



February 9, 2009

## Vertex Pharmaceuticals Provides HCV and Cystic Fibrosis Update and Reports 2008 Financial Results

CAMBRIDGE, Mass., Feb 09, 2009 (BUSINESS WIRE) -- [Vertex Pharmaceuticals Incorporated](#):

- *Telaprevir dosing in Phase 3 ADVANCE clinical trial complete and Phase 3 REALIZE clinical trial completes enrollment*
- *VX-770 expected to commence registration program for cystic fibrosis in first half of 2009*
- *Cash, cash equivalents and marketable securities are \$832 million as of December 31, 2008*

[Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today reviewed its recent development progress in hepatitis C (HCV) and cystic fibrosis (CF), reported consolidated financial results for the year ended December 31, 2008 and provided financial guidance for 2009.

"Vertex made great advancements in 2008 that have helped position us to progress with registration and commercialization of our major product opportunities in hepatitis C and cystic fibrosis," said Joshua Boger, Ph.D., Chief Executive Officer of Vertex Pharmaceuticals. "As we enter 2009, we are making steady progress with the broad telaprevir registration program and are on track to file an NDA for telaprevir in 2010."

"VX-770, our investigational CFTR potentiator compound for cystic fibrosis, emerged as a further potential product opportunity in 2008, and we look forward to the initiation of a registration program for this potentially major treatment advancement in the first half of 2009. Additionally, we expect the initiation of a Phase 2a study in CF patients with our lead investigational corrector compound, VX-809, which may further expand the opportunity to address the unmet medical need in CF," continued Dr. Boger.

Dr. Boger also acknowledged, "We are fortunate to have the financial strength to support the progress of these late-stage product opportunities during 2009, and we plan to make disciplined investments to help manage our balance sheet in this difficult financial environment."

### HCV - Telaprevir Registration Program and Additional Studies

#### ***Phase 3 registration program fully enrolled in treatment-naïve and treatment-failure HCV patients***

- Vertex announced that dosing of telaprevir or placebo (8 or 12 weeks, depending on treatment arm assignment), as part of the combination regimen with pegylated interferon (peg-IFN) and ribavirin (RBV), is complete in all patients enrolled in the Phase 3 ADVANCE trial. The global 3-arm pivotal Phase 3 trial is focused on 24-week telaprevir-based response-guided regimens in genotype 1 treatment-naïve HCV patients. Vertex expects to have sustained viral response (SVR) data from the ADVANCE trial in the first half of 2010. The ADVANCE trial completed enrollment of approximately 1,050 HCV patients in October 2008.
- In addition, Vertex completed enrollment of approximately 500 patients in December 2008 in the ILLUMINATE trial. On this basis, Vertex expects telaprevir dosing in ILLUMINATE to be complete in April 2009. In this trial, telaprevir is being dosed for 12 weeks in the combination regimen. The Company expects to have SVR data in the first half of 2010 from this trial. ILLUMINATE is a global 2-arm trial that is evaluating telaprevir-based response-guided regimens in genotype 1 treatment-naïve HCV patients. This trial is designed to supplement SVR data obtained from the pivotal Phase 3 ADVANCE trial and to evaluate the benefit/risk advantage, for patients who achieve a rapid viral response, of extending treatment with peg-IFN and RBV from 24 to 48 weeks.
- Vertex also today announced that enrollment in the global 3-arm pivotal Phase 3 REALIZE trial is complete with approximately 650 patients. The REALIZE trial, which is being conducted by Tibotec, is evaluating genotype 1 HCV patients who failed to achieve SVR with prior treatment of peg-IFN and RBV. The REALIZE trial enrolled all major treatment-failure groups, including hard to treat null responder patients. Vertex expects all telaprevir dosing to be complete in the REALIZE trial in May 2009.

#### ***Potential for Twice-Daily Profile***

Vertex and Tibotec are conducting additional clinical studies to evaluate the potential role of telaprevir treatment for important HCV sub-populations as well as part of different dosing regimens.

