



July 29, 2013

## **Vertex Reports Second Quarter 2013 Financial Results and Reviews Recent Progress and Upcoming Milestones in Clinical Development Programs**

*-Second quarter 2013 total revenues of \$311 million, including net product revenues of \$99 million for KALYDECO in cystic fibrosis and \$156 million for INCIVEK in hepatitis C; cash position of approximately \$1.43 billion on June 30, 2013-*

*-Data from Phase 3 study of ivacaftor monotherapy support submission of supplemental New Drug Application (sNDA) planned for second half of 2013 for gating mutations-*

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended June 30, 2013. Vertex reported total second quarter 2013 revenues of \$311 million, including net product revenues of \$99 million from KALYDECO™ (ivacaftor) and \$156 million from INCIVEK® (telaprevir). The GAAP net loss attributable to Vertex was \$(57.2) million, or \$(0.26) per share, for the second quarter of 2013, including certain charges of \$51.0 million, comprised primarily of stock-based compensation expense. Non-GAAP net loss attributable to Vertex for the second quarter of 2013 was \$(6.2) million, or \$(0.03) per diluted share. The company reported \$1.43 billion in cash, cash equivalents and marketable securities as of June 30, 2013 and has no outstanding convertible debt following the completion of a call of its outstanding Convertible Senior Subordinated Notes due 2015.

"Entering the second half of 2013, we continue to progress key programs in cystic fibrosis, hepatitis C and rheumatoid arthritis and have strengthened our financial position to support continued investment in our business," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Our progress in the first half of the year was marked by the initiation of a Phase 3 program in CF for people with the most common form of the disease, continued progress in bringing KALYDECO to people with CF around the world and the initiation of all-oral studies for VX-135 in hepatitis C."

### **Development Program Updates**

#### **Cystic Fibrosis**

Vertex's strategy in cystic fibrosis (CF) is to provide benefit to as many CF patients as possible, and to maximize the benefit for these patients, with our approved and investigational medicines.

#### *Data from Phase 3 Label-Expansion Study of Ivacaftor Monotherapy in Gating Mutations Show Statistically Significant Improvements in Lung Function*

- In a separate press release issued today, Vertex announced data from a Phase 3 label-expansion study in people with CF who have at least one non-G551D cystic fibrosis transmembrane conductance regulator (*CFTR*) gating mutation that showed statistically significant improvements in lung function (percent predicted forced expiratory volume in one second; FEV<sub>1</sub>). Additional details on these data were provided in the separate press release.
- Based on these data, Vertex plans to submit a supplemental New Drug Application (sNDA) in the United States and a Marketing Authorization Application (MAA) variation in Europe in the second half of 2013 for the use of ivacaftor monotherapy in people with CF ages 6 and older who have at least one non-G551D *CFTR* gating mutation. Approximately 400 people with CF ages six and older have a non-G551D gating mutation worldwide.

#### *Continued Progress in Additional Label-Expansion Studies for Ivacaftor Monotherapy*

- Two additional Phase 3 label-expansion studies are ongoing for ivacaftor monotherapy, including a study in people with CF ages 6 and older who have at least one copy of the R117H mutation and a study in children with CF ages 2 to 5 who have a gating mutation, including the G551D mutation. Data from the study in the R117H mutation are expected in the second half of 2013, and, pending study results, Vertex plans to submit an sNDA in early 2014 for the use of ivacaftor monotherapy in people with CF ages 6 and older who have the R117H mutation. The pharmacokinetic portion of the study in children ages 2 to 5 is complete and a dose has been selected for the 24-week dosing period, which is now underway. Data from this study are expected in mid-2014.

## *Study of People with CF Who Have Evidence of Residual CFTR Function*

- Enrollment is ongoing in a Phase 2 proof-of-concept study evaluating ivacaftor in people with CF who have clinical evidence of residual CFTR function. Data from this study are expected in the first half of 2014.

Vertex believes that ivacaftor monotherapy may provide clinical benefit in 10 to 15 percent of the estimated 70,000 CF patients worldwide, pending results from our clinical studies and regulatory approvals.

## *Enrollment Ongoing in Phase 3 Registration Program for VX-809 in Combination with Ivacaftor*

- Two 24-week Phase 3 studies of VX-809 in combination with ivacaftor are ongoing in people ages 12 and older with two copies of the most common mutation in the *CFTR* gene, known as F508del. The company expects to complete enrollment in these studies in the second half of 2013. Vertex plans to submit a New Drug Application in the United States for this combination treatment in 2014, pending study results. Worldwide, nearly half of people with CF have two copies of the F508del mutation.
- The pivotal program for VX-809 in combination with ivacaftor also includes an evaluation of this combination in people with one copy (heterozygous) of the F508del mutation and a pharmacokinetic and safety evaluation of this combination in children ages 6 to 11 with two copies of the F508del mutation. Enrollment in these additional studies is expected to begin in the second half of 2013.

