

October 27, 2008

# Vertex Pharmaceuticals Highlights Progress with Hepatitis C and Cystic Fibrosis Programs and Reports Third Quarter 2008 Results

- -- Phase 3 ADVANCE study of telaprevir in HCV completes enrollment --
- -- Registration program for VX-770 in cystic fibrosis being discussed with global authorities --
- -- As of September 30, 2008, Vertex had \$920 million of cash, cash equivalents and marketable securities--

CAMBRIDGE, Mass., Oct 27, 2008 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported recent clinical progress and consolidated financial results for the quarter ended September 30, 2008.

"2008 has been a year of significant progress across all aspects of our business," said Joshua Boger, Ph.D., President and Chief Executive Officer of Vertex Pharmaceuticals. "We have made substantial clinical progress in two very important areas--hepatitis C and cystic fibrosis. Telaprevir, our protease inhibitor targeting the treatment of hepatitis C, is well into a broad registration program for HCV genotype-1 treatment naAve and treatment failure patients, and recently we completed enrollment in the Phase 3 pivotal study known as ADVANCE for treatment naAve patients. A further registration study, REALIZE, is ongoing for HCV treatment failure patients. Additionally, we have made great advancements in the area of cystic fibrosis. On the basis of positive results for our lead CFTR potentiator VX-770, we are engaged in discussions with global authorities to design a registration program for VX-770 in cystic fibrosis."

"Vertex could therefore be positioned to make a major impact on the treatment of two serious diseases, with the potential for registration programs underway in parallel in 2009," continued Dr. Boger. "We're well-positioned financially to undertake these investments, and our organization is excited about the possibility of improving outcomes for both HCV and CF patients."

**HCV - Telaprevir Development Program** 

Phase 3 Development in Treatment-NaAve Population

- -- Vertex and Tibotec have completed enrollment in the global 3-arm pivotal Phase 3 ADVANCE trial that is focused on 24-week telaprevir-based response-guided regimens. In the ADVANCE study, telaprevir is being dosed for 8 or 12 weeks. Vertex expects to have sustained viral response (SVR) data from the study in the first half of 2010.
- -- Vertex has begun enrollment in a study of 500 treatment-naAve HCV patients that will include evaluation of 24-week and 48-week telaprevir-based regimens. These regimens include 12-week telaprevir dosing durations. The Company expects to complete enrollment in this study by the end of 2008 and expects to have SVR data from the study in the first half of 2010.

Phase 3 Development in Patients Who Failed to Achieve SVR with Prior Treatment

-- Patient dosing is underway in the Phase 3 REALIZE clinical study of telaprevir in patients who failed to achieve SVR with prior treatment of pegylated interferon (peg-IFN) and ribavirin (RBV). This study is focused on 48-week telaprevir-based regimens, which include dosing of telaprevir for 12 weeks. REALIZE is expected to complete enrollment of approximately 650 patients in the United States and Europe in the first guarter of 2009.

Interim Analysis of Telaprevir Twice-Daily Dosing Regimen

-- In July, Vertex reported results of an interim analysis of study C208, a four-arm Phase 2a clinical study of approximately 160 genotype 1 treatment-naAve HCV patients. The interim results showed that greater than 80% of patients (intent-to-treat analysis) had undetectable HCV RNA (<10 IU/mL) at weeks 4 and 12 in both the twice-daily and three times daily dosing arms of telaprevir, with pegylated interferon alfa-2a (PEGASYS) and RBV. The type and frequency of adverse events across the study arms were generally consistent with previous studies of telaprevir. No substantial differences in safety profile between twice daily and three times daily dosing regimens were observed. These data support continued clinical evaluation of twice-daily dosing of telaprevir.

Telaprevir Clinical Data

## AASLD

-- Six abstracts on telaprevir have been accepted for presentation at the 59th Annual Meeting of the American Association for the Study of Liver Diseases (AASLD),
October 31st -- November 4th in San Francisco, including presentations that include final results from the PROVE 2 study in treatment-naAve patients, interim data from the
PROVE 3 study and Study 107 in treatment failure patients, and interim results from study C208 evaluating twice-daily dosing of telaprevir.

