



January 10, 2010

## **Vertex Reviews 2010 Business Priorities to Support Goal of Becoming Fully-Capable Biopharmaceutical Company**

***-HCV: New Drug Application planned for telaprevir in second half of 2010, robust commercialization and launch preparedness activities ongoing-***  
***-CF: Advancing development efforts in orphan disease of cystic fibrosis; Phase 3 STRIVE trial with VX-770 completes planned enrollment, VX-809 Phase 2 data expected in first quarter 2010-***  
***-Pipeline: Proof-of-concept clinical trials planned for 2010 with novel combination regimens for hepatitis C and cystic fibrosis and with compounds for rheumatoid arthritis and epilepsy-***  
***-Financial: Vertex enters 2010 with approximately \$1.3 billion in cash, cash equivalents & marketable securities and approximately \$32 million in outstanding convertible debt-***

SAN FRANCISCO, Jan 10, 2010 (BUSINESS WIRE) -- [Vertex Pharmaceuticals Incorporated](http://www.vrtx.com) (NASDAQ: VRTX) today provided an update on key 2010 business priorities in conjunction with the 28th Annual J.P. Morgan Healthcare Conference in San Francisco. The company also discussed recent progress in its lead development programs in hepatitis C virus (HCV) infection and cystic fibrosis (CF) and outlined proof-of-concept clinical trials planned in other serious diseases for 2010.

"2010 will be a defining year for Vertex as we seek to evolve into a fully-capable biopharmaceutical company," said Matthew Emmens, Chairman, President and Chief Executive Officer of Vertex. "With ongoing Phase 3 programs in hepatitis C virus infection and cystic fibrosis, a broad pipeline of other emerging product candidate opportunities and a strong capital structure, we believe Vertex today has a unique opportunity to build a successful company focused on bringing multiple important therapies to patients. In 2010, we look forward to a series of defining events from late-stage and proof-of-concept clinical trials that may support further growth in the years ahead."

Mr. Emmens will deliver a live webcast presentation from the J.P. Morgan Healthcare Conference on Tuesday, January 12 at 2:30 p.m. PT (5:30 p.m. ET) where he will discuss recent clinical progress and provide an overview of Vertex's 2010 priorities. The webcast will be available on Vertex's website, [www.vrtx.com](http://www.vrtx.com).

"Phase 3 data for telaprevir, our lead drug candidate for the treatment of hepatitis C virus infection, will begin to emerge in the spring of 2010 to support the planned submission of a New Drug Application in the second half of this year. Our more than decade-long commitment to improving patient care in HCV is unwavering, and the Phase 3 program for telaprevir will remain our primary focus over the coming year. Importantly, we also recognize the need for continued innovation in the treatment of this disease, and we are preparing to initiate the first clinical trial combining telaprevir with the investigational HCV polymerase inhibitor VX-222 this quarter," stated Mr. Emmens.

"Beyond HCV, Vertex is conducting mid-stage and late-stage development of two novel compounds aimed at addressing, for the first time, the underlying mechanism of the orphan disease of cystic fibrosis. The VX-770 Phase 3 registration program is advancing rapidly, and we expect to obtain Phase 3 data for VX-770 early in 2011. Additionally, we also expect to obtain clinical data from a Phase 2 trial of VX-809 in the coming weeks that could potentially support the evaluation of VX-770 and VX-809 as part of a combination regimen in patients with the most common mutation of this disease.

"Supporting our vision to become a fully-capable biopharmaceutical company, Vertex is also planning multiple proof-of-concept clinical trials in other diseases, such as rheumatoid arthritis and epilepsy, and remains committed to maintaining investment into research to enable future product opportunities," concluded Mr. Emmens.

### **Phase 3 Registration Program for Telaprevir Nears Completion**

***Sustained viral response (SVR) data expected from Phase 3 ADVANCE trial in second quarter 2010 and from Phase 3 ILLUMINATE & REALIZE trials in third quarter 2010***

- The ADVANCE, ILLUMINATE and REALIZE trials are evaluating telaprevir-based regimens as part of a global Phase 3 registration program in more than 2,200 genotype 1 treatment-naïve and treatment-failure patients with HCV infection.
- Vertex today announced that all patients in the ADVANCE and ILLUMINATE trials, which are evaluating telaprevir in

treatment-naïve patients, have completed dosing of all study drugs, including pegylated-interferon (peg-IFN) and ribavirin (RBV), and are now in the post-treatment follow-up period to determine the number of patients who achieve SVR (defined as undetectable HCV RNA 24 weeks after the end of treatment). Vertex expects SVR data to become available from ADVANCE in the second quarter of 2010 and from ILLUMINATE in the third quarter of 2010.

