Vertex Reports Second-Quarter 2018 Financial Results

July 25, 2018

-Second-quarter 2018 total CF product revenues of $750 million, a 46% increase compared to $514 million in the second quarter of 2017-

-Company increases full-year 2018 total CF product revenue guidance to $2.9 to $3.0 billion; reiterates full-year 2018 combined non-GAAP R&D and SG&A expense guidance of $1.50 to $1.55 billion-

-Provides update for triple combination regimens for CF; Phase 3 programs for both VX-659 and VX-445 expected to complete enrollment in the second half of 2018-

BOSTON--(BUSINESS WIRE)--Jul. 25, 2018-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the second quarter ended June 30, 2018 and reviewed recent progress with its approved and investigational medicines. Vertex also updated its guidance for full-year 2018 total CF product revenues and reiterated its guidance for combined GAAP and non-GAAP R&D and SG&A expenses.

Second-Quarter 2018 Financial Highlights

	Three Months Ended June 30,		
	2018	2017	% Change
	(in millions, except per share and percentage data)		
TOTAL CF product revenues, net	$ 750	$ 514	46%
GAAP net income	$ 207	$ 18	
GAAP net income per share - diluted	$ 0.80	$ 0.07	
Non-GAAP net income	$ 244	$ 99	147%
Non-GAAP net income per share - diluted	$ 0.94	$ 0.39	141%

“We have made tremendous progress across our business in the first half of 2018 marked by the successful launch of SYMDEKO, the fast enrollment of two Phase 3 programs for our triple combination regimens, and continued success in bringing our CF medicines to more people outside of the U.S.,” said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. “As we enter the second half of the year, we are well positioned to continue our progress toward developing new medicines for all people with CF as well as advancing other transformative medicines outside of CF from research into development.”

Second-Quarter 2018 CF Net Product Revenues

	Three Months Ended June 30,	
	2018	2017
	(in millions)	
TOTAL CF product revenues, net	$ 750	$ 514
KALYDECO product revenues, net	$ 253	$ 190
ORKAMBI product revenues, net	$ 311	$ 324
SYMDEKO product revenues, net	$ 186	$ —

Total CF net product revenues increased 46% compared to the second quarter of 2017 driven by the rapid uptake of SYMDEKO in the U.S. across all eligible patients.

Second-Quarter 2018 R&D and SG&A Expenses

	Three Months Ended June 30,	
	2018	2017
	(in millions)	
Combined GAAP R&D and SG&A expenses	$ 475	$ 417
GAAP R&D expense	$ 338	$ 289
GAAP SG&A expense	$ 137	$ 127
Combined Non-GAAP R&D and SG&A expenses	$ 388	$ 333
Non-GAAP R&D expense	$ 281	$ 240
Non-GAAP SG&A expense	$ 107	$ 93

Combined GAAP and non-GAAP R&D and SG&A expenses increased compared to the second quarter of 2017 due to the advancement of the company's portfolio of triple combination regimens for CF and investments to support the treatment of CF globally.

Non-GAAP net income increased $145 million to $244 million compared to the second quarter of 2017 largely driven by the strong growth in total CF product revenues. GAAP net income increased $189 million to $207 million compared to the second quarter of 2017 due to an increase in CF product revenues and an increase of $53.9 million in the fair value of the company's strategic investments in CRISPR Therapeutics.

Cash, cash equivalents and marketable securities as of June 30, 2018 were approximately $2.8 billion, an increase of approximately $700 million compared to $2.1 billion as of December 31, 2017.

2018 Financial Guidance

Vertex today increased its full-year 2018 total CF product revenue guidance and reiterated guidance for combined GAAP and non-GAAP R&D and SG&A expenses as summarized below:

	Current FY 2018	Previous FY 2018
TOTAL CF product revenues	$ 2.9 - 3.0 billion	$2.65 - 2.80 billion
Combined GAAP R&D and SG&A expenses	$1.80 - 1.95 billion	Unchanged
Combined Non-GAAP R&D and SG&A expenses	$1.50 - 1.55 billion	Unchanged

The increase in total CF product revenue guidance reflects the continued rapid uptake and strong demand for SYMDEKO in the U.S. among people ages 12 and older.