## *Advancing Multiple First- and Second-Generation Correctors*

- **VX-661:** At the European Cystic Fibrosis Society (ECFS) conference in June, Vertex presented data from a Phase 2 study of VX-661 in combination with ivacaftor. Additional details on these data are available in a press release issued on June 5, 2013. Vertex is preparing to begin Phase 2 evaluation of a 4-week regimen of VX-661 in combination with ivacaftor in people with one copy of the F508del mutation and one copy of the G551D mutation. This is the first proof-of-concept study of a combination of a corrector and ivacaftor in people with the G551D mutation. This exploratory evaluation is based on *in vitro* data presented at ECFS by Vertex researchers that showed increased chloride transport in human bronchial epithelial cells with one copy of the F508del mutation and one copy of the G551D mutation after treatment with a corrector and ivacaftor, as compared to the use of ivacaftor alone. Vertex's strategy is to evaluate multiple first-generation correctors, including VX-661 and VX-983, in combination with ivacaftor to identify regimens that may provide benefit to people with the F508del mutation.
- **Second-generation Correctors:** Vertex has an active research program focused on second-generation correctors that could be used as part of a future dual-corrector regimen in combination with ivacaftor in people with one or two copies of the F508del mutation. Vertex's goal is to advance a second-generation corrector into clinical development by the end of 2014. The proposed use of a dual-corrector combination regimen is supported by *in vitro* data presented at ECFS that showed a combination of two correctors and ivacaftor increased chloride transport in human bronchial epithelial cells with one or two copies of the F508del mutation, as compared to the use of a single corrector in combination with ivacaftor.

## **Hepatitis C**

Vertex's strategy in hepatitis C is to develop new all-oral treatment regimens of 12 weeks or less in duration with a goal of providing a high viral cure rate and improved tolerability over currently available treatment options. Multiple Phase 2 studies of VX-135 as part of all-oral treatment regimens are ongoing, including studies of VX-135 in combination with ribavirin in the United States and Europe and a study of VX-135 in combination with daclatasvir, an NS5A replication complex inhibitor, in New Zealand. Dosing of 100 mg and 200 mg of VX-135 is complete in the European study and ongoing in the New Zealand study. Dosing of 100 mg of VX-135 is ongoing in the U.S. study. Under a previously announced partial clinical hold, Vertex will not evaluate a 200 mg dose of VX-135 in the United States without authorization from the FDA. Vertex provided a comprehensive update on the status of these studies in a press release issued July 25, 2013.

## **Autoimmune Diseases**

Vertex's strategy in autoimmune diseases is to maximize the value of VX-509 across multiple autoimmune diseases globally. The company will evaluate collaborative opportunities that provide funding and capabilities to broaden and accelerate global development of VX-509.

## *Ongoing Phase 2b Study of VX-509 in Rheumatoid Arthritis*

- Enrollment is complete in a 24-week Phase 2b study of VX-509, a selective JAK3 inhibitor, in people with moderate to severe rheumatoid arthritis (RA) receiving methotrexate. The primary endpoints of this study will be measured after 12 weeks of treatment, and data from this study are expected in the second half of 2013.

## Second Quarter 2013 Financial Results

**Total Revenues:** Total revenues for the second quarter of 2013 were \$310.8 million, compared with \$418.3 million in total revenues for the second quarter of 2012. The components of total revenues for the second quarter and first six months of 2013 and 2012 were:

	<b>Three Months Ended June 30,</b>		<b>Six Months Ended June 30,</b>	
	<b>2013</b>	<b>2012</b>	<b>2013</b>	<b>2012</b>
<b>Product Revenues</b>	(in millions)		(in millions)	
INCIVEK revenues, net	\$ 155.8	\$ 327.7	\$ 361.4	\$ 684.6
KALYDECO revenues, net	99.0	45.5	160.8	63.9
Total product revenues, net	254.8	373.3	522.2	748.7
<b>Royalty revenues</b>				
Royalty revenues from INCIVO	44.1	28.0	83.1	60.9
Other royalty revenues	5.0	5.5	9.5	11.5
Total royalty revenues	49.1	33.5	92.7	72.5
<b>Collaborative revenues</b>	6.8	11.6	24.3	36.0
<b>Total revenues</b>	<b>\$ 310.8</b>	<b>\$ 418.3</b>	<b>\$ 639.1</b>	<b>\$ 857.0</b>

A table of the components of total revenues for the second quarter of 2013, first quarter of 2013 and second, third and fourth quarters of 2012 is provided following the Condensed Consolidated Statements of Operations Data.