- Vertex today also announced that all patients in the REALIZE trial, which is being conducted by Vertex's collaborator Tibotec and is evaluating telaprevir in patients who did not achieve SVR with a prior pegylated interferon-based treatment, are expected to complete dosing of all study drugs, including pegylated-interferon and ribavirin, by the end of January. Vertex expects SVR data to become available from REALIZE in the third quarter of 2010.
- Vertex plans to submit a New Drug Application (NDA) for telaprevir in the second half of 2010 for both treatment-naïve and treatment-failure patients.

### ***Twice-daily dosing of telaprevir***

- In November 2009, Vertex announced SVR results from Study C208, an exploratory Phase 2, open-label clinical study of telaprevir. The study was conducted by Tibotec in Europe and evaluated a twice-daily (1125mg q12h) dosing schedule of telaprevir in combination with peg-IFN-alfa-2a (PEGASYS<sup>(R)</sup>) or peg-IFN-alfa-2b (PEGINTRON<sup>(R)</sup>) and RBV, as compared to the three-times-daily (750mg q8h) telaprevir dosing schedule used in telaprevir clinical trials to date. The C208 trial enrolled 161 treatment-naïve patients with genotype 1 HCV infection. The C208 data included the first SVR data for telaprevir-based regimens in treatment-naïve patients as part of a response-guided therapy trial design, similar to that being used in the ADVANCE and ILLUMINATE Phase 3 trials of telaprevir.
- Vertex has submitted the Study C208 clinical data to the U.S. FDA, and Vertex and Tibotec have initiated discussions with regulatory authorities in the U.S. and E.U. regarding future development plans for telaprevir as part of an every-12-hour (q12h) dosing regimen.

### ***Vertex collaborator completes dosing of telaprevir in Phase 3 trials in Japan***

- Vertex today announced that its collaborator, Mitsubishi Tanabe Pharma Corporation, has completed the dosing portion for telaprevir in three ongoing Phase 3 trials of telaprevir-based combination therapy in approximately 300 treatment-naïve and treatment-failure HCV patients in Japan.

### ***New capabilities to support potential launch of telaprevir***

- Vertex continues to build its commercial infrastructure in preparation for the potential launch of telaprevir and has begun to develop and implement internal systems and processes to support the company's commercial operation and the potential future sale of telaprevir.
- The company expects to establish key elements of its customer-facing operation in 2010, including the hiring of the sales management team to be charged with the implementation of a fully-functioning commercial sales force in 2011.
- To inform launch preparedness activities, Vertex continues to conduct extensive HCV market research and is in discussions with key managed markets organizations and specialty pharmacies to generate additional insights on the HCV patient population, HCV patient care and flow, market communication strategies, and reimbursement processes for the HCV market.
- Vertex has assembled a global supply chain network to support the potential launch of telaprevir and is currently manufacturing telaprevir at a commercial (metric ton) scale. The company has successfully completed all registration campaigns as well as validation campaigns of the active pharmaceutical ingredient (API) and is prepared to complete drug product validation in advance of the potential launch of telaprevir.

### **Potential Future Combination Regimens for HCV with Telaprevir and the HCV Polymerase Inhibitor VX-222**

#### ***Initiation of first clinical trial of telaprevir combined with VX-222 planned for first quarter 2010***

- Vertex recently completed a multiple-dose Phase 1b viral kinetic study of the investigational oral HCV polymerase inhibitor VX-222. Interim results from the trial are consistent with the findings of a previously-conducted three-day viral kinetic study and support future clinical evaluation of VX-222, including the initiation of the first clinical trial of VX-222 in combination with telaprevir. Additional results from this Phase 1b study of VX-222 are planned for presentation at a medical meeting in 2010.
- Upon completion of ongoing discussions with regulatory authorities, Vertex plans to initiate a combination trial of telaprevir and VX-222 in the first quarter of 2010. This trial is expected to evaluate SVR rates using multiple regimens of telaprevir/VX-222-based therapy in HCV patients.

