



April 21, 2010

## **Vertex Pharmaceuticals Reports First Quarter 2010 Financial Results and Highlights Recent Business and Clinical Progress**

***-HCV: Rolling submission of New Drug Application for telaprevir expected to begin in summer of 2010; first Phase 3 SVR data expected in second quarter 2010 from ADVANCE trial-***

***-CF: STRIVE, ENVISION and DISCOVER trials dosing patients as part of Phase 3 registration program for VX-770; data expected in first half of 2011-***

***-Pipeline: Multiple ongoing or planned proof-of-concept clinical trials with compounds for rheumatoid arthritis and epilepsy and with novel combination regimens for HCV and CF-***

***-Financial: Vertex ends first quarter with cash position of approximately \$1.1 billion-***

CAMBRIDGE, Mass., Apr 21, 2010 (BUSINESS WIRE) -- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today provided an update on recent progress in its development programs in hepatitis C virus (HCV) infection, cystic fibrosis (CF) and other diseases and reported consolidated financial results for the quarter ended March 31, 2010.

"Vertex continues to make important advancements across the business as we prepare to submit a New Drug Application for telaprevir in the second half of 2010," said Matthew Emmens, Chairman, President and Chief Executive Officer of Vertex Pharmaceuticals.

"Our Phase 3 registration program for telaprevir in hepatitis C virus infection is nearing completion, and this quarter, we expect to receive the first Phase 3 SVR registration data from the ADVANCE trial in treatment-naïve patients, followed by additional Phase 3 SVR registration data from the REALIZE trial in treatment-failure patients in the third quarter," continued Mr. Emmens.

"Importantly, we now plan to submit our NDA for telaprevir on a rolling basis beginning this summer and expect to complete the NDA submission in the second half of 2010 with clinical data from Phase 3 trials in both treatment-naïve and treatment-failure patients. Our commercial and launch preparation activities remain a key focus for 2010, and we are on track to achieve important milestones in the NDA submission process.

"In cystic fibrosis, the STRIVE and DISCOVER trials are fully enrolled as part of the Phase 3 registration program for VX-770. We expect to receive data from this program in early 2011, which could help support a planned NDA submission for VX-770 in the second half of 2011.

"Beyond our Phase 3 programs, we are making significant progress toward our objective to explore novel combination regimens that could potentially enhance the future treatment of HCV and CF. In the second quarter, we expect to complete enrollment in a proof-of-concept trial that will combine telaprevir with our lead HCV polymerase inhibitor, VX-222, as part of our long-term commitment to improving HCV care. Additionally, in CF we plan to conduct further dose-ranging activities for VX-809 and to initiate the first clinical trial to combine VX-770 and VX-809 for the treatment of CF.

"Financially, we continue to manage our capital structure as we approach the planned NDA submission for telaprevir later this year. We ended the first quarter with a cash position of approximately \$1.1 billion and now have no outstanding convertible debt obligations after the recently completed conversion of our outstanding convertible debt.

"This is an exciting time for Vertex. We have multiple late-stage and mid-stage development programs advancing in serious diseases, and we continue to build and strengthen our business as we prepare for the potential launch of telaprevir," Mr. Emmens concluded.

### **Recent Clinical Development Progress**

#### ***Hepatitis C Virus Infection:***

*Telaprevir: SVR and Safety Data from Phase 3 ADVANCE Trial Expected in Second Quarter 2010*

- In the second quarter of 2010, Vertex expects to obtain SVR and safety data from the Phase 3 ADVANCE clinical trial of

telaprevir in treatment-naïve HCV patients.

- Additional Phase 3 SVR and safety data are expected in the third quarter of 2010 from the ILLUMINATE clinical trial in treatment-naïve HCV patients and from the REALIZE clinical trial in treatment-failure HCV patients.
- Vertex today announced that it has reached agreement with the U.S. Food and Drug Administration to submit its New Drug Application (NDA) for telaprevir on a rolling basis. Vertex expects to submit the Non-clinical and Chemistry, Manufacturing and Controls (CMC) sections of the NDA in the summer of 2010. Vertex plans to complete the submission of the NDA for telaprevir in the second half of 2010 with clinical data from Phase 3 trials in both treatment-naïve and treatment-failure HCV patients.

