in Hepatitis C and Cystic Fibrosis; Balanced Investment to Maintain Financial Strength

## - Broad telaprevir registration program for treatment-naive and treatment-failure HCV patients nears enrollment completion -- Vertex expects to initiate VX-770 registration program in cystic fibrosis -- Vertex ends 2008 with approximately $830 million in cash, cash equivalents & marketable securities -

SAN FRANCISCO, Jan 12, 2009 (BUSINESS WIRE) --

Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today provided an update on the Company's clinical development programs and announced 2009 business priorities in conjunction with the 27th Annual J.P. Morgan Healthcare Conference in San Francisco. The Company also provided an update on its cash position and outlined its strategy for maintaining financial strength in 2009. Joshua Boger, Ph.D., President and Chief Executive Officer of Vertex, will discuss recent progress and provide an overview of Vertex's 2009 objectives as part of a live webcast presentation, which will be available on Vertex's website, www.vrtx.com, on Tuesday, January 13 at 1:30 p.m. PT (4:30 p.m. ET).

"Our industry's greatest successes - of which there are many - resulted from transformational products that improved patients' lives and delivered real value to the health care system," said Dr. Boger. "The companies that tackled the most serious unmet medical needs, such as HIV, cancer and cardiovascular disease, are today the companies that are the anchors of the biotechnology industry. At Vertex, we target nothing less than some of the world's most devastating diseases. Today we have two major medicines in clinical development for hepatitis C and cystic fibrosis that we believe have the potential to drive major advances in the treatment of these diseases.

"In clinical trials to date, telaprevir has demonstrated an unprecedented ability to improve outcomes for both treatment-naive and treatment-failure patients chronically infected with the hepatitis C virus," continued Dr. Boger. "Enrollment is expected to be complete by the end of the first quarter for telaprevir's broad registration program, positioning Vertex to potentially be first to market with a highly differentiated direct acting therapy for patients with HCV.

"Beyond HCV, Vertex is finalizing the design of a registration program for VX-770 in patients with cystic fibrosis. By targeting the underlying defect in CF, VX-770 has shown the potential in early clinical trials to increase lung function in patients with the G551D mutation and enhance the function of the defective protein responsible for this disease. We look forward to commencing the registration program for VX-770, which will be focused on adult and pediatric patients with the G551D mutation."

### Broad Clinical Development Program for Telaprevir

#### *ADVANCE and ILLUMINATE trials in treatment-naive patients fully enrolled*

- Vertex today announced that the ILLUMINATE clinical trial is fully enrolled. Together with the ADVANCE clinical trial, which was fully enrolled in October 2008, Vertex has enrolled more than 1,500 genotype 1 treatment-naive HCV patients as part of the Company's broad registration program for telaprevir:
  - ADVANCE: Vertex and Tibotec completed enrollment in October 2008 in the global 3-arm pivotal Phase 3 ADVANCE trial that is focused on 24-week telaprevir-based response-guided regimens in genotype 1 treatment-naive HCV patients. In the ADVANCE trial, telaprevir is being dosed for 8 or 12 weeks. All patients are expected to have completed 8 or 12 weeks of dosing with telaprevir or placebo by the end of January 2009. Vertex expects to have sustained viral response (SVR) data from the ADVANCE trial in the first half of 2010. The ADVANCE trial enrolled approximately 1,050 patients.
  - ILLUMINATE: Vertex today announced that the Company has completed enrollment in the global 2-arm ILLUMINATE trial that will include evaluation of 24-week and 48-week telaprevir-based regimens in genotype 1 treatment-naive HCV patients. In the ILLUMINATE trial, telaprevir is being dosed for 12 weeks. The Company expects to have SVR data from the ILLUMINATE trial in the first half of 2010, which will supplement SVR data obtained from the pivotal Phase 3 ADVANCE trial. The ILLUMINATE trial enrolled approximately 500 patients.

***Differentiated profile in treatment-failure patients***

- Full enrollment of approximately 650 patients in the global 3-arm pivotal Phase 3 REALIZE clinical trial is expected in the first quarter of 2009. This trial is focused on 48-week telaprevir-based regimens in genotype 1 HCV patients who failed to achieve SVR with prior treatment of pegylated interferon (peg-IFN) and ribavirin (RBV). The REALIZE trial is expected to enroll relapser, partial responder and the most difficult to treat null responder patients who are well-documented with respect to their prior response to HCV therapy. In the REALIZE trial, telaprevir is being dosed for 12 weeks.