- Tibotec is conducting study C208, a Phase 2 clinical study in Europe that is evaluating 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. In an interim analysis, researchers reported at the AASLD conference in November 2008 that 80% or more of patients who received telaprevir twice daily or three times daily in combination with pegylated-interferon alfa-2a and ribavirin had undetectable HCV RNA at weeks 4 and 12. Researchers also reported that no significant differences in the safety profile or viral breakthrough rates between the twice daily and three-times-daily dosing regimens were observed at 12 weeks. The side effect profile was consistent with that seen in previous studies. These interim data support the potential for further evaluation of telaprevir in a twice-daily dosing regimen. Vertex expects SVR data from this study to be presented at a medical meeting in 2009.
- Vertex and Tibotec are planning to initiate a Phase 2 study in patients with HIV/HCV co-infection in the second half of 2009.

### ***Update on HCV portfolio strategy***

- In 2008, telaprevir demonstrated promising SVR results in large Phase 2b studies of treatment-naive and treatment-failure patients, and also demonstrated the potential to be dosed in a twice daily regimen.
- Vertex is conducting early-stage development of novel HCV protease inhibitors with the goal of identifying molecules for further development, including combination therapy, which could further advance the treatment of HCV.
- Today, Vertex announced that results of a Phase 1b dose-ranging, 3-day viral kinetic study with VX-500 in treatment-naive genotype 1 HCV patients did not meet criteria for further clinical advancement of the compound. The Company continues to advance other novel HCV protease inhibitors, including VX-813, which has completed a multi-dose Phase 1a study in healthy volunteers, and VX-985, which is in early development.
- Vertex is also committed to the clinical exploration of telaprevir in combination with other novel specifically targeted antiviral therapies for HCV (STAT-C).

Updates on the status of telaprevir clinical trials are available at [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

### **Broad Program Targeting Cystic Fibrosis Advancing**

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

- Vertex is currently working with global regulatory authorities to finalize the design of the registration program for VX-770, an investigational oral Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) potentiator compound, and, pending agreement with regulatory authorities, plans to begin the registration program 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 focus on CF patients who carry the G551D mutation. It will consist of three separate trials, including a primary trial designed to enroll patients ages 12 and older who carry the G551D mutation on at least one allele. Vertex expects to initiate this trial in the first half of 2009.
- In addition, as part of the registration program for VX-770, Vertex expects to evaluate pediatric patients aged 6 to 11 with the G551D mutation on at least one allele. Vertex will also evaluate CF patients with the F508del mutation on both alleles. 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.

#### ***VX-809 positioned to enter Phase 2 development for cystic fibrosis***

- Vertex has completed an escalating single dose pharmacokinetic and safety trial of the investigational oral CFTR corrector compound VX-809 in patients who carry the F508del mutation. Vertex expects to initiate a Phase 2a, 28-day study in patients with CF in the first half of 2009.

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

- Vertex has completed a Phase 1 clinical trial of VX-509, a novel Janus kinase 3 (JAK3) inhibitor. The Phase 1 trial studied three cohorts of healthy volunteers dosed for 14 days with ascending doses of VX-509. It is anticipated that a Phase 2 study in rheumatoid arthritis patients will commence in the second half of 2009. VX-509 is a potential candidate for investigation in the treatment of 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 other R&D investment.

### **Merck Conducting Aurora Kinase Inhibitor Clinical Development Program**

- Vertex's collaborator Merck is conducting a Phase 1 trial of the Aurora kinase MK-5108 (VX-689) alone and in combination with docetaxel in patients with advanced and/or refractory solid tumors.

## Full Year Results

For the year ended December 31, 2008, the Company's GAAP net loss was \$459.9 million, or \$3.27 per share, including \$62.3 million of stock-based compensation and restructuring charges. The GAAP net loss for the year ended December 31, 2007 was \$391.3 million, or \$3.03 per share, including \$66.5 million of stock-based compensation and restructuring charges.