Updates on the status of telaprevir clinical trials are available at www.clinicaltrials.gov.

Two Additional Novel HCV Protease Inhibitors in Clinical Development

-- Vertex is advancing a portfolio of HCV protease inhibitors with potentially differentiated profiles. Vertex has initiated a Phase 1b clinical trial of VX-500 in patients with HCV and expects data from this study in the first quarter of 2009. Vertex also has initiated Phase 1 clinical development of VX-813.

Cystic Fibrosis -- Targeting the CFTR Protein Responsible for Cystic Fibrosis

## VX-770

- -- Based on data presented to date, Vertex intends to work with global regulatory authorities on the design of a registration program for VX-770 which, if agreed upon, could begin in the first half of 2009.
- -- In April and on October 23rd at the 22nd Annual North American Cystic Fibrosis Conference in Orlando, researchers presented interim data from Part 1 (14-day) and Part 2 (28-day), respectively, of a Phase 2a trial of VX-770, an investigational Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) potentiator compound for the treatment of CF. In the Phase 2a study, researchers reported the following:
- -- VX-770 was well tolerated when dosed at 25mg, 75mg and 150mg twice daily for 14 days and at 150mg and 250mg when dosed twice daily for 28-days. -- No patients discontinued treatment and no serious adverse events were reported due to VX-770. -- With measures of lung function (FEV1) and measures of CFTR activity (nasal potential difference (NPD) and sweat chloride levels) a dose response was observed. -- Significant improvements in lung function, as measured by an increase from baseline in FEV1. -- Significant improvements in the function of the CFTR protein, as measured by changes from baseline in biomarkers of CFTR activity -- sweat chloride levels and NPD.

## VX-809

-- Vertex recently completed dosing in two Phase 1 trials of VX-809, an investigational oral CFTR corrector compound for the treatment of CF, in healthy volunteers. The first trial is a single and multiple dose study while the second is a single dose study examining the pharmacokinetics (PK) and safety of a solid dosage form. Based on these results, Vertex has initiated a single dose PK and safety trial of VX-809 in patients with CF. The Company plans to initiate a Phase 2a study in patients with CF in the first half of 2009.

Advancements in Pipeline of Other Novel Drug Candidates

## VX-509

-- Vertex is conducting 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.

### Aurora Kinase Inhibitors

-- Vertex's collaborator Merck is conducting a Phase 1 clinical trial of the Aurora kinase inhibitor MK-5108 (VX-689) in patients with advanced and/or refractory solid tumors. In addition, in the third quarter Merck selected additional Aurora kinase inhibitors for development and Vertex recognized a milestone of \$6.0 million.

#### Third Quarter Results

For the quarter ended September 30, 2008, the Company's GAAP net loss was \$130.0 million, or \$0.93 per share, compared to a GAAP net loss for the quarter ended September 30, 2007 of \$107.0 million, or \$0.82 per share. The increased net loss is attributable to decreased total revenues and an increase of total operating costs and expenses, as well as a reduction in net interest income.

The non-GAAP loss, before stock-based compensation expense and restructuring expense, for the quarter ended September 30, 2008 was \$114.7 million, or \$0.82 per share, compared to \$93.2 million, or \$0.72 per share, for the quarter ended September 30, 2007.

Total revenues for the quarter ended September 30, 2008 were \$31.6 million, compared to \$41.0 million for the third quarter of 2007. The reduction of revenues is attributable to reduced royalty revenues, due to the sale of the Company's HIV drug royalty stream in the second quarter, and a reduction in collaborative revenues principally due to the manner in which telaprevir development activities are shared between Vertex and Tibotec.

