



# Full-Year and Fourth-Quarter 2018 Financial Results



February 5, 2019

# Agenda

## Introduction

*Michael Partridge, Senior Vice President, Investor Relations*

## Business Highlights

*Jeff Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer*

## Commercial Update

*Stuart Arbuckle, Executive Vice President and Chief Commercial Officer*

## Financial Results

*Paul Silva, Senior Vice President and Interim Chief Financial Officer*

## Q&A

*Reshma Kewalramani, M.D., Executive Vice President and Chief Medical Officer*

# Safe Harbor Statement & Non-GAAP Financial Measures

This presentation contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, (i) information pertaining to our medicines and the ongoing discovery, development and commercialization of Vertex's product candidates, (ii) 2019 financial guidance and (iii) Vertex's 2019 key milestones and goals. While the Company believes that these forward-looking statements are accurate, these statements are subject to risks and uncertainties that could cause actual outcomes to differ materially from the Company's current expectations. These risks and uncertainties include, among other things, the risk 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, the Company's expectations regarding future financial performance may be incorrect, and the risks and uncertainties listed under Risk Factors in the Company's 10-K and other filings with the SEC.

In this presentation, 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 from Vertex's pre-tax net income (i) stock-based compensation expense, (ii) revenues and expenses related to business development transactions including collaboration agreements and asset acquisitions, (iii) revenues and expenses related to consolidated variable interest entities, including asset impairment charges and the effects of the deconsolidation of variable interest entities in 2017 and 2018 and (iv) other adjustments, including gains or losses related to the fair value of the company's strategic investments. The company's non-GAAP financial results also exclude from its provision for or benefit from income taxes (i) the estimated tax impact related to its non-GAAP adjustments to pre-tax net income described above as well as (ii) non-operating tax adjustments, which are not associated with Vertex's normal, recurring operations and include the release of the company's valuation allowance on the majority of its net operating losses and other deferred tax assets in the fourth quarter of 2018. 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 and its anticipated income taxes as a percentage of pre-tax net income 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 associated with any potential future business development activities. The guidance regarding the GAAP effective tax rate is based on currently available information and could be lower than the current guidance of 21 to 22% due to actual value of equity exercises by employees in 2019, geographic mix of business and business development activities. The most recent reconciliation of the GAAP financial results to non-GAAP financial results is included in the Company's February 5, 2019 press release.

# Vertex Strategy and Business Model

## *A Blueprint for Serial Innovation*

### CORPORATE STRATEGY

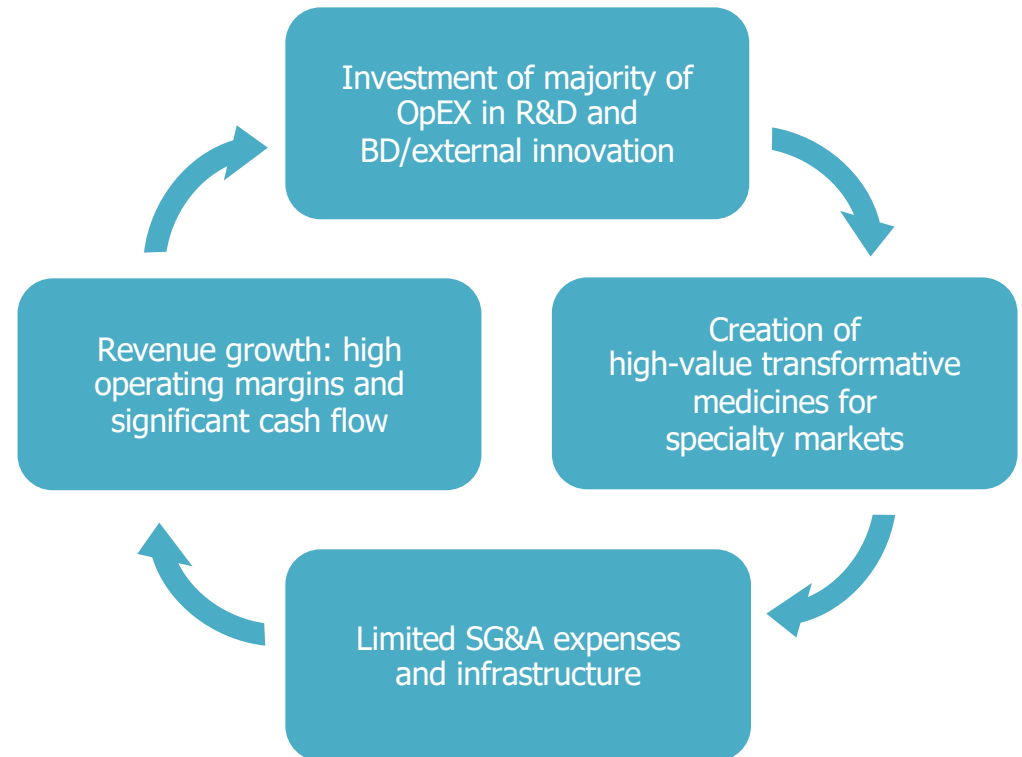
Vertex invests in **scientific innovation** to create **transformative medicines** for people with **serious diseases** with a focus on **specialty markets**