- Vertex presented interim clinical data, including safety information, from the Phase 2 PROVE 3 trial in November 2008 at AASLD. The data showed a 52% SVR12 rate in treatment-failure HCV patients, with a 24-week telaprevir-based treatment regimen. All patients in PROVE 3 completed dosing in the second quarter of 2008. Vertex expects to present final SVR data from the telaprevir and control arms of PROVE 3 at a medical meeting in the first half of 2009.

***Vertex exploring utility of telaprevir in other patient populations and dosing regimens***

- Interim results presented in November 2008 from the ongoing, Phase 2, open-label, randomized C208 study examining a twice-daily (q12h) telaprevir dosing regimen versus a three-times-daily (q8h) regimen in combination with RBV and peg-IFN-alfa-2a (PEGASYS(R)) or peg-IFN-alfa-2b (PEGINTRON(TM)) in treatment-naive genotype 1 HCV patients, including safety information from the study, support the potential for twice-daily dosing of telaprevir. Tibotec expects to present additional data from this trial, including SVR data for patients who completed dosing and have been followed 24 weeks post-treatment, at a medical meeting in 2009.
- Vertex and Tibotec plan to discuss a proposed HIV/HCV co-infection program with the U.S. FDA and European health authorities in the coming months.

***HCV portfolio strategy advancing***

- Vertex is also developing VX-500 and VX-813, additional HCV protease inhibitors that are currently in Phase 1 clinical development. Vertex expects to have safety, pharmacokinetic and viral kinetic data for VX-500 from Phase 1 studies in the first quarter of 2009.
- The combination of telaprevir with other novel specifically targeted antiviral therapies for HCV (STAT-C) is a key business priority for Vertex in 2009. The Company continues to evaluate other STAT-C agents as they advance in clinical development.

***Vertex collaborator initiates Phase 3 clinical development in Japan***

- Vertex today announced that its collaborator Mitsubishi Tanabe Pharma Corporation has initiated Phase 3 clinical development of telaprevir that is designed to include evaluation of 24-week telaprevir-based regimens in approximately 300 genotype 1 treatment-naive and treatment-failure HCV patients in Japan. In Phase 3 studies of telaprevir being conducted by Mitsubishi, telaprevir is being dosed for 12 weeks in combination with peg-IFN-alfa-2b (PEGINTRON(TM)) and RBV. Mitsubishi expects to have SVR data from its Phase 3 clinical trials of telaprevir in mid-2011.

**Two Novel Compounds for Treating the Underlying Defect in Cystic Fibrosis**

***VX-770 registration program to begin in first half of 2009***

- Based on positive results announced in 2008 from a Phase 2 trial of VX-770, an investigational oral Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) potentiator compound, in patients with CF who carry the G551D mutation in *CFTR*, Vertex expects to initiate a registration program for VX-770 in the first half of 2009. VX-770 is intended to increase chloride ion transport through the defective CFTR protein.
- The VX-770 registration program will be focused on CF patients who carry the G551D mutation. The registration program is expected to consist of three separate clinical trials:
    - The primary trial in the VX-770 registration program will enroll patients ages 12 and older who carry the G551D mutation on at least one allele. The trial is expected to evaluate VX-770 administered orally twice daily compared to placebo. Vertex expects to initiate this trial in the first half of 2009.
    - A clinical trial in patients aged 6 to 11 with the G551D mutation on at least one allele will also be part of the VX-770 registration program. This trial is expected to evaluate VX-770 administered orally twice daily compared to placebo.
    - Vertex will also conduct a clinical trial of VX-770 that will enroll CF patients with the F508del mutation on both *CFTR* alleles. The trial will evaluate VX-770 when administered orally twice daily. This trial will provide additional safety data for the VX-770 registration program and will be the first clinical trial to evaluate the clinical activity of VX-770 in patients with the F508del mutation on both alleles.
    - The primary endpoint of all trials in the VX-770 registration program will be FEV1. Additional secondary endpoints,

including sweat chloride, will also be measured to determine the effect of VX-770 on helping to restore the function of the defective CFTR protein.
    - Vertex is currently working with global regulatory authorities to finalize the design of the VX-770 registration program and, pending agreement with regulatory authorities, is on track to begin the registration program in the first half of 2009.