The non-GAAP loss, before stock-based compensation and restructuring charges, for the year ended December 31, 2008 was \$397.5 million, or \$2.83 per share, compared to the non-GAAP loss, before stock-based compensation and restructuring and certain other non-recurring charges and gains, of \$324.8 million, or \$2.52 per share, for the year ended December 31, 2007. The increased loss is largely attributable to decreased total revenues, an increase in total operating costs and expenses to support telaprevir's global Phase 3 registration program and commercialization, and a reduction in net interest income.

Total revenues for the year ended December 31, 2008 were \$175.5 million, compared to \$199.0 million for 2007. The decrease is primarily due to a reduction in royalty revenues, due to the sale of the Company's HIV drug royalty stream in the second quarter of 2008, and a reduction in R&D collaborative revenues principally due to the completion of funding under the Company's collaborative agreement with the Cystic Fibrosis Foundation Therapeutics (CFFT) in early 2008.

Research and development (R&D) expenses for the year ended December 31, 2008 were \$516.3 million, compared to \$518.7 million in R&D expenses for 2007. Vertex's R&D investment is principally comprised of clinical investment in telaprevir, VX-770, and earlier-stage programs, drug discovery, and commercial supply investment for telaprevir. Vertex and Tibotec share the cost of development activities for telaprevir.

Sales, general and administrative (SG&A) expenses for the year ended December 31, 2008 were \$101.9 million, compared to \$79.1 million for 2007. This increase reflects building of infrastructure, including an increase in the number of employees and our initial commercial investments, to support advancement of the business.

Other income, net, for the year ended December 31, 2008 was \$2.9 million, compared to \$28.5 million for 2007. This decrease resulted from reduced portfolio yields, reflecting conditions in the broader economic environment, and interest expense incurred following the February 2008 issuance of the 2013 convertible senior subordinated debt.

At December 31, 2008, Vertex had approximately \$832.1 million in cash, cash equivalents and marketable securities.

## Full Year 2009 Financial Guidance

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

"We made significant progress in strengthening our balance sheet in 2008 in order to support our key investment areas of HCV and cystic fibrosis, and our investment into product creation," said Ian Smith, Executive Vice President and Chief Financial Officer of Vertex. "We enter 2009 with approximately \$832 million in cash and equivalents. We expect that collaborations may contribute additional capital to the Company during the course of the year and may favorably contribute to the management of our operating investment."

**Loss:** Vertex anticipates a GAAP net loss for 2009, including restructuring charges and stock-based compensation expense, in the range of \$495 to \$530 million. The 2009 GAAP net loss includes an estimate of approximately \$95 million in stock-based compensation expense and restructuring expense. Vertex expects that the 2009 non-GAAP loss, excluding restructuring charges and stock-based compensation expense, will be in the range of \$400 to \$435 million.

**Revenues:** Vertex expects that full-year 2009 total revenue will be in the range of \$140 to \$160 million. This range includes an estimated \$60 million to \$80 million of revenues generated from business development activities related to certain clinical product opportunities and current R&D collaborations.

**Research and Development Expense:** The Company expects that R&D expense will be in the range of \$500 to \$530 million for 2009, inclusive of approximately \$75 million of stock-based compensation expense. The principal development investment continues to be focused in HCV and CF, with the investment in research activities relatively consistent with prior years.

**Sales, General and Administrative (SG&A) Expense:** Vertex expects SG&A expense to be in the range of \$130 to \$140 million in 2009, inclusive of approximately \$16 million of stock-based compensation expense.

## 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 its fourth quarter and

full-year 2008 and 2007 loss and guidance for its 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. A reconciliation of non-GAAP financial results to GAAP financial results is included in the attached financial statements.

## **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.

PEGASYS is a registered trademark of Hoffman-La Roche Ltd. PEGINTRON is a registered trademark of Schering-Plough Corporation