#### *EASL Data Presentations: VX-222 and Study 107*

- In conjunction with oral presentations conducted last week at the 45th Annual Meeting of the European Association for the Study of the Liver (EASL) in Vienna, Vertex announced results from a Phase 1b clinical trial of the investigational oral HCV polymerase inhibitor, VX-222, as well as results from Study 107, an open-label Phase 2 rollover study of patients who did not achieve SVR after receiving pegylated interferon and ribavirin in the control arms of the Phase 2 PROVE trials of telaprevir. Additional details, including safety and efficacy data, were provided in press releases issued on April 15, 2010.

#### *Telaprevir/VX-222 Combination Trial*

- Vertex is initiating the first clinical trial to evaluate telaprevir dosed in combination with Vertex's lead HCV polymerase inhibitor, VX-222. This Phase 2 proof-of-concept trial will evaluate SVR rates using multiple 12-week response-guided regimens of telaprevir/VX-222-based combination therapy, including two-drug regimens that contain only telaprevir and VX-222. Vertex expects to complete patient enrollment for this trial in the second quarter of 2010 and to obtain interim clinical data, including safety and viral kinetic data, from this trial in the second half of 2010.

#### **Cystic Fibrosis:**

##### *VX-770: Phase 3 Registration Program Ongoing*

- Three trials of the novel CFTR potentiator VX-770 are ongoing as part of a global Phase 3 registration program focused on patients with the G551D mutation. The Phase 3 STRIVE trial in patients aged 12 years and older with the G551D mutation and the Phase 2 DISCOVER trial in patients aged 12 and older with the F508del mutation are fully enrolled. Enrollment is ongoing in the Phase 3 ENVISION trial in patients aged six to 11 years.
- Data from this Phase 3 registration program of VX-770 are expected in the first half of 2011.

##### *VX-809: Dose-ranging Studies to Support Combination Trial with VX-770*

- In February, Vertex announced data from a Phase 2 trial of the CFTR corrector VX-809. Based on these data, Vertex plans to conduct additional dose-ranging activities for VX-809 and to initiate a proof-of-concept clinical trial to evaluate a combination regimen of VX-770 and VX-809.

#### **Pipeline:**

##### *VX-509: Rheumatoid Arthritis*

- Vertex is now dosing patients in a recently initiated Phase 2 proof-of-concept clinical trial of the novel Janus kinase 3 (JAK3) inhibitor VX-509 in rheumatoid arthritis (RA).

##### *VX-765: Epilepsy*

- Vertex is now dosing patients in a recently initiated Phase 2 proof-of-concept clinical trial of the novel caspase-1 inhibitor VX-765 in epilepsy.

Interim data from both of these trials are expected as early as the second half of 2010.

#### **First Quarter Results**

For the quarter ended March 31, 2010, the Company's GAAP net loss was \$165.3 million, or \$0.83 per share, including certain charges totaling \$25.2 million, compared to a GAAP net loss for the quarter ended March 31, 2009 of \$162.7 million, or \$1.04

per share, including certain charges totaling \$34.0 million.

The non-GAAP loss, before certain charges, for the quarter ended March 31, 2010 was \$140.1 million, or \$0.70 per share, compared to \$128.7 million, or \$0.83 per share, for the quarter ended March 31, 2009. The increase in the Company's 2010 non-GAAP loss was principally attributable to increased costs to support advancement of telaprevir toward potential launch.

Total revenues for the quarter ended March 31, 2010 were \$22.4 million, compared to \$24.0 million for the first quarter of 2009.