### • **Net Product Revenues from INCIVEK**

Vertex's second quarter 2013 net product revenues from INCIVEK were \$155.8 million, compared to \$327.7 million for the second quarter of 2012. The reduced revenues from INCIVEK were due to fewer HCV patients initiating treatment in the second quarter of 2013 compared to the second quarter of 2012.

### • **Net Product Revenues from KALYDECO**

Vertex's second quarter 2013 net product revenues from KALYDECO were \$99.0 million, compared to \$45.5 million for the second quarter of 2012. The increased revenues, compared to the second quarter of 2012, resulted primarily from the rapid uptake of KALYDECO in eligible patients in Europe following the conclusion of reimbursement discussions. Nearly all eligible patients with the G551D mutation in the United States and Europe have started treatment with KALYDECO.

### • **Royalty Revenues from INCIVO®**

Vertex recognized \$44.1 million in INCIVO royalty revenues for the second quarter of 2013 from our collaborator Janssen, compared to \$28.0 million in INCIVO royalty revenues for the second quarter of 2012. The increase in INCIVO royalties was due to expanded availability of INCIVO in international markets, most notably in Latin America.

**Cost of Product Revenues:** Cost of product revenues was \$24.7 million for the second quarter of 2013, compared to cost of product revenues of \$104.5 million for the second quarter of 2012. The cost of product revenues for the second quarter 2012 included a \$78.0 million reserve against the potential for excess INCIVEK inventory.

**Research and Development (R&D) Expenses:** R&D expenses were \$222.5 million for the second quarter of 2013, including \$31.3 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex, compared to \$196.5 million for the second quarter of 2012, including \$23.3 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex. The increase in Vertex's R&D investment is principally due to progression and expansion of clinical development programs in cystic fibrosis and hepatitis C, including initiation of a pivotal program for a combination of VX-809 and ivacaftor.

**Sales, General and Administrative (SG&A) Expenses:** SG&A expenses were \$106.5 million for the second quarter of 2013, including \$17.0 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex, compared to \$117.5 million for the second quarter of 2012, including \$12.5 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex. This decrease in SG&A expenses resulted primarily from reduced HCV marketing and commercial expenses.

**GAAP Net Loss Attributable to Vertex:** Vertex's second quarter 2013 GAAP net loss was \$(57.2) million, or \$(0.26) per share, including certain charges of \$51.0 million, comprised primarily of stock-based compensation expense. Vertex's GAAP net loss for the second quarter of 2012 was \$(64.9) million, or \$(0.31) per diluted share, including \$164.7 million in certain charges.

**Non-GAAP Net Income (Loss) Attributable to Vertex:** Vertex's second quarter 2013 non-GAAP net loss was \$(6.2) million, or \$(0.03) per diluted share. Vertex's non-GAAP net income for the second quarter of 2012 was \$99.8 million, or \$0.46 per diluted share. The decrease in the company's second quarter 2013 non-GAAP net income, compared to the second quarter of 2012, is primarily attributable to a decrease in total revenues, specifically decreased INCIVEK revenues due to fewer HCV patients initiating treatment. Total non-GAAP operating expenses for the second quarter of 2013 were consistent with the second quarter of 2012.

**Cash Position:** As of June 30, 2013, Vertex had \$1.43 billion in cash, cash equivalents and marketable securities compared to \$1.32 billion in cash, cash equivalents and marketable securities as of December 31, 2012.

**Convertible Debt:** As of June 30, 2013, Vertex had no outstanding convertible debt following the completion of a call of its outstanding Convertible Senior Subordinated Notes due 2015.

## **2013 Financial Guidance**

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

Vertex today updated its financial guidance for total 2013 revenues and total 2013 KALYDECO net revenues. The company now expects total 2013 revenues to be in the range of \$1.10 billion to \$1.2 billion. The prior range, provided on January 29, 2013, was for total 2013 revenues to be in the range of \$1.10 to \$1.25 billion. The company also now expects total 2013 KALYDECO net revenues to be in the range of \$345 million to \$360 million. The prior range, provided on April 30, 2013, was for total 2013 KALYDECO net revenues to be in the range of \$300 million to \$340 million.

The company today reiterated its financial guidance for total 2013 non-GAAP operating expenses, excluding cost of revenues, stock-based compensation expense, intangible asset impairment charges and Alios expenses related to the accounting for the collaboration with Vertex, of \$1.09 billion to \$1.15 billion, including full-year 2013 non-GAAP R&D expenses of \$750 million to \$790 million and full-year 2013 non-GAAP SG&A expenses of \$340 million to \$360 million.