Research and development (R&D) expenses for the quarter ended September 30, 2008 were \$130.0 million, compared to \$128.9 million in R&D expenses for the third quarter of 2007. R&D expenses have remained relatively similar to prior year despite the advancement of development programs. This is principally due to the sharing of development activities for telaprevir between Vertex and Tibotec.

Sales, general and administrative (SG&A) expenses for the quarter ended September 30, 2008 were \$27.2 million, compared to \$21.4 million for the third quarter of 2007. This increase reflects building of infrastructure, including an increase in the number of employees and in our initial commercial investments, to support advancement of telaprevir.

Net interest income for the quarter ended September 30, 2008 was \$0.6 million, compared to net interest income of \$6.8 million for the third quarter of 2007. This decrease resulted from reduced portfolio yields, reflecting the broader economic environment, and interest expense incurred following the February 2008 issuance of the 2013 convertible senior subordinated debt.

At September 30, 2008, Vertex had \$920.1 million in cash, cash equivalents and marketable securities. This includes the net proceeds of approximately \$217.3 million received in the third quarter of 2008 from the Company's public offering of 8,625,000 shares of common stock. The Company also has outstanding \$287.5 million of convertible senior subordinated debt due in 2013, with a conversion price of \$23.14 per share.

## Full Year 2008 Financial Guidance

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

Vertex today is reiterating its guidance for 2008 GAAP net loss and non-GAAP loss, which was provided on July 31, 2008.

## 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 third quarter 2008 and 2007 loss and guidance for its projected 2008 loss, excluding restructuring expense 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.

## Special Note Regarding Forward-looking Statements

This press release contains forward-looking statements, including statements regarding Vertex's expectation that (i) it is positioned to make a major impact on the treatment of HCV and CF, with the potential to conduct registration programs in parallel in 2009, (ii) it is well-positioned financially to support investment in its HCV and CF opportunities, (iii) it will have SVR data from the ADVANCE study in the first half of 2010, (iv) it will enroll approximately 500 patients in a Phase 3 clinical trial to evaluate 24-week and 48-week telaprevir-based regimens by the end of 2008 and SVR data from this study will be available in the first half of 2010, (v) the REALIZE study will complete enrollment in the first quarter of 2009, (vi) interim data from Phase 2 clinical study evaluating telaprevir dosing regimens support continued evaluation of twice-daily dosing of telaprevir, (vii) data from Phase 1b clinical trial of VX-500 in HCV patients will be available in the first quarter of 2009, (viii) it will reach agreement with regulatory authorities on a registration program for VX-770 and initiate the study in the first half of 2009, (ix) it will initiate a Phase 2a clinical trial of VX-809 in patients with CF in the first half of 2009, (x) VX-509 will be investigated for the treatment of multiple immune-mediated inflammatory diseases, (xi) the 2008 guidance (including its estimates of stock-based compensation expense) will be in the ranges referenced in the Company's previous guidance. 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 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 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 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, that unexpected costs associated with one or more of the Company's programs will necessitate 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 cystic fibrosis , 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.

Vertex Pharmaceuticals Incorporated

outstanding

2008 Third Quarter and Nine Month Results Consolidated Statements of Operations Data (In thousands, except per share amounts) (Unaudited) Three Months Ended Nine Months Ended September 30, September 30, 2008 2007 2008 2007 Revenues: \$28,355 Royalty revenues (Note 5) \$7,763 \$12,522 \$33,285 Collaborative and other R&D revenues 23,846 28,492 114,338 114,735 Total revenues 31,609 41,014 142,693 148,020 Costs and expenses: Royalty expenses (Note 5) 4,194 3.562 11,471 10.232 128,949 Research and development expenses (R&D) 371.682 397.714 129.968 77,702 Sales, general & administrative expenses (SG&A) 27,190 21,416 61,275 Restructuring expense 885 882 2,683 6,843 Total costs and expenses 162,237 154,809 463,538 476,064 (113,795) Loss from operations (130,628) (320,845) (328,044) Net interest income (expense) 584 6.762 3.326 22.516 \$(130,044) \$(107,033) \$(317,519) Net loss \$(305,528) \$(0.82) \$(2.30) Basic and diluted net loss per common share \$(0.93) \$(2.38) Basic and diluted weighted-average number of common shares 137.788 128.378 140.109 130,006