***VX-809 positioned to enter Phase 2 development***

- Based on safety and pharmacokinetic data from Phase 1 trials of VX-809, an investigational oral CFTR corrector compound, Vertex has initiated a single dose pharmacokinetic and safety trial of VX-809 in patients who carry the F508del mutation on at least one allele. Vertex expects to initiate a Phase 2a study in patients with CF in the first half of 2009. Corrector compounds such as VX-809 are designed to increase the amount of F508del-CFTR on the surface of cells lining the airway and may also increase chloride ion transport across the cell surface through the defective CFTR protein. The F508del mutation is the most common mutation and is present on at least one allele in approximately 90 percent of CF patients. *In vitro,* correctors have shown the ability to restore defective F508del-CFTR protein toward a wild-type state, with increased trafficking of F508del-CFTR to the cell surface and enhanced gating activity of F508del-CFTR on the cell surface.

**JAK3 Inhibitor for Multiple Immune-Mediated Inflammatory Diseases**

- Vertex is nearing completion of a Phase 1 clinical trial of VX-509, a novel Janus kinase 3 (JAK3) inhibitor. It is anticipated that VX-509 will be investigated for the treatment of multiple immune-mediated inflammatory diseases.
- As part of its business development initiatives in 2009, Vertex may seek to out-license VX-509 to fund and support R&D investment and continued financial strength.

**2009 Financial Strategy**

This section contains forward-looking guidance about the financial outlook for Vertex Pharmaceuticals.

"Telaprevir and VX-770 continue to drive the growth of our business, and with a strong cash position entering 2009, Vertex is well-positioned to support the continued development and commercialization of these potentially breakthrough medicines," said Ian Smith, Executive Vice President and Chief Financial Officer of Vertex. "We believe Vertex is prepared to advance its business in 2009, however we realize that a challenging year lies ahead for our industry and for the global financial markets. At Vertex, we recognize these changing market dynamics and will seek to make disciplined investment decisions to protect our current balance sheet strength and support key business objectives throughout the year."

- As of December 31, 2008, Vertex had approximately $830 million in cash, cash equivalents and marketable securities.
- Vertex anticipates a GAAP net loss for 2008, including restructuring charges and stock-based compensation expense, of approximately $460 million. Vertex anticipates a 2008 non-GAAP loss, excluding restructuring charges and stock-based compensation expense, of approximately $400 million. The 2008 GAAP net loss includes an estimate of approximately $60 million in stock-based compensation expense and restructuring expense.
- Vertex anticipates a GAAP net loss for 2009, including restructuring charges and stock-based compensation expense, of approximately $475 to $510 million. Vertex expects a 2009 non-GAAP loss, excluding restructuring charges and stock-based compensation expense, of approximately $400 to $435 million. The 2009 GAAP net loss includes an estimate of approximately $75 million in stock-based compensation expense and restructuring expense.
- Vertex expects that revenue from business development activities, including the out-licensing of certain early-stage assets and the completion of transactions to provide alternative, non-dilutive forms of capital to Vertex, will help support and fund R&D investment and balance sheet strength in 2009.
- Vertex will report full-year 2008 financial results and full financial guidance for 2009 on February 11, 2009.

**Non-GAAP Financial Measures**

In this press release, Vertex's financial results are provided both in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, Vertex provides guidance for its full-year 2008 loss and projected 2009 loss, excluding restructuring charges and stock-based compensation expense, which in each case results in a non-GAAP financial measure. These results are 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 are important in comparing current results with prior period results. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally, and to manage the Company's business and to evaluate its performance.

**Webcast**

Vertex Pharmaceuticals will webcast its corporate presentation at the 27th Annual J.P. Morgan Healthcare Conference on January 13, 2009 at 1:30 p.m. PT (4:30 p.m. ET). A link to the live webcast will be available via Vertex's website, www.vrtx.com, in the Events & Presentations section. An archived webcast of the presentation will be available on Vertex's website through January 26, 2009.