## **Non-GAAP Financial Measures**

In this press release, Vertex's financial results and financial guidance are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, Vertex provides its non-GAAP net income (loss) for the periods ending June 30, 2013 and 2012 excluding stock-based compensation expense, restructuring expense, inventory reserves, intangible asset impairment charges, net of tax, certain interest expenses related to the 2015 Notes and charges related to changes in the fair value of expected future payments under Vertex's collaboration with Alios. 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 GAAP financial results to non-GAAP financial results is included in the attached financial statements.

**Vertex Pharmaceuticals Incorporated**  
**Second Quarter and Six Month Results**  
**Condensed Consolidated Statements of Operations Data**  
(in thousands, except per share amounts)  
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	2012	2013	2012
Revenues:				
Product revenues, net	\$ 254,789	\$ 373,273	\$ 522,170	\$ 748,648
Royalty revenues	49,120	33,480	92,693	72,461
Collaborative revenues	6,841	11,552	24,255	35,933
Total revenues	<u>310,750</u>	<u>418,305</u>	<u>639,118</u>	<u>857,042</u>
Costs and expenses:				

Cost of product revenues (Note 1)	24,695	104,549	55,650	130,467
Royalty expenses	13,236	9,874	25,024	23,167
Research and development expenses (R&D)	222,455	196,544	440,550	392,915
Sales, general and administrative expenses (SG&A)	106,521	117,514	199,400	228,660
Restructuring expense	776	594	815	954
Intangible asset impairment charge (Note 2)	—	—	412,900	—
Total costs and expenses	367,683	429,075	1,134,339	776,163
Income (loss) from operations	(56,933)	(10,770)	(495,221)	80,879
Other expense, net (Note 3)	(6,578)	(3,635)	(11,230)	(7,376)
Income (loss) before provision for (benefit from) income taxes	(63,511)	(14,405)	(506,451)	73,503
Provision for (benefit from) income taxes (Note 2)	(1,799)	20,063	(132,112)	20,095
Net income (loss)	(61,712)	(34,468)	(374,339)	53,408
Net loss (income) attributable to noncontrolling interest (Note 4)	4,547	(30,463)	9,158	(26,749)
Net income (loss) attributable to Vertex	<u>\$ (57,165)</u>	<u>\$ (64,931)</u>	<u>\$ (365,181)</u>	<u>\$ 26,659</u>

Net income (loss) per share attributable to Vertex common shareholders:

Basic	\$ (0.26)	\$ (0.31)	\$ (1.67)	\$ 0.13
Diluted	\$ (0.26)	\$ (0.31)	\$ (1.67)	\$ 0.12

Shares used in per share calculations:

Basic	222,053	211,344	218,795	209,681
Diluted	222,053	211,344	218,795	212,957

### Consolidated Revenues

(in millions)  
(unaudited)

#### Three Months Ended

	June 30, 2013	March 31, 2013	December 31, 2012	September 31, 2012	June 30, 2012
<b>Product revenues</b>					
INCIVEK revenues, net	\$ 155.8	\$ 205.6	\$ 222.8	\$ 254.3	\$ 327.7
KALYDECO revenues, net	99.0	61.8	58.5	49.2	45.5
Total product revenues, net	254.8	267.4	281.3	303.5	373.3
<b>Royalty revenues</b>					
Royalty revenues from INCIVO	44.1	39.0	36.8	20.0	28.0
Other royalty revenues	5.0	4.5	6.7	5.6	5.5
Total royalty revenues	49.1	43.6	43.5	25.6	33.5
<b>Collaborative revenues</b>	6.8	17.4	9.2	6.9	11.6
<b>Total revenues</b>	<u>\$ 310.8</u>	<u>\$ 328.4</u>	<u>\$ 334.0</u>	<u>\$ 336.0</u>	<u>\$ 418.3</u>

### Reconciliation of GAAP to Non-GAAP Financial Information-Second Quarter

(in thousands, except per share amounts)  
(unaudited)

Three Months  
Ended June 30,  
2013

	GAAP	Adjustments						Non-GAAP
		Alios Transaction	Stock-based Compensation Expense	Inventory Write-off	Intangible Asset Impairment Charge, Net of Tax	Debt Conversion Costs	Restructuring Expense	
Income (loss) from operations	\$(56,933)	7,007	41,263	5,083	—	—	776	\$(2,804)