Business Highlights

TRIPLE COMBINATION REGIMENS

In the second half of 2018, Vertex expects to complete enrollment across all four studies of its Phase 3 programs evaluating VX-659 and VX-445 in people with CF ages 12 and older who have one *F508del* mutation and one minimal function mutation and who have two copies of the *F508del* mutation. Based on the anticipated completion of enrollment for both programs, the company expects to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) no later than mid-2019.

APPROVED CF MEDICINES

Strong demand for SYMDEKO: In the second quarter of 2018, Vertex saw strong demand for SYMDEKO following the U.S. launch in February 2018. SYMDEKO has received broad access and coverage from commercial and government payers, which is similar to past U.S. launches of KALYDECO and ORKAMBI.

On June 28, 2018, SYMDEKO was approved in Canada to treat people ages 12 and older who have two copies of the *F508del* mutation or who have one copy of the *F508del* mutation and one of the following mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene: P67L, D110H, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272-26A→G, and 3849+10kbC→T. Vertex expects approval for the tezacaftor/ivacaftor combination in the European Union (EU) in the second half of 2018.

Establishing long-term reimbursement outside of the U.S.: On June 28, 2018, Vertex announced that it reached a pricing and reimbursement agreement for ORKAMBI in Sweden. The innovative, long-term access agreement also includes a framework for assessment and access to Vertex's future CF medicines. In addition to Sweden, ORKAMBI is also reimbursed for eligible people with CF in Austria, Denmark, Germany, Ireland, Italy, Luxembourg and the Netherlands. Vertex continues to work toward establishing pricing and reimbursement agreements in other countries in the EU, Canada and Australia to ensure all eligible patients have access to this important medicine as quickly as possible.

Treating patients at younger ages with CFTR modulators: The company continues to make significant progress toward developing CF medicines to be used earlier in the course of disease progression. Recent highlights include:

- Data expected in the second half of 2018 from a Phase 3 study evaluating **ivacaftor** in infants ages 6 to <12 months.
- Data expected in the second half of 2018 from a Phase 3 study evaluating **tezacaftor/ivacaftor** in children ages 6 to 11.
- Phase 3 study evaluating **lumacaftor/ivacaftor** in children ages 12 to <24 months is planned to start in the second half of 2018.
- Pending approval of **ivacaftor** in children ages 12 to <24 months with a Prescription Drug User Fee Act (PDUFA) action date of August 15, 2018.
- Pending approval of **lumacaftor/ivacaftor** in children ages 2 to 5 years old with a PDUFA date of August 7, 2018.

SICKLE CELL DISEASE & β-THALASSEMIA

In beta thalassemia, Vertex and its partner CRISPR Therapeutics obtained approval in the U.K. for a Clinical Trial Application (CTA) for CTX001 earlier this year and recently obtained a CTA approval in Canada. The companies remain on track to initiate the first study of CTX001 in beta thalassemia later this year. In sickle cell disease, the companies also recently obtained CTA approvals in Canada and the U.K. and continue to work with the FDA to address the agency's questions regarding the IND for CTX001 that was submitted earlier this year.

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, non-GAAP financial results and guidance exclude (i) stock-based compensation expense, (ii) revenues and expenses related to business development transactions including collaboration agreements, asset acquisitions and consolidated variable interest entities, (iii) non-operating tax adjustments and (iv) other adjustments, including gains or losses related to the fair value of the company's strategic investments in CRISPR and Moderna Therapeutics, Inc. 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 the company's 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. The company adjusts, where appropriate, for both revenues and expenses in order to reflect the company's operations. The company provides guidance regarding product revenues in accordance with GAAP and provides guidance regarding combined research and development and sales, general, and administrative expenses on both a GAAP and a non-GAAP basis. The guidance regarding GAAP research and development expenses and sales, general and administrative expenses does not include estimates regarding expenses associated with any potential future business development activities. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial information.