## Addressing the Underlying Defect of Cystic Fibrosis

### ***VX-770 Phase 3 registration program progressing; STRIVE trial completes planned enrollment***

- Vertex is currently conducting the ENDEAVOR Phase 3 registration program of VX-770, an investigational Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) potentiator compound for the treatment of CF. The program consists of three ongoing clinical trials, known as STRIVE, ENVISION and DISCOVER, and is designed to evaluate the utility of VX-770 across different age groups and genotypes, including children as young as six years of age.
- Vertex today announced that the STRIVE trial, which is evaluating VX-770 in patients aged 12 years and older who carry the G551D mutation on at least one allele, has completed planned patient enrollment. To date, more than 120 patients have enrolled in the STRIVE trial and additional patients currently are completing the screening process. Vertex expects that all patients in STRIVE will have received their first dose of VX-770 or placebo by the end of February 2010.
- The ENVISION trial, which is evaluating VX-770 in patients aged six to 11 who carry the G551D mutation on at least one allele, is ongoing. Vertex expects to complete enrollment for ENVISION in the first half of 2010.
- Vertex today also announced that the DISCOVER trial, which is evaluating VX-770 in patients 12 years and older who are homozygous for the F508del mutation, has enrolled more than 60 patients and additional patients currently are completing the screening process. Vertex expects to enroll approximately 120 patients in DISCOVER and that all patients will have received their first dose of VX-770 or placebo by the end of February 2010.
- The primary endpoint for patients with the G551D mutation (STRIVE and ENVISION trials) is change in forced expiratory volume in one second (FEV<sub>1</sub>), which will be measured at 24 weeks, with additional FEV<sub>1</sub> measurements at 48 weeks as a secondary endpoint to assess durability of any observed response. Patients in the STRIVE and ENVISION trials will receive either VX-770 or placebo for 48 weeks to gain additional safety data in G551D patients. For patients with the F508del mutations (DISCOVER trial), the primary endpoints are safety and change in FEV<sub>1</sub>, which will be measured at 16 weeks. Additional secondary endpoints, including sweat chloride, will be measured in each trial to evaluate the potential effect of VX-770 on improving the function of the defective CFTR protein.
- Based on 48-week clinical data from the STRIVE and ENVISION trials, and on 16-week clinical data from the DISCOVER trial, Vertex plans to submit an NDA for VX-770 in patients with the G551D CFTR mutation in the second half of 2011.

### ***Phase 2a trial of VX-809 complete; Data expected in first quarter 2010***

- Vertex recently completed a Phase 2a trial of VX-809, an investigational CFTR corrector compound for the treatment of CF, in patients homozygous for the F508del CFTR mutation.
- Vertex expects to obtain clinical data from the trial, including measures of sweat chloride and nasal potential difference, in the first quarter of 2010. These data could potentially support the initiation of a combination trial of VX-770 and VX-809 in the second half of 2010, with the goal of generating initial proof-of-concept clinical data in late 2010.

## Additional Proof-of-Concept Trials and Research Progress Support Future Growth

### ***Janus kinase 3 (JAK3) inhibitor VX-509 to enter Phase 2 trial in rheumatoid arthritis***

- Vertex plans to initiate a Phase 2 proof-of-concept clinical trial of VX-509 in approximately 200 patients with moderate to severe rheumatoid arthritis (RA) in the first quarter of 2010. The company expects to obtain interim clinical data, including measurements of safety, tolerability and clinical activity, in the second half of 2010.
- The double-blind, randomized, placebo-controlled trial will evaluate the safety, tolerability and clinical activity of four doses of VX-509. Patients will receive 12 weeks of treatment with VX-509 dosed twice daily compared to placebo.
- The primary endpoints of the trial are to evaluate safety and to measure the improvement in clinical signs and symptoms of RA in patients after 12 weeks of treatment. Efficacy assessments will include the American College of Rheumatology criteria (ACR20, ACR50 and ACR70) for defining clinical improvement in RA patients. ACR20, ACR50 and ACR70 are standardized measures of the number of patients who achieve at least a 20, 50 or 70 percent improvement, respectively, in ACR-specified measures of RA activity. The trial will also utilize disease activity score (DAS) and European League Against Rheumatism (EULAR) response criteria as additional efficacy assessments.
- VX-509 may have broad potential for the treatment of multiple immune-mediated inflammatory diseases. Vertex plans to pursue collaborative opportunities for VX-509 with major pharmaceutical companies.

### ***Caspase-1 inhibitor VX-765 to enter Phase 2 trial in epilepsy***

- Vertex plans to initiate a Phase 2 proof-of-concept clinical trial of VX-765 in patients with epilepsy in the first quarter of 2010, which could result in interim clinical data being available as early as the second half of 2010.

- VX-765 has been shown to inhibit acute seizures in preclinical models of chronic epilepsy and has shown activity in preclinical models of chronic epilepsy that do not respond to standard anti-epileptic drugs.
- The Phase 2 trial announced today for VX-765 is expected to enroll approximately 75 patients with treatment-resistant epilepsy. The double-blind, randomized, placebo-controlled trial will evaluate the safety, tolerability and clinical activity of VX-765. Patients will receive six weeks of treatment with VX-765 following a six-week baseline period to monitor seizure frequency. The primary endpoints of the trial are safety and tolerability. The secondary endpoints will evaluate the clinical efficacy of VX-765 based on the percent reduction in seizure frequency and percent of patients with a 50 percent or greater reduction in seizure frequency, known as the responder-rate.