Other income (expense), net	(6,578)	(183)	—	—	—	3,908	—	(2,853)
Income (loss) before provision for (benefit from) income taxes	(63,511)	6,824	41,263	5,083	—	3,908	776	(5,657)
Provision for (benefit from) income taxes	(1,799)	2,357	—	—	—	—	—	558
Net income (loss)	(61,712)	4,467	41,263	5,083	—	3,908	776	(6,215)
Net loss (income) attributable to noncontrolling interest (Alios)	4,547	(4,547)	—	—	—	—	—	—
Net income (loss) attributable to Vertex	<u>\$(57,165)</u>	<u>(80)</u>	<u>41,263</u>	<u>5,083</u>	<u>—</u>	<u>3,908</u>	<u>776</u>	<u>\$(6,215)</u>

Net income (loss) per diluted share attributable to Vertex common shareholders (Note 5)	\$ (0.26)							\$ (0.03)
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**Three Months Ended June 30, 2012**

	Adjustments							Non-GAAP
	GAAP	Alios Transaction	Stock-based Compensation Expense	Inventory Write-off	Intangible Asset Impairment Charge, Net of Tax	Debt Conversion Costs	Restructuring Expense	
Income (loss) from operations	\$(10,770)	4,646	31,169	78,000	—	—	594	\$103,639
Other income (expense), net	(3,635)	(179)	—	—	—	—	—	(3,814)
Income (loss) before provision for (benefit from) income taxes	(14,405)	4,467	31,169	78,000	—	—	594	99,825
Provision for (benefit from) income taxes	20,063	(21,240)	—	1,239	—	—	—	62
Net income (loss)	(34,468)	25,707	31,169	76,761	—	—	594	99,763
Net loss (income) attributable to noncontrolling interest (Alios)	(30,463)	30,463	—	—	—	—	—	—
Net income (loss) attributable to Vertex	<u>\$(64,931)</u>	<u>56,170</u>	<u>31,169</u>	<u>76,761</u>	<u>—</u>	<u>—</u>	<u>594</u>	<u>\$ 99,763</u>

Net income (loss) per diluted share attributable to Vertex common shareholders (Note 5)	\$ (0.31)							\$ 0.46
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	Three Months Ended June 30,	
	2013	2012
GAAP operating costs and expenses	\$ 367,683	\$ 429,075
Adjustments:		
Cost of product revenues (Note 1)	(24,695)	(104,549)

Royalty expenses	(13,236)	(9,874)
Stock-based compensation expense	(41,263)	(31,169)
Alios transaction	(7,007)	(4,646)
Intangible asset impairment charge (Note 2)	—	—
Restructuring expense	(776)	(594)
<b>Non-GAAP operating costs and expenses</b>	<b>\$ 280,706</b>	<b>\$ 278,243</b>
GAAP research and development expenses	\$ 222,455	\$ 196,544
Adjustments:		
Stock-based compensation expense	(25,700)	(19,665)
Alios transaction (Note 4)	(5,566)	(3,658)
<b>Non-GAAP research and development expenses</b>	<b>\$ 191,189</b>	<b>\$ 173,221</b>
GAAP sales, general, and administrative expenses	\$ 106,521	\$ 117,514
Adjustments:		
Stock-based compensation expense	(15,563)	(11,504)
Alios transaction (Note 4)	(1,441)	(988)
<b>Non-GAAP sales, general, and administrative expenses</b>	<b>\$ 89,517</b>	<b>\$ 105,022</b>

**Reconciliation of GAAP to Non-GAAP Financial Information-Six Month**  
(in thousands, except per share amounts)  
(unaudited)

**Six Months Ended  
June 30, 2013**

	Adjustments							Non-GAAP
	GAAP	Alios Transaction	Stock-based Compensation Expense	Inventory Write-off	Intangible Asset Impairment Charge, Net of Tax	Debt Conversion Costs	Restructuring Expense	
Income (loss) from operations	\$ (495,221)	12,296	72,416	5,083	412,900	—	815	\$ 8,289
Other income (expense), net	(11,230)	(175)	—	—	—	3,908	—	(7,497)
Income (loss) before provision for (benefit from) income taxes	(506,451)	12,121	72,416	5,083	412,900	3,908	815	792
Provision for (benefit from) income taxes	(132,112)	5,783	—	—	127,586	—	—	1,257
Net income (loss)	(374,339)	6,338	72,416	5,083	285,314	3,908	815	(465)
Net loss (income) attributable to noncontrolling interest (Alios)	9,158	(9,158)	—	—	—	—	—	—
Net income (loss) attributable to Vertex	<u>\$ (365,181)</u>	<u>(2,820)</u>	<u>72,416</u>	<u>5,083</u>	<u>285,314</u>	<u>3,908</u>	<u>815</u>	<u>\$ (465)</u>
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 5)	\$ (1.67)							\$ (0.00)