Vertex Pharmaceuticals Incorporated
Second-Quarter Results
Consolidated Statements of Operations Data
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	**2017**	**2018**	**2017**
Revenues:				
Product revenues, net	$ 749,912	$ 513,988	$ 1,387,641	$ 994,610
Royalty revenues	1,085	2,861	2,441	4,412
Collaborative revenues (Note 1)	1,160	27,286	2,874	259,831
Total revenues	752,157	544,135	1,392,956	1,258,853
Costs and expenses:				
Cost of sales	104,382	71,205	175,995	118,193

Research and development expenses	337,532	289,451	648,085	563,014
Sales, general and administrative expenses	137,303	127,249	267,111	240,575
Restructuring expenses (income)	62	3,523	(14)	13,522
Total costs and expenses	579,279	491,428	1,091,177	935,304
Income from operations	172,878	52,707	301,779	323,549
Interest expense, net	(10,106)	(14,664)	(21,203)	(31,429)
Other income (expense), net (Note 2)	53,819	(2,537)	150,657	(3,081)
Income from operations before provision for (benefit from) income taxes (Note 3)	216,591	35,506	431,233	289,039
Provision for (benefit from) income taxes (Note 3)(Note 4)	10,341	4,337	(2,318)	8,322
Net income	206,250	31,169	433,551	280,717
Loss (income) attributable to noncontrolling interest (Note 3)	1,110	(13,173)	(15,928)	(14,965)
Net income attributable to Vertex	$ 207,360	$ 17,996	$ 417,623	$ 265,752
Amounts per share attributable to Vertex common shareholders:				
Net income:				
Basic	$ 0.82	$ 0.07	$ 1.65	$ 1.08
Diluted	$ 0.80	$ 0.07	$ 1.61	$ 1.06
Shares used in per share calculations:				
Basic	254,135	247,521	253,685	246,782
Diluted	258,584	251,635	258,557	250,199

Reconciliation of GAAP to Non-GAAP Net Income
Second-Quarter Results
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	**2017**	**2018**	**2017**
GAAP net income attributable to Vertex	$ 207,360	$ 17,996	$ 417,623	$ 265,752
Stock-based compensation expense	82,436	72,582	160,572	141,564
Collaborative and transaction revenues and expenses (Note 5)	1,352	4,051	25,898	(222,249)
Other adjustments (Note 6)	(52,092)	4,268	(147,254)	15,236
Non-operating tax adjustments (Note 7)	5,030	—	(16,829)	—
Non-GAAP net income attributable to Vertex	$ 244,086	$ 98,897	$ 440,010	$ 200,303
Amounts per diluted share attributable to Vertex common shareholders:				
GAAP	$ 0.80	$ 0.07	$ 1.61	$ 1.06
Non-GAAP	$ 0.94	$ 0.39	$ 1.70	$ 0.80
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	258,584	251,635	258,557	250,199

Reconciliation of GAAP to Non-GAAP Revenues and Expenses
Second-Quarter Results
(in thousands)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	**2017**	**2018**	**2017**
GAAP total revenues	$ 752,157	$ 544,135	$ 1,392,956	$ 1,258,853
Collaborative and transaction revenues (Note 5)	(941)	(27,222)	(2,860)	(259,684)
Non-GAAP total revenues	$ 751,216	$ 516,913	$ 1,390,096	$ 999,169

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
GAAP cost of sales	$ 104,382	$ 71,205	$ 175,995	$ 118,193
Stock-based compensation expense (Note 8)	(1,191)	—	(2,004)	—
Non-GAAP cost of sales	$ 103,191	$ 71,205	$ 173,991	$ 118,193
GAAP research and development expenses	$ 337,532	$ 289,451	$ 648,085	$ 563,014
Stock-based compensation expense	(51,612)	(43,832)	(100,100)	(88,669)
Collaborative and transaction expenses (Note 5)	(3,605)	(5,024)	(5,460)	(7,033)
Other adjustments (Note 6)	(1,522)	(136)	(1,740)	(272)
Non-GAAP research and development expenses	$ 280,793	$ 240,459	$ 540,785	$ 467,040
GAAP sales, general and administrative expenses	$ 137,303	$ 127,249	$ 267,111	$ 240,575
Stock-based compensation expense	(29,633)	(28,750)	(58,468)	(52,895)
Collaborative and transaction expenses (Note 5)	(240)	(4,984)	(1,415)	(6,988)
Other adjustments (Note 6)	(242)	(609)	(396)	(1,442)
Non-GAAP sales, general and administrative expenses	$ 107,188	$ 92,906	$ 206,832	$ 179,250
Combined non-GAAP R&D and SG&A expenses	$ 387,981	$ 333,365	$ 747,617	$ 646,290