### **Capital Structure Supports Investment into Key Business Priorities**

"Vertex is committed to maintaining a strong balance sheet and capital structure that will support our mission to discover, develop and commercialize new medicines that provide high therapeutic benefit to patients with major diseases and deliver return to our shareholders," said Ian Smith, Executive Vice President and Chief Financial Officer of Vertex. "With a cash position of approximately \$1.3 billion and approximately \$32 million of 2013 convertible debt, Vertex enters 2010 in a strong financial position to support the advancement of our key development programs in HCV and CF and to generate important proof-of-concept clinical data in other significant diseases.

"As our late-stage compounds advance, the company's financial strategy will evolve toward ensuring that our potential marketed products provide financial support for continued investment in development programs and in product creation from research," continued Mr. Smith.

As of December 31, 2009, Vertex had approximately \$1.3 billion in cash, cash equivalents and marketable securities, approximately \$32 million of outstanding 2013 convertible notes and approximately 200 million shares outstanding.

Vertex anticipates a GAAP net loss for 2009 of less than \$650 million. Vertex anticipates a 2009 non-GAAP loss of less than \$515 million, excluding stock-based compensation and executive transition expenses, restructuring expense, acquisition-related expenses, loss on exchange of convertible subordinated notes, and interest expense related to the September 2009 financial transactions.

Vertex will report full-year 2009 financial results and financial guidance for 2010 on February 4, 2010.

### **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 2009 loss, excluding stock-based compensation and executive transition expenses, restructuring expense, acquisition-related expenses, loss on exchange of convertible subordinated notes, and interest expense related to the September 2009 financial transactions, which 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 28th Annual J.P. Morgan Healthcare Conference on January 12, 2010 at 2:30 p.m. PT (5:30 p.m. ET). A link to the live webcast will be available via Vertex's website, [www.vrtx.com](http://www.vrtx.com), in the Events & Presentations section. An archived webcast of the presentation will be available on Vertex's website through January 26, 2010.

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

Lexiva<sup>(R)</sup> is a registered trademark of the GlaxoSmithKline group of companies.

## Special Note Regarding Forward-looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including statements regarding (i) the potential to make NDA filings for telaprevir in the second half of 2010 and for VX-770 in the second half of 2011; (ii) the Company's expectations regarding the availability of clinical data from ADVANCE in the second quarter of 2010, from ILLUMINATE and REALIZE in the third quarter of 2010, from the VX-770 registration program early in 2011, and from the Phase 2 clinical trial of VX-809 in the first quarter of 2010; (iii) the initiation of planned proof-of-concept clinical trials with novel combination regimens for hepatitis C and cystic fibrosis and compounds for rheumatoid arthritis and epilepsy; (iv) Mr. Emmens' statements in the second and fourth through six paragraphs of this press release, including statements regarding planned or potential clinical trials, expected data from ongoing clinical trials, the company's priorities in 2010 and the company's opportunities for growth in 2010 and the years ahead; (v) the new activities Vertex is engaging in to support the potential launch of telaprevir; (vi) expectations regarding commencement of dosing and the number of patients in the STRIVE and DISCOVER clinical trials and the completion of enrollment for the ENVISION clinical trial; (vii) the timing of the initiation, the potential design and the date by which interim or final data would be obtained from the planned combination trial of telaprevir and VX-222, the potential combination trial of VX-770 and VX-809, the planned clinical trial of VX-509 and the planned clinical trial of VX-765; (viii) the potential of Vertex's drug candidates to treat the indications identified in the press release; (ix) the company's commitment to maintain a strong balance sheet, (x) the anticipation that the Company's projected GAAP and non-GAAP 2009 annual loss will be within the ranges set forth under the heading "Capital Structure Supports Investments Into Key Business Priorities" and that the Company's identified expenses for 2009 and capital structure, including its cash, cash equivalents and marketable securities balance, will be as set forth therein. 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 clinical trials and studies, and in particular its planned clinical trials of telaprevir, may not be favorable, that regulatory authorities may require supplemental clinical trials in order to support registration of telaprevir and/or VX-770, that planned or potential clinical trials may be delayed or may not be conducted, that the Company may not be able to successfully develop telaprevir, VX-770, VX-509, VX-765 or combination therapies involving telaprevir and VX-222 or VX-770 and VX-809, that the Company's expectations regarding its 2009 GAAP and non-GAAP net loss or financial position as of December 31, 2009 may be incorrect 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](http://www.vrtx.com). The Company disclaims any obligation to update the information contained in this press release as new information becomes available.

Vertex's press releases are available at [www.vrtx.com](http://www.vrtx.com).

(VRTX - GEN)

SOURCE: Vertex Pharmaceuticals Incorporated

Vertex Pharmaceuticals Incorporated

Investors:

Michael Partridge, 617-444-6108

or

Lora Pike, 617-444-6755

or

Media:

Zachry Barber, 617-444-6470 or 617-767-9533 (cell)

Copyright Business Wire 2010