### RESEARCH STRATEGY

Combine **transformative advances** in the **understanding of human disease** and in the **science of therapeutics** to dramatically advance human health

- Focus on **validated targets** that address causal human biology
- Create **predictive lab assays** and **clinical biomarkers**
- Identify **rapid path to registration** and approval
- Discover and develop medicines that offer transformative benefit, **regardless of modality**

### DIFFERENTIATED BUSINESS MODEL



# 2018 Achievements and 2019 Milestones

2018

2019

## **ACHIEVE OUR VISION IN CYSTIC FIBROSIS**

- Phase 2 data for triple combinations in CF patients
- Initiation of pivotal development of up to two triple combination regimens
- Approval for tezacaftor/ivacaftor combination in the U.S. (Europe anticipated in 2H 2018)
- Advance additional next-generation correctors into development

- Phase 3 data for VX-445 in patients ages 12+
- Submit NDA for a triple combination regimen no later than mid-2019
- U.S. approval for SYMDEKO for children ages 6 through 11
- Initiate POC study of additional next-generation corrector
- Phase 2 dose-ranging study of potential once-daily regimen VX-561

## **EXPAND PIPELINE BEYOND CF**

- Advance one or more compounds from research into clinical development
- Initiate clinical development of CRISPR-Cas9 treatment in Beta Thalassemia & Sickle Cell Disease

- Complete Phase 1 studies in at least two new diseases
- Bolster pipeline with internal and external assets
- Advance one or more compounds from research into clinical development

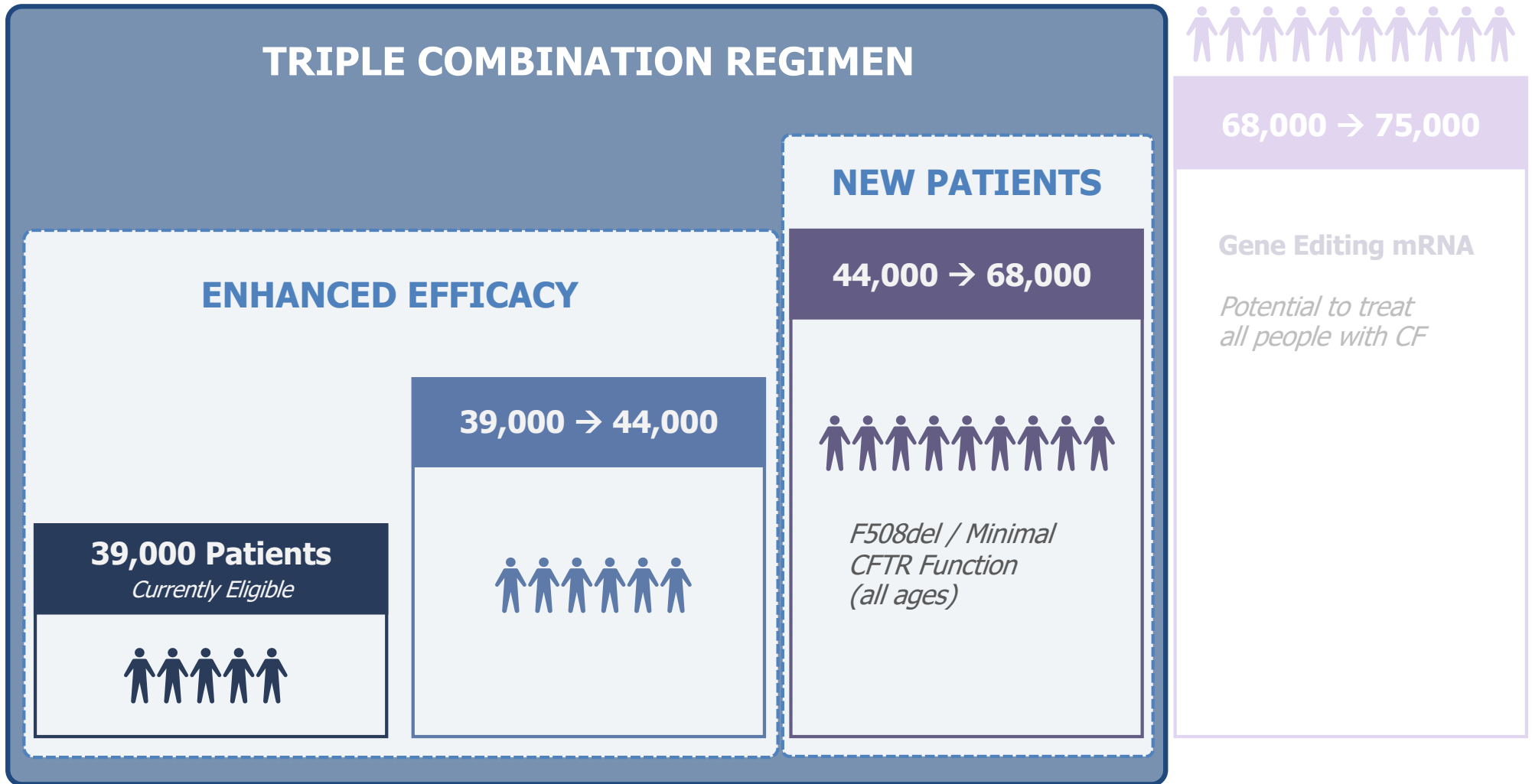
## **BUILD FINANCIAL STRENGTH**

- Significantly increase 2018 total CF product revenues
- Obtain reimbursement for ORKAMBI in additional countries outside the U.S.
- Continued management of non-GAAP combined R&D and SG&A expenses
- Continue to increase operating margins and cash flows