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
GAAP interest expense, net and other income (expense), net	$ 43,713	$ (17,201)	$ 129,454	$ (34,510)
Collaborative and transaction expenses (Note 5)	(26)	(40)	(34)	(74)
Other adjustments (Note 6)	(53,918)	—	$ (149,376)	—
Non-GAAP interest expense, net and other (income) expense, net	$ (10,231)	$ (17,241)	$ (19,956)	$ (34,584)
GAAP provision for (benefit from) income taxes	$ 10,341	$ 4,337	$ (2,318)	$ 8,322
Collaborative and transaction expenses (Note 5)	416	(8,132)	(5,989)	(8,523)
Non-operating tax adjustments (Note 7)	(5,030)	—	16,829	—
Non-GAAP provision for (benefit from) income taxes (Note 4)	$ 5,727	$ (3,795)	$ 8,522	$ (201)

Condensed Consolidated Balance Sheets Data
(in thousands)
(unaudited)

	June 30, 2018	December 31, 2017
Assets		
Cash, cash equivalents and marketable securities	$ 2,767,755	$ 2,088,666
Accounts receivable, net	393,439	281,343
Inventories	115,025	111,830
Property and equipment, net	815,928	789,437
Intangible assets and goodwill	79,384	79,384
Other assets	163,855	195,354
Total assets	$ 4,335,386	$ 3,546,014
Liabilities and Shareholders' Equity		
Accounts payable and accruals	$ 562,154	$ 517,955
Other liabilities	478,889	415,501
Deferred tax liability	9,335	6,341
Construction financing lease obligation	570,125	563,911

Shareholders' equity	2,714,883	2,042,306
Total liabilities and shareholders' equity	$ 4,335,386	$ 3,546,014
Common shares outstanding	254,883	253,253

Note 1: In the six months ended June 30, 2017, collaborative revenues were primarily attributable to a $230.0 million upfront payment earned from the company's collaboration with Merck KGaA, Darmstadt, Germany. During the three and six months ended June 30, 2017, collaborative revenues includes $20.0 million that Parion Science Inc., a company that Vertex consolidated as a variable interest entities ("VIE") during the first three quarters of 2017, earned from a collaboration agreement with a third party.

Note 2: The company recorded gains of $53.9 million and $146.5 million to "Other income (expense), net" in the three and six months ended June 30, 2018, respectively, related to increases in the fair value of the company's investment in CRISPR Therapeutics AG. The company adopted ASU No. 2016-01, *Recognition and Measurement of Financial Assets and Financial Liabilities* effective January 1, 2018. Prior to the adoption of ASU 2016-01 on January 1, 2018, changes in the fair value of the company's investment in CRISPR were recorded to equity on the company's condensed consolidated balance sheets until the related gains and losses were realized; therefore, there was no comparable income in the three and six months ended June 30, 2017.

Note 3: The company consolidates the financial statements of one of its collaborators, BioAxone Biosciences, Inc. during the three and six months of 2018 and 2017. BioAxone is consolidated because Vertex has licensed the rights to develop its most significant intellectual property asset. Each reporting period Vertex estimates the fair value of the contingent payments by Vertex to BioAxone. Any increase in the fair value of these contingent payments results in a decrease in net income attributable to Vertex on a dollar-for-dollar basis. The fair value of contingent payments is evaluated each quarter and any change in the fair value is reflected in the company's statement of operations. Vertex also consolidated Parion during the first three quarters of 2017.

Note 4: The company continues to maintain a valuation allowance on the majority of its net operating losses and other deferred tax assets. Due to this valuation allowance, the company did not record a significant provision for income taxes in the three and six months of 2018 and 2017. The company is profitable from a U.S. federal income tax perspective and has used a portion of its net operating losses to offset this income since becoming profitable. The company may release all or a portion of the valuation allowance in the near-term; however, the release of the valuation allowance, as well as the exact timing and amount of such release, continue to be subject to, among other things, the company's level of profitability, revenue growth, clinical program progression and expectations regarding future profitability. In the period of the release of the valuation allowance, the company will recognize a significant non-cash credit to net income and will reflect a deferred tax asset, which is currently subject to the valuation allowance, on its condensed consolidated balance sheet. Following the release, the company expects to continue to utilize its net operating losses to offset income, but would begin recording a provision for income taxes reflecting the utilization of the deferred tax assets. As of December 31, 2017, the company's U.S. Federal net operating loss carry forwards totaled approximately $3.6 billion and its total deferred tax asset balance subject to the valuation allowance was approximately $1.6 billion.