**Six Months Ended  
June 30, 2012**

Adjustments  
Intangible

	GAAP	Alios Transaction	Stock-based Compensation Expense	Inventory Write-off	Asset Impairment Charge, Net of Tax	Debt Conversion Costs	Restructuring Expense	Non-GAAP
Income (loss) from operations	\$80,879	9,732	58,796	78,000	—	—	954	\$228,361
Other income (expense), net	(7,376)	(241)	—	—	—	—	—	(7,617)
Income (loss) before provision for (benefit from) income taxes	73,503	9,491	58,796	78,000	—	—	954	220,744
Provision for (benefit from) income taxes	20,095	(18,960)	—	1,239	—	—	—	2,374
Net income (loss)	53,408	28,451	58,796	76,761	—	—	954	218,370
Net loss (income) attributable to noncontrolling interest (Alios)	(26,749)	26,749	—	—	—	—	—	—
Net income (loss) attributable to Vertex	\$26,659	55,200	58,796	76,761	—	—	954	\$218,370
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 5)	\$ 0.12							\$ 1.01

	Six Months Ended June 30,	
	2013	2012
GAAP operating costs and expenses	\$ 1,134,339	\$ 776,163
Adjustments:		
Cost of product revenues (Note 1)	(55,650)	(130,467)
Royalty expenses	(25,024)	(23,167)
Stock-based compensation expense	(72,416)	(58,796)
Alios transaction	(12,296)	(9,732)
Intangible asset impairment charge (Note 2)	(412,900)	—
Restructuring expense	(815)	(954)
<b>Non-GAAP operating costs and expenses</b>	<b>\$ 555,238</b>	<b>\$ 553,047</b>
GAAP research and development expenses	\$ 440,550	\$ 392,915
Adjustments:		
Stock-based compensation expense	(44,973)	(36,826)
Alios transaction (Note 4)	(9,614)	(7,619)
<b>Non-GAAP research and development expenses</b>	<b>\$ 385,963</b>	<b>\$ 348,470</b>
GAAP sales, general, and administrative expenses	\$ 199,400	\$ 228,660
Adjustments:		
Stock-based compensation expense	(27,443)	(21,970)
Alios transaction (Note 4)	(2,682)	(2,113)
<b>Non-GAAP sales, general, and administrative expenses</b>	<b>\$ 169,275</b>	<b>\$ 204,577</b>

**Condensed Consolidated Balance Sheets Data**

(in thousands)  
(unaudited)

**June 30, 2013 December 31, 2012**



<b>Assets</b>		
Cash, cash equivalents and marketable securities	\$ 1,430,696	\$ 1,321,215
Restricted cash and cash equivalents (Alios) (Note 4)	58,288	69,983
Accounts receivable, net	164,866	143,250
Inventories (Note 1)	19,509	30,464
Other current assets	43,231	24,673
Restricted cash	122	31,934
Property and equipment, net	581,738	433,609
Intangible assets (Note 2)	250,600	663,500
Goodwill	30,992	30,992
Other non-current assets	4,287	9,668
<b>Total assets</b>	<b>\$ 2,584,329</b>	<b>\$ 2,759,288</b>
<b>Liabilities and Shareholders' Equity</b>		
Other liabilities	\$ 387,842	\$ 429,372
Accrued restructuring expense	22,052	23,328
Deferred tax liability (Note 2)	149,706	280,367
Deferred revenues	116,966	123,808
Construction financing lease obligation	359,100	268,031
Convertible notes (due 2015) (Note 3)	—	400,000
Noncontrolling interest (Alios) (Note 4)	226,210	235,202
Shareholders' equity (Vertex)	1,322,453	999,180
<b>Total liabilities and shareholders' equity</b>	<b>\$ 2,584,329</b>	<b>\$ 2,759,288</b>

Common shares outstanding	232,177	217,287
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**Note 1:** In the three and six months ended June 30, 2013 and 2012, the company recorded within cost of product revenues reserves for excess and obsolete inventories of \$5.1 million and \$78.0 million, respectively.

**Note 2:** As of June 30, 2013, the intangible assets and deferred tax liability reflected in the condensed consolidated balance sheet relate to the company's collaboration agreement with Alios BioPharma, Inc. (Alios).

In the first quarter of 2013, the company determined that the value of VX-222 had become impaired and that the fair value of VX-222 was zero as of March 31, 2013. This resulted in a \$412.9 million impairment charge. In connection with this impairment charge, the company recorded a credit of \$127.6 million in its provision for income taxes.