Non-GAAP Loss and Loss per Common Share Reconciliation	Three Months Ended		Nine Months Ended	
	September 30,		September 30,	
	2008	2007	2008	2007
GAAP Net Loss	\$(130,044)	\$(107,033)	\$(317,519)	\$(305,528)
Pro Forma Adjustments:				
Stock-based compensation expense included in R&D (Note 1):	\$12,046	\$10,624	\$36,734	\$38,564
Stock-based compensation expense included in SG&A (Note 1):	2,439	2,348	7,416	8,185
Total stock-based compensation expense	14,485	12,972	44,150	46,749
Restructuring expense (Note 2)	885	882	2,683	6,843
Non-GAAP Loss	\$(114,674)	\$(93,179)	\$(270,686)	\$(251,936)
Basic and diluted non-GAAP loss per common share	\$(0.82)	\$(0.72)	\$(1.96)	\$(1.96)

Note 1: For the three and nine months ended September 30, 2008, the Company incurred \$14.5 million and \$44.2 million, respectively, in stock-based compensation expense of which \$12.0 million and \$36.7 million is included in research and development expenses and \$2.4 million and \$7.4 million, respectively, is included in sales, general and administrative expenses. For the three and nine months ended September 30, 2007, the Company incurred \$13.0 million and \$46.7 million, respectively, in stock-based compensation expense of which \$10.6 million and \$38.6 million, respectively, is included in research and development expenses and \$2.3 million and \$8.2 million, respectively, is included in sales, general and administrative expenses.

Note 2: For the three and nine months ended September 30, 2008, the Company incurred restructuring expenses of \$0.9 million and \$2.7 million, respectively. The expense for both periods is primarily a result of the imputed interest cost related to the restructuring liability. For the three and nine months ended September 30, 2007, the Company incurred restructuring expenses of \$0.9 million and \$6.8 million, respectively. The three month expense is primarily a result of the imputed interest cost related to the restructuring liability. The nine month expense is the result of incremental lease obligations related to the revision of certain key estimates and assumptions about building operating costs as well as the imputed interest cost related to the restructuring liability. The expense and the related liability have been estimated in accordance with SFAS 146 "Accounting for Costs Associated with Exit or Disposal Activities" and are reviewed quarterly for changes in circumstances.

Note 3: In September2008, the Company completed a public offering of 8,625,000 shares of common stock, including the underwriters' over-allotment of 1,125,000 shares, at a price of \$25.50 per share. This transaction resulted in net proceeds of \$217.3 million to the Company. The net proceeds include an underwriting discount of \$2.2 million and other expenses of \$0.4 million related to the equity offering that were recorded as an offset to additional paid-in-capital.

In February2008, the Company completed a public offering of 6,900,000 shares of common stock, including the underwriters' over-allotment of 900,000 shares, at a price of \$17.14 per share. This transaction resulted in net proceeds of \$112.7 million to the Company. The net proceeds include an underwriting discount of \$5.3 million and other expenses of \$0.2 million related to the equity offering that were recorded as an offset to additional paid-in-capital.

Note 4: In February2008, the Company completed an offering of \$287.5 million aggregate principal amount of 4.75% convertible senior subordinated notes due February2013 (the "2013 Notes"), including \$37.5 million aggregate principal amount of notes purchased by the underwriters pursuant to their over-allotment option. The 2013 Notes are convertible, at the option of the holder, into common stock at a price equal to approximately \$23.14 per share, subject to adjustment under certain circumstances. The 2013 Notes bear interest at the rate of 4.75% per year, and the Company is required to make semi-annual interest payments on the outstanding principal balance of the notes on February15 and August15 of each year. This transaction resulted in net proceeds of \$278.6 million to the Company. The net proceeds include an underwriting discount of \$8.6 million and other expenses of \$0.3 million related to the convertible debt offering that were recorded as deferred issuance costs and are in included in other assets on the Company's condensed consolidated balance sheets.