Research and development (R&D) expenses for the quarter ended March 31, 2010 were \$143.0 million, compared to \$143.6 million for the first quarter of 2009. First quarter 2010 R&D expenses reflect decreased costs related to the conduct of Phase 3 clinical trials for telaprevir, which were largely offset by increased costs related to commercial supply investment for telaprevir and additional trials being conducted in CF.

Sales, general and administrative (SG&A) expenses for the quarter ended March 31, 2010 were \$35.6 million, compared to \$28.5 million for the first quarter of 2009. This increase primarily reflects building of capabilities, including an increase in the number of employees and our commercial investments, to support advancement of telaprevir toward potential launch.

At March 31, 2010, Vertex had \$1.1 billion in cash, cash equivalents and marketable securities. Vertex has no remaining 2013 convertible notes outstanding.

### **Full Year 2010 Financial Guidance**

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

The Company is today reiterating its guidance for 2010 GAAP net loss of approximately \$700 million and for 2010 non-GAAP net loss of approximately \$600 million, as provided 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 its first quarter 2010 and 2009 loss, and guidance for its projected 2010 loss, excluding stock-based compensation and executive transition expenses, restructuring expense, acquisition-related expenses, and expenses related to certain September 2009 financial transactions. 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, are important in comparing current results with prior period results and provide additional information regarding its financial position. 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 the other 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, cystic fibrosis, inflammation, autoimmune diseases, epilepsy, cancer, and pain.

### **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 plan to submit the Company's NDA for telaprevir on a rolling basis beginning with the submission of the CMC and Non-clinical sections in the summer of 2010 and the expectation that the Company will complete the NDA submission in the second half of 2010 with clinical data from Phase 3 trials in both treatment-naïve and treatment-failure patients, (ii) the expectation that Phase 3 SVR and safety data will be received in the second quarter of 2010 from ADVANCE and in the third quarter of 2010 from REALIZE and ILLUMINATE, (iii) the expectation that data from the Phase 3 registration program for VX-770 will be received in early 2011, which could help support a planned NDA submission for VX-770 in the second half of 2011, (iv) the expectation that enrollment in a proof-of-concept trial that will combine telaprevir with the Company's lead HCV polymerase inhibitor will be completed in the second quarter of 2010 and that interim clinical data, including safety and viral kinetic data, from this trial will be obtained in the second half of 2010, (v) the potential for novel combination regimens to enhance future treatment of HCV and CF, (vi) the plan to conduct additional dose-ranging activities for VX-809 and a clinical trial to combine VX-770 and VX-809, (vii) the expectation that interim data from the clinical trials of VX-509 and VX-765 will be available as early as the second half of 2010 and (viii) the Company's guidance that its 2010 GAAP and

non-GAAP net losses will be as provided on February 4, 2010. 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 2010 GAAP and non-GAAP net loss 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 Pharmaceuticals Incorporated**  
**2010 First Quarter Results**  
**Consolidated Statements of Operations Data**  
(in thousands, except per share amounts)  
(unaudited)

	Three Months Ended March 31,	
	2010	2009
Revenues:		
Royalty revenues	\$ 6,407	\$ 6,140
Collaborative revenues	16,022	17,839
Total revenues	22,429	23,979
Costs and expenses:		
Royalty expenses	3,367	3,576
Research and development expenses (R&D)	143,012	143,581
Sales, general & administrative expenses (SG&A)	35,552	28,520
Restructuring expense	780	2,402
Acquisition-related expenses (Note 2)	---	7,793
Total costs and expenses	182,711	185,872
Loss from operations	(160,282)	(161,893)
Net interest expense (Note 1)	(3,500)	(779)
Loss on derivative instruments (Note 1)	(1,489)	---
Net loss	\$ (165,271)	\$ (162,672)
Basic and diluted net loss per common share	\$ (0.83)	\$ (1.04)
Basic and diluted weighted-average number of common shares outstanding	198,935	155,860