**Note 3:** In the second quarter of 2013, the company elected to redeem \$400.0 million in aggregate principal amount of 3.35% convertible senior subordinated notes due 2015 ("2015 Notes"). In response, the holders of the 2015 Notes converted their 2015 Notes into approximately 8.2 million shares of the company's common stock. In accordance with the terms of the 2015 Notes, the company made additional make-whole interest payments of \$6.7 million, payable in shares of the company's common stock.

**Note 4:** The company has consolidated the financial statements of its collaborator Alios as of June 30, 2013, December 31, 2012, and for the three and six months ended June 30, 2013 and 2012. The company's interest and obligations with respect to Alios' assets and liabilities are limited to those accorded to the company in its collaboration agreement with Alios. Restricted cash and cash equivalents (Alios) reflects Alios' cash and cash equivalents, which Vertex does not have any interest in and which will not be used to fund the collaboration. Each reporting period Vertex estimates the fair value of the contingent milestone payments and royalties payable by Vertex to Alios. Any increase in the fair value of these contingent milestone and royalty payments results in a decrease in net income attributable to Vertex (or an increase in net loss attributable to Vertex) on a dollar-for-dollar basis.

**Note 5:** Shares used in non-GAAP net income (loss) per diluted share attributable to Vertex common shareholders were 222,053,000 and 224,124,000 for the three months ended June 30, 2013 and 2012, respectively, and 218,795,000 and 221,694,000 for the six months ended June 30, 2013 and 2012, respectively.

#### **Indication and Important Safety Information for KALYDECO™ (ivacaftor)**

Ivacaftor (150mg tablets) is indicated for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have a G551D mutation in the *CFTR* gene.

Ivacaftor is not for use in people with CF due to other mutations in the *CFTR* gene. It is not effective in CF patients with two copies of the F508del mutation (F508del/F508del) in the *CFTR* gene. The efficacy and safety of ivacaftor in children younger than 6 years of age have not been evaluated.

High liver enzymes (transaminases, ALT and AST) have been reported in patients receiving ivacaftor. It is recommended that ALT and AST be assessed prior to initiating ivacaftor, every 3 months during the first year of treatment, and annually thereafter. Patients who develop increased transaminase levels should be closely monitored until the abnormalities resolve. Dosing should be interrupted in patients with ALT or AST of greater than 5 times the upper limit of normal. Following resolution of transaminase elevations, consider the benefits and risks of resuming ivacaftor dosing. Moderate transaminase elevations are common in subjects with CF. Overall, the incidence and clinical features of transaminase elevations in clinical trials was similar between subjects in the ivacaftor and placebo treatment groups. In the subset of patients with a medical history of elevated transaminases, increased ALT or AST have been reported more frequently in patients receiving ivacaftor compared to placebo.

Use of ivacaftor with medicines that are strong CYP3A inducers such as the antibiotics rifampin and rifabutin; seizure medications (phenobarbital, carbamazepine, or phenytoin); and the herbal supplement St. John's Wort substantially decreases exposure of ivacaftor, which may diminish effectiveness. Therefore, co-administration is not recommended.

The dose of ivacaftor must be adjusted when concomitantly used with potent and moderate CYP3A inhibitors. The dose of ivacaftor must be adjusted when used in patients with moderate or severe hepatic disease.

Ivacaftor can cause serious adverse reactions including abdominal pain and high liver enzymes in the blood. The most common side effects associated with ivacaftor include headache; upper respiratory tract infection (the common cold), including sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; and dizziness. These are not all the possible side effects of ivacaftor. A list of the adverse reactions can be found in the full product labeling for each country where ivacaftor is approved. Patients should tell their healthcare providers about any side effect that bothers them or doesn't go away.

Please see full U.S. Prescribing Information for KALYDECO at [www.KALYDECO.com](http://www.KALYDECO.com), the EU Summary of Product Characteristics for KALYDECO at <http://goo.gl/N3Tz4>, and the KALYDECO Canadian Product Monograph at [www.vrtx.ca](http://www.vrtx.ca).

## Indication and Important Safety Information for INCIVEK (telaprevir)

INCIVEK® (telaprevir) is a prescription medicine used with the medicines peginterferon alfa and ribavirin to treat chronic (lasting a long time) hepatitis C genotype 1 infection in adults with stable liver problems, who have not been treated before or who have failed previous treatment. It is not known if INCIVEK is safe and effective in children under 18 years of age.

### Important Safety Information

INCIVEK® (telaprevir) should always be used in combination with peginterferon alfa and ribavirin. INCIVEK combination treatment may cause serious side effects including skin rash and serious skin reactions, anemia (low red blood cell count) that can be severe, and birth defects or death of an unborn baby.