Note 5: On May30, 2008, the Company entered into a purchase agreement with Fosamprenavir Royalty, L.P. pursuant to which the Company sold, and Fosamprenavir Royalty, L.P. purchased, the Company's right to receive future royalty payments, net of sub-royalty payments due to a third party, arising from sales of Lexiva/Telzirand Ageneraseunder the Company's 1993 license agreement with GlaxoSmithKline plc for periods commencing April1, 2008, in return for a one-time cash payment of \$160.0 million. In accordance with the purchase agreement, GlaxoSmithKline plc will (i)make all future royalty payments due to Vertex under the license agreement directly to Fosamprenavir Royalty, L.P. and (ii)make royalty payments due to a third party in connection with the HIV product sales under the license agreement, which payments had been made directly by the Company prior to the royalty sale transaction.

In the second quarter of 2008, in accordance with Emerging Issues Task Force Issue No.88-18, "Sales of Future Revenues," the Company began recognizing deferred

revenues relating to the \$160.0 million one-time cash payment from Fosamprenavir Royalty L.P. under the "units-of-revenue" method. In each period, the Company will recognize a portion of the deferred revenues together with additional royalty revenues equal to royalties payable to the third party on net sales of Agenerase and Lexiva/Telzir. The Company will recognize royalty expense in each period based on (i)deferred transaction expenses (included in other assets on the Company's condensed consolidated balance sheets) in the same manner and over the same period in which the related deferred revenues are recognized as royalty revenues plus (ii) the royalties paid the third party on net sales of Agenerase and Lexiva/Telzir for the period.

Condensed Consolidated Balance Sheets Data (In thousands) (Unaudited)

(onaudiced)		
	September 30,	December 31,
	2008	2007
Assets		
Cash, cash equivalents and marketable securities	\$920,067	\$467,796
Other current assets	32,951	35,980
Property and equipment, net	69,713	66,509
Restricted cash	30,258	30,258
Other non-current assets (Notes 4 & 5)	14,565	934
Total assets	\$1,067,554	\$601,477
Liabilities and Stockholders' Equity		
Other current liabilities	\$137,551	\$148,148
Accrued restructuring expense	34,382	35,292
Deferred revenues (Note 5)	258,228	126,745
Collaborator development loan (due May 2008)		19,997
Convertible notes (due 2013) (Note 4)	287,500	
Stockholders' equity	349,893	271,295
Total liabilities and stockholders' equity	\$1,067,554	\$601,477
Common shares outstanding (Note 3)	150,411	132,876

Conference Call and Webcast: Third Quarter Financial Results:

Vertex Pharmaceuticals will host a conference call and webcast today, Monday, October 27, 2008 at 5:00 p.m. EDT to review financial results and recent developments. This call and webcast will be broadcast via the Internet at www.vrtx.com. 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 (706) 634-2224 (International) and the conference ID number is 69583241. Vertex is also providing a podcast MP3 file available for download on the Vertex website at www.vrtx.com.

The call will be available for replay via telephone commencing October 27, 2008 at 8:00 p.m. EDT running through 5:00 p.m. EST on November 3, 2008. 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 69583241. Following the live webcast, an archived version will be available on Vertex's website until 5:00 p.m. EST on November 10, 2008.

Vertex's press releases are available at www.vrtx.com.

(VRTX-GEN)

SOURCE: Vertex Pharmaceuticals Incorporated

Vertex Pharmaceuticals Incorporated Michael Partridge, 617-444-6108 Senior Director, Strategic Communications or Lora Pike, 617-444-6755 Manager, Investor Relations or Zachry Barber, 617-444-6470 Manager, Media Relations

Copyright Business Wire 2008

News Provided by COMTEX