## **Special Note Regarding Forward-looking Statements**

This press release contains forward-looking statements, including statements that (i) the Company expects that it will commence a registration program for VX-770 in the first half of 2009, (ii) the Company is positioned to progress to registration and commercialization of its major product opportunities in HCV and CF, (iii) the Company is making steady progress with the broad telaprevir registration program and is on track to file an NDA for telaprevir in 2010, (iv) the Company looks forward to the initiation of a registration program for this potentially major treatment advancement in the first half of 2009, (v) Vertex expects that it will initiate a Phase 2a study with VX-809 that may further expand the opportunity to address the unmet medical need in CF, (vi) the Company will make disciplined investments to manage its balance sheet strength in this difficult financial environment, (vii) set forth expected dates by which the Company will have SVR data from the ADVANCE and ILLUMINATE clinical trials, (viii) the Company expects SVR data from ILLUMINATE to supplement the data from ADVANCE, (ix) set forth expected dates by which telaprevir dosing will be complete in the ILLUMINATE trial and REALIZE trial, (x) reference potential for twice-daily dosing of telaprevir, (xi) the Company expects that additional data from the C208 clinical trial will be presented at a medical meeting in 2009, (xii) the Company plans to initiate a Phase 2 study in patients with HIV/HCV co-infection in the second half of 2009, (xiii) the Company intends to investigate combination therapy of telaprevir with other investigational HCV therapies in 2009, (xiv) the expected clinical trial designs for the registration program for VX-770, including the evaluation of pediatric patients and patients with the F508del mutation, will actually be conducted as planned (xv) the Company expects to initiate a Phase 2a, 28-day clinical trial of VX-809 in the first half of 2009, (xvi) the Company expects to initiate a Phase 2 clinical trial of VX-509 in rheumatoid arthritis in the second half of 2009, and the Company expects that VX-509 is a candidate for the treatment of immune-mediated inflammatory diseases, (xvii) the Company may seek to outlicense VX-509 in order to fund and support R&D investment and continued financial strength, (xviii) the Company's statements regarding its financial position and strength, (xix), the Company expects that collaborations will contribute additional capital during 2009 and contribute to the management of its operating investment, (xx) the Company's projected 2009 annual loss, revenues, R&D expense, and SG&A expense, will be within the ranges stated above in the Company's financial guidance, (xxi) the Company expects that its principal development investment will continue to be focused in HCV and CF, with the investment in research activities relatively consistent with prior years, (xii) the Company's estimates of its stock-based compensation expenses will be as stated above. 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 or 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 and potentially unfavorable interpretations of data produced by 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 NDA filing than currently expected, that one or more of the Company's assumptions underlying its revenue expectations -- including clinical and scientific progress that could lead to milestone payments under existing collaboration agreements or other payments under new collaborations -- or its expense expectations -- including estimates of the variables that go into determining stock-based compensation expenses -- will not be realized, or that Vertex will be unable to realize one or more of its financial objectives for 2009 due to unexpected and costly program delays or any number of other financial, technical or collaboration considerations, that unexpected costs associated with one or more of the Company's programs will necessitate a reduction in its investment in other programs or a change in the Company's financial projections, that future competitive or other market factors may adversely affect the commercial potential for the Company's product candidates in

HCV or other potential indications, that due to scientific, medical or technical developments, the Company's drug discovery efforts will not ultimately result in commercial products or assets that can generate revenue, that the Company will be unable to enter into new collaborative relationships 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](http://www.vrtx.com). We disclaim any obligation to update the information contained in this press release as new data become available.

**Vertex Pharmaceuticals Incorporated**  
**2008 Fourth Quarter and Twelve Month Results**  
**Consolidated Statements of Operations Data**

(In thousands, except per share amounts)  
(Unaudited)

	Three Months Ended		Twelve Months Ended	
	December 31,		December 31,	
	2008	2007	2008	2007
Revenues:				
Royalty revenues (Note 6)	\$ 9,128	\$ 14,688	\$ 37,483	\$ 47,973
Collaborative and other R&D revenues	23,683	36,304	138,021	151,039
<b>Total revenues</b>	<b>32,811</b>	<b>50,992</b>	<b>175,504</b>	<b>199,012</b>
Costs and expenses:				
Royalty expenses (Note 6)	4,215	3,672	15,686	13,904
Research and development expenses (R&D) (Note 1)	139,162	117,586	516,292	518,677
Sales, general & administrative expenses (SG&A) (Note 1)	29,656	21,206	101,910	79,104
Restructuring expense	1,641	276	4,324	7,119
<b>Total costs and expenses</b>	<b>174,674</b>	<b>142,740</b>	<b>638,212</b>	<b>618,804</b>
Loss from operations	(141,863)	(91,748)	(462,708)	(419,792)
Net interest income (expense)	(469)	5,997	2,857	28,513
<b>Net loss</b>	<b>\$(142,332)</b>	<b>\$(85,751)</b>	<b>\$(459,851)</b>	<b>\$(391,279)</b>
Basic and diluted net loss per common share	\$(0.96)	\$(0.66)	\$(3.27)	\$(3.03)
Basic and diluted weighted-average number of common shares outstanding	148,783	130,741	140,556	128,986