Note 5: In the three and six months ended June 30, 2018 and 2017, "Collaborative and transaction revenues and expenses" primarily consisted of (i) revenues and operating costs and expenses attributable to BioAxone, (ii) changes in the fair value of contingent milestone payments and royalties payable by Vertex to BioAxone, and (iii) collaboration revenues and payments. "Collaborative and transaction revenues and expenses" included a $22.9 million increase in the fair value of contingent milestone payments and royalties payable by Vertex to BioAxone that were attributable to Vertex in the six months ended June 30, 2018. "Collaborative and transaction revenues and expenses" included (i) the $230.0 million upfront payment earned from Merck KGaA in the six months ended June 30, 2017 and (ii) the $20.0 million of collaborative revenues related to Parion in the three and six months ended June 30, 2017, both of which are discussed in Note 1.

Note 6: In the three and six months ended June 30, 2018, "Other adjustments" primarily consisted of the increase in fair value of the company's investment in CRISPR Therapeutics AG discussed in Note 2 above as well as a $2.9 million increase in the fair value of the company's investment in Moderna Therapeutics, Inc. that was recorded in the three months ended March 31, 2018. In the three and six months ended June 30, 2017, "Other adjustments" primarily consisted of restructuring charges related to the company's decision to consolidate its research activities into its Boston, Milton Park and San Diego locations and to close its research site in Canada.

Note 7: In the three and six months ended June 30, 2018, "Non-operating tax adjustments" included discrete items related to stock-based compensation. On a GAAP basis, the company recorded a provision for income taxes related to stock-based compensation of $5.0 million in the three months ended June 30, 2018 and a benefit from stock-based compensation of $16.8 million in the six months ended June 30, 2018. The company expects the net benefit from income taxes for the six months ended June 30, 2018, as well as any amounts recorded in the third quarter of 2018, to reverse in the fourth quarter of 2018 resulting in no effect on its GAAP annual provision for income taxes. Accordingly, the company is excluding these adjustments from its Non-GAAP measures.

Note 8: In the three and six months ended June 30, 2018, “Cost of sales” included $1.2 million and $2.0 million, respectively, in stock-based compensation expense. In the three and six months ended June 30, 2017, “Cost of sales” included $0.5 million and $1.0 million, respectively, in stock-based compensation expense. Beginning with the first quarter of 2018, the company is adjusting

for the stock-based compensation expense recorded in “Cost of sales” in its reconciliation of "Non-GAAP net income attributable to Vertex" and "Non-GAAP cost of sales". In its Non-GAAP reconciliation, the company is not adjusting for the stock-based compensation expense recorded in “Cost of sales” for the three and six months ended June 30, 2017.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases. In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases.

Founded in 1989 in Cambridge, Mass., Vertex's headquarters is now located in Boston'sInnovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada and Australia. Vertex is consistently recognized as one of the industry's top places to work, including being named to *Science* magazine's Top Employers in the life sciences ranking for eight years in a row.

For additional information and the latest updates from the company, please visit www.vrtx.com.

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 this press release, the information provided in the sections captioned "2018 Financial Guidance" and statements regarding (i) the timing and expected outcome of regulatory applications, including NDAs and MAAs and (ii) the development plan and timelines for our product development candidates, including tezacaftor in combination with ivacaftor and our next-generation triple combination regimens. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and 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 2018 CF net product revenues and expenses may be incorrect (including because one or more of the company's assumptions underlying its expectations may not be realized), that data from the company's development programs may not support registration or further development of its compounds due to safety, efficacy or other reasons, 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.

Conference Call and Webcast

The company will host a conference call and webcast today at 4:30 p.m. ET. To access the call, please dial (866) 501-1537 (U.S.) or +1 (720) 545-0001 (International). The conference call will be webcast live and a link to the webcast can be accessed through Vertex's website at www.vrtx.com in the "Investors" section under "Events and Presentations." To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast. An archived webcast will be available on the company's website.

(VRTX-E)

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