**Skin rashes are common with INCIVEK combination treatment. Sometimes these skin rashes and other skin reactions can become serious, require treatment in a hospital, and may lead to death. Patients should call their healthcare provider right away if they develop any skin changes during treatment with INCIVEK.** Their healthcare provider will decide if they need treatment or if they need to stop INCIVEK or any of their other medicines. Patients should not stop taking INCIVEK combination treatment without talking with their healthcare provider first.

Patients' healthcare providers will do blood tests regularly to check for anemia. If anemia is severe, the healthcare providers may tell them to stop taking INCIVEK.

INCIVEK combined with peginterferon alfa and ribavirin may cause birth defects or death of an unborn baby. Therefore, a patient should not take INCIVEK combination treatment if she is pregnant or may become pregnant, or if he is a man with a sexual partner who is pregnant. Females who can become pregnant and females whose male partner takes these medicines must have a negative pregnancy test before starting treatment, every month during treatment, and for 6 months after treatment ends. Patients must use two forms of effective birth control during treatment and for 6 months after all treatment has ended. These two forms of birth control should not contain hormones, as these may not work during treatment with INCIVEK.

INCIVEK and other medicines can affect each other and can also cause side effects that can be serious or life-threatening. There are certain medicines patients cannot take with INCIVEK combination treatment. Patients should tell their healthcare providers about all the medicines they take, including prescription and non-prescription medicines, vitamins and herbal

supplements.

The most common side effects of INCIVEK combination treatment include itching, nausea, diarrhea, vomiting, anal or rectal problems (including hemorrhoids, discomfort, burning or itching around or near the anus), taste changes and tiredness. There are other possible side effects of INCIVEK, and side effects associated with peginterferon alfa and ribavirin also apply to INCIVEK combination treatment. Patients should tell their healthcare provider about any side effect that bothers them or doesn't go away.

Please see full Prescribing Information including Boxed Warning, and the Medication Guide for INCIVEK available at [www.INCIVEK.com](http://www.INCIVEK.com).

## **About Vertex**

Vertex creates new possibilities in medicine. Our team discovers, develops and commercializes innovative therapies so people with serious diseases can lead better lives.

Vertex scientists and our collaborators are working on new medicines to cure or significantly advance the treatment of hepatitis C, cystic fibrosis, rheumatoid arthritis and other life-threatening diseases.

Founded more than 20 years ago in Cambridge, Mass., we now have ongoing worldwide research programs and sites in the U.S., U.K. and Canada. Today, Vertex has more than 2,000 employees around the world, and for three years in a row, *Science* magazine has named Vertex one of its Top Employers in the life sciences.

## **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, without limitation, Dr. Leiden's statements in the second paragraph of the press release, the information provided in the section captioned "2013 Financial Guidance" and statements regarding (i) Vertex's strategies in cystic fibrosis, HCV and autoimmune diseases; (ii) the timing of initiation or completion of enrollment and/or receipt of clinical data for clinical trials; (iii) the timing of potential regulatory filings, including potential sNDAs and New Drug Application filings in the U.S. and an MAA variation filing in Europe; (iv) the percentage of patients that Vertex may be able to treat with ivacaftor monotherapy; (v) ongoing and planned studies involving VX-135; (vi) the goal of advancing a second-generation corrector into clinical development by the end of 2014 and (vii) the evaluation of collaborative opportunities that could providing funding and capabilities to broaden and accelerate global development of VX-509. While Vertex 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 company's expectations regarding its 2013 revenues and/or operating expenses may be incorrect (including because one or more of the company's assumptions underlying its revenue or expense expectations may not be realized), that the outcomes of Vertex's ongoing and planned clinical studies may not be favorable, that the initiation of planned studies may be delayed or prevented, 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). Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

## **Conference Call and Webcast**

Vertex will host a conference call and webcast today, July 29, 2013 at 5:00 p.m. ET to review financial results and recent developments. The conference call will be webcast live, and a link to the webcast may be accessed from the 'Vertex Events' page of Vertex's website at [www.vrtx.com](http://www.vrtx.com).

To listen to the live call on the telephone, dial 1-866-501-1537 (United States and Canada) or 1-720-545-0001 (International). To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast.

The conference ID number for the live call and replay is 13583425 .

The call will be available for replay via telephone commencing July 29, 2013 at 8:00 p.m. ET running through 5:00 p.m. ET on August 5, 2013. The replay phone number for the United States and Canada is 1-855-859-2056. The international replay number is 1-404-537-3406.

Following the live webcast, an archived version will be available on Vertex's website until 5:00 p.m. ET on August 5, 2013. Vertex is also providing a podcast MP3 file available for download on the Vertex website at [www.vrtx.com](http://www.vrtx.com).

(VRTX-GEN)

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