**Non-GAAP Loss and Loss per Common Share Reconciliation**

	Three Months Ended		Twelve Months Ended	
	December 31,		December 31,	
	2008	2007	2008	2007
GAAP Net Loss	\$(142,332)	\$(85,751)	\$(459,851)	\$(391,279)
Pro Forma Adjustments:				
Stock-based compensation expense	\$ 10,576	\$ 10,077	\$ 45,524	\$ 48,418
included in R&D (Note 1&2):				
Stock-based compensation expense				
included in SG&A (Note 1&2):	3,261	2,581	12,463	10,989
<b>Total stock-based compensation expense</b>	<b>13,837</b>	<b>12,658</b>	<b>57,987</b>	<b>59,407</b>
Restructuring expense (Note 3)	1,641	276	4,324	7,119
<b>Non-GAAP Loss</b>	<b>\$(126,854)</b>	<b>\$(72,817)</b>	<b>\$(397,540)</b>	<b>\$(324,753)</b>



(Unaudited)

	December 31, December 31,	
	2008	2007
<b>Assets</b>		
Cash, cash equivalents and marketable securities	\$ 832,101	\$ 467,796
Other current assets	35,480	35,980
Property and equipment, net	68,331	66,509
Restricted cash	30,258	30,258
Other non-current assets (Notes 5 & 6)	14,309	934
Total assets	\$ 980,479	\$ 601,477

**Liabilities and Stockholders' Equity**

Other current liabilities	\$ 172,567	\$ 148,148
Accrued restructuring expense	34,064	35,292
Deferred revenues (Note 6)	247,474	126,745
Collaborator development loan (due May 2008)	---	19,997
Convertible notes (due 2013)(Note 5)	287,500	---
Stockholders' equity	238,874	271,295
Total liabilities and stockholders' equity	\$ 980,479	\$ 601,477
Common shares outstanding (Note 4)	151,245	132,876

**Conference Call and Webcast: Full Year 2008 Financial Results:**

Vertex Pharmaceuticals will host a conference call and webcast today, Monday, February 9, 2009 at 5:00 p.m. EST to review financial results and recent developments. This call and webcast will be broadcast via the Internet at [www.vrtx.com](http://www.vrtx.com). Updates to Vertex's web site may require users to install or update their Flash player. It is suggested that webcast participants go to the web site at least 10 minutes in advance of the call to ensure that they can access the slides. The link to the webcast is available on the "Events and Presentations" button on the home page.

To listen to the call on the telephone, dial (800) 374-0296 (U.S. and Canada) or (702) 696 - 4937 (International). Vertex is also providing a podcast MP3 file available for download on the Vertex website at [www.vrtx.com](http://www.vrtx.com).

The call will be available for replay via telephone commencing February 9, 2009 at 8:00 p.m. EST running through 5:00 p.m. EST on February 16, 2009. The replay phone number for the U.S. and Canada is (800) 642-1687. The international replay number is (706) 645-9291 and the conference ID number is 81968318. Following the live webcast, an archived version will be available on Vertex's website until 5:00 p.m. EST on February 23, 2009.

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

(VRTX-GEN)

SOURCE: Vertex

**Vertex Contacts**

**Investors**

Michael Partridge, 617-444-6108

or

Lora Pike, 617-444-6755

or

**Media**

Jane Kramer, 617-444-6924

or

Zachry Barber, 617-444-6470