**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 focused on viral diseases, inflammation, autoimmune diseases, cancer, pain and cystic fibrosis. Vertex co-discovered the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

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

**Special Note Regarding Forward-looking Statements**

This press release contains forward-looking statements, including statements regarding (i) the Company's targeting of some of the world's most devastating diseases and the potential for our two major medicines in clinical development for HCV and CF to drive major advances in the treatment of these diseases, (ii) the expectation that enrollment of the telaprevir registration program will be completed by the end of the first quarter of 2009, positioning Vertex to potentially be first to market with a highly differentiated direct acting therapy for patients with HCV, (iii) finalizing the design of a registration program for VX-770, (iv) Vertex's expectation that it will commence the registration program for VX-770 in the first half of 2009 and the work Vertex is engaging in with global regulatory authorities to finalize the design of this program, (v) the expectation that all patients in the ADVANCE clinical trial will have completed 8 or 12 weeks of dosing with telaprevir or placebo by the end of January 2009, (vi) the expected dates by which the Company will have SVR data from the ADVANCE and ILLUMINATE clinical trials, (vii) the expectation that SVR data from ILLUMINATE will supplement the data from ADVANCE, (viii) the expectation that enrollment in the REALIZE clinical trial will be completed in the first quarter of 2009, (ix) the expectation that additional data from the PROVE 3 and C208 clinical trials will be presented at a medical meeting in the first half of 2009, (x) the potential for twice-daily dosing of telaprevir, (xi) the Company's plans to discuss a proposed HIV/HCV co-infection program with the U.S. FDA and European health authorities in the coming months, (xii) Vertex's expectation that it will have data from Phase 1 clinical trials of VX-500 in the first quarter of 2009, (xiii) combination therapy of telaprevir with other novel specifically targeted antiviral therapies being a key business priority in 2009, (xiv) Mitsubishi Tanabe expecting to have SVR data from its Phase 3 clinical trials of telaprevir in mid-2011, (xv) the expected clinical trial designs for the registration program for VX-770, including the evaluation of pediatric patients and patients with the F508del mutation, and the expected primary and secondary endpoints for the VX-770 trials, (xvi) the expected initiation of a Phase 2a clinical trial of VX-809 in the first half of 2009, (xvii) the potential effect of corrector compounds on chloride ion transport, (xviii) the expected completion of a Phase 1 clinical trial of VX-509 and the expectation that VX-509 will be investigated for the treatment of multiple immune-mediated inflammatory diseases, (xix) the possibility that Vertex may seek to outlicense VX-509 to fund and support R&D investment and continued financial strength, (xx) the Company's statements regarding its financial position and strength, anticipated 2008 GAAP and Non-GAAP net loss, projected 2009 GAAP and Non-GAAP loss, and expenses (xxi) the Company's belief that it is prepared to advance its business in 2009, (xxii) the Company's intention to protect its current balance sheet strength and support key business objectives throughout the year, and (xxiii) expectations regarding revenue from business development activities helping support balanced R&D investment and balance sheet strength in 2009. While the Company believes the forward-looking statements contained in this press release are accurate, there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that the outcomes for each of its planned and ongoing clinical trials and studies, and in particular its planned and ongoing clinical trials of telaprevir and VX-770, may not be favorable, that regulatory authorities may require supplemental clinical trials in order to support registration of telaprevir in any particular indication, that the Company will not be able to secure agreement from regulatory authorities on a registration program for VX-770, that there may be varying interpretations of data produced in one or more of our clinical trials, that enrollment may be more difficult or slower than we currently anticipate or that planned clinical trials may not start when planned due to regulatory issues, site startup delays, availability of clinical trial material or other reasons, that regulatory authorities will require more extensive data for a telaprevir or VX-770 NDA filing than currently expected, that *in vitro* data may not be predictive of clinical outcomes, that one or more of the Company's assumptions underlying its net loss expectations for 2009 -- including clinical and scientific progress that could lead to payments under new collaborations, anticipated expenses, and variables that go into determining stock-based compensation expenses -- will not be realized, that unexpected costs associated with one or more of the Company's programs will necessitate a change in the Company's financial projections, that the Company's 2008 annual net loss may differ from the anticipated net loss stated above, that future competitive or other market factors may adversely affect the commercial potential for the Company's product candidates in HCV or cystic fibrosis, that business development opportunities to support the Company's R&D investment and balance sheet strength in 2009 may not be available on acceptable terms and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the Company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

(VRTX-GEN)

SOURCE: Vertex Pharmaceuticals Incorporated

Michael Partridge, 617-444-6108
(at the J.P. Morgan Healthcare conference: 617-767-6108)
Senior Director, Strategic Communications
or
Lora Pike, 617-444-6755
(at the J.P. Morgan Healthcare conference: 857-413-0947)
Manager, Investor Relations
or
Zachry Barber, 617-444-6470
(at the J.P. Morgan Healthcare Conference: 617-767-9533)
Manager, Media Relations