	Three Months Ended March 31,	
	2010	2009
GAAP Net Loss	\$(165,271)	\$(162,672)
Pro Forma Adjustments:		
Stock-based compensation and executive transition expenses included in R&D	\$ 14,320	\$ 18,573
Stock-based compensation and executive transition expenses included in SG&A	5,013	5,205
Total stock-based compensation and executive transition expenses	\$ 19,333	\$ 23,778
Expenses related to September 2009 financial transactions (Note 1)	5,072	---
Restructuring expense	780	2,402
Acquisition-related expenses (Note 2)	---	7,793
Non-GAAP Loss	\$(140,086)	\$(128,699)

Basic and diluted non-GAAP loss per common share \$ (0.70) \$ (0.83)

**Note 1:** The loss on derivative instruments and a portion of the net interest expense reflected in the Consolidated Statements of Operations Data, and the liabilities related to milestone transactions reflected in the Condensed Consolidated Balance Sheets Data, relate to two financial transactions that the Company entered into in September 2009 relating to future milestone payments under the Company's collaboration agreement with Janssen Pharmaceutica, N.V. In the first quarter of 2010, the Company recorded interest expense of \$3.6 million related to its secured notes (due 2012) and an additional aggregate expense of \$1.5 million related to the changes in estimated fair values of the rights to the \$95.0 million in potential future milestone payments and the derivative embedded in the secured notes (due 2012).

**Note 2:** The acquisition-related expenses reflected in the Consolidated Statements of Operations Data, and the intangible assets, the goodwill and the deferred tax liability reflected in the Condensed Consolidated Balance Sheets Data, relate to the Company's acquisition of ViroChem Pharma Inc. in 2009.

**Note 3:** In the first quarter of 2010, the holders of the remaining \$32.1 million in aggregate principal amount of 4.75% convertible senior subordinated notes due February 2013 (the "2013 Notes") converted their 2013 Notes into 1.4 million shares of the Company's common stock in full satisfaction of the 2013 Notes.

### Condensed Consolidated Balance Sheets Data

(in thousands)  
(unaudited)

	March 31, 2010	December 31, 2009
<b>Assets</b>		
Cash, cash equivalents and marketable securities	\$1,092,683	\$ 1,284,913
Other current assets	34,085	22,113
Property and equipment, net	58,109	62,279
Restricted cash	30,313	30,313
Intangible assets (Note 2)	518,700	518,700
Goodwill (Note 2)	26,102	26,102
Other non-current assets	10,310	11,068
Total assets	<u>\$1,770,302</u>	<u>\$ 1,955,488</u>
<b>Liabilities and Stockholders' Equity</b>		
Other liabilities	\$ 136,324	\$ 172,273
Accrued restructuring expense	33,333	34,017
Deferred tax liability (Note 2)	160,278	160,278
Deferred revenues	285,063	300,531
Convertible notes (due 2013) (Note 3)	--	32,071
Liabilities related to milestone transactions (Note 1)	164,692	159,972
Stockholders' equity (Note 3)	990,612	1,096,346
Total liabilities and stockholders' equity	<u>\$1,770,302</u>	<u>\$ 1,955,488</u>
Common shares outstanding (Note 3)	<u>202,123</u>	<u>199,955</u>

### Conference Call and Webcast: First Quarter Financial Results:

Vertex Pharmaceuticals will host a conference call and webcast today, Wednesday, April 21, 2010 at 5:00 p.m. ET to review financial results and recent developments. This call and webcast will be broadcast via the Internet at [www.vrtx.com/finances](http://www.vrtx.com/finances). 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. To listen to the call on the telephone, dial (888) 245-0960 (U.S. and Canada) (913) 312-1489 (International) using conference ID number 5059974. 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 April 21, 2010 at 8:00 p.m. EDT running through 5:00 p.m. EDT on April 28, 2010. The replay phone number for the US and Canada is (888) 203-1112, the international replay number is (719) 457-0820 and the conference ID number is 5059974. Following the live webcast, an archived version will be available on Vertex's website until 5:00 p.m. ET on May 5, 2010.

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

(VRTX-GEN)

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Vertex Pharmaceuticals Incorporated

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