- Continued CF product revenue growth
- Continued uptake and reimbursement for ORKAMBI and SYMDEKO in additional countries outside the U.S.
- Continued management of non-GAAP combined R&D and SG&A expenses
- Continued expansion of non-GAAP operating margins and cash flow



# Triple Combination Regimens Have Potential to Treat Up to 90% of CF Patients



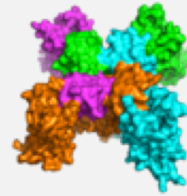
# Beyond CF

## *Multiple Opportunities for Transformative Medicines*



### **Alpha-1 Antitrypsin Deficiency**

First molecule advanced into clinical development in December 2018; Multiple additional molecules in late preclinical development



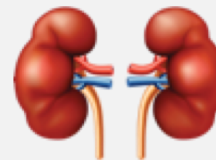
### **Pain**

First selective NaV1.8 inhibitor (VX-150) to demonstrate proof-of-concept in acute, inflammatory and neuropathic pain; Ongoing research to discover/develop additional NaV1.8 inhibitors and other potential pain molecules



### **Sickle Cell Disease / Beta Thalassemia**

Initiated Phase 1/2 studies of gene editing therapy CTX001



### **Focal Segmental Glomerulosclerosis**

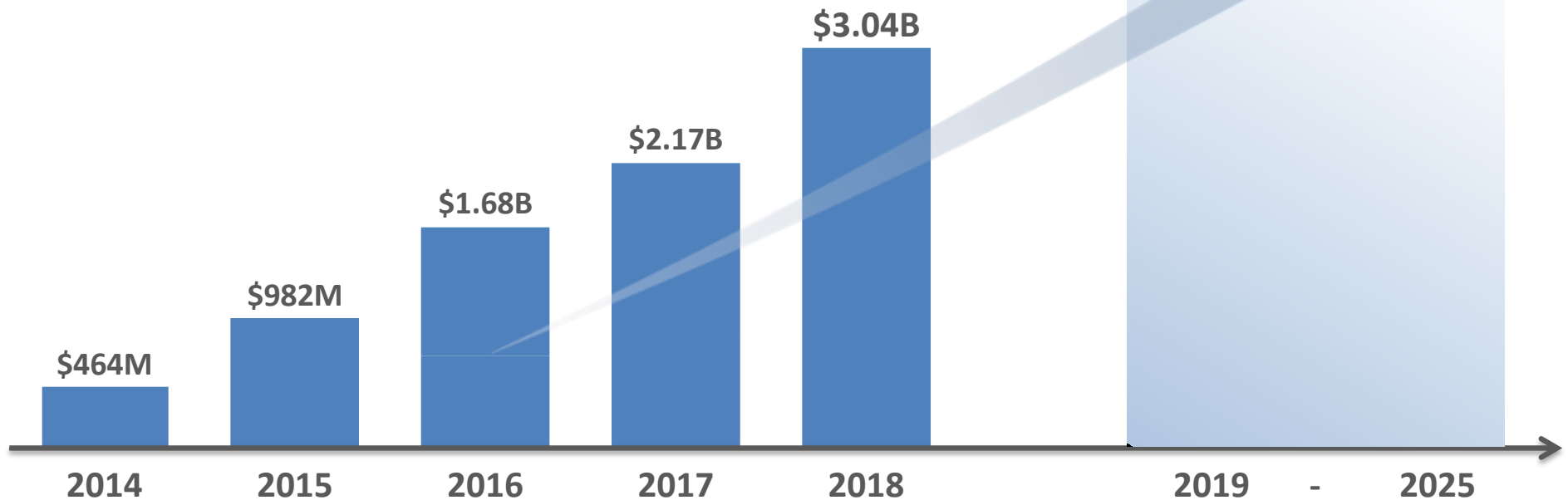
Novel approach to underlying biology of severe kidney disease; Potential clinical candidate in 2019

# CF Revenue Growth to Continue in 2019 and Beyond

Of ~**39,000** patients currently eligible for an existing VRTX CF medicine, ~**18,000** are currently on treatment

The increase from ~**39,000** to ~**44,000** patients will be based on treating younger patients and label expansions with our current medicines

With a triple combination regimen, VRTX has the potential to increase treatment from ~**44,000** to ~**68,000** patients worldwide



**GLOBAL CF PRODUCT REVENUES**



Note: 2019 - 2025 potential growth in CF revenues is provided as a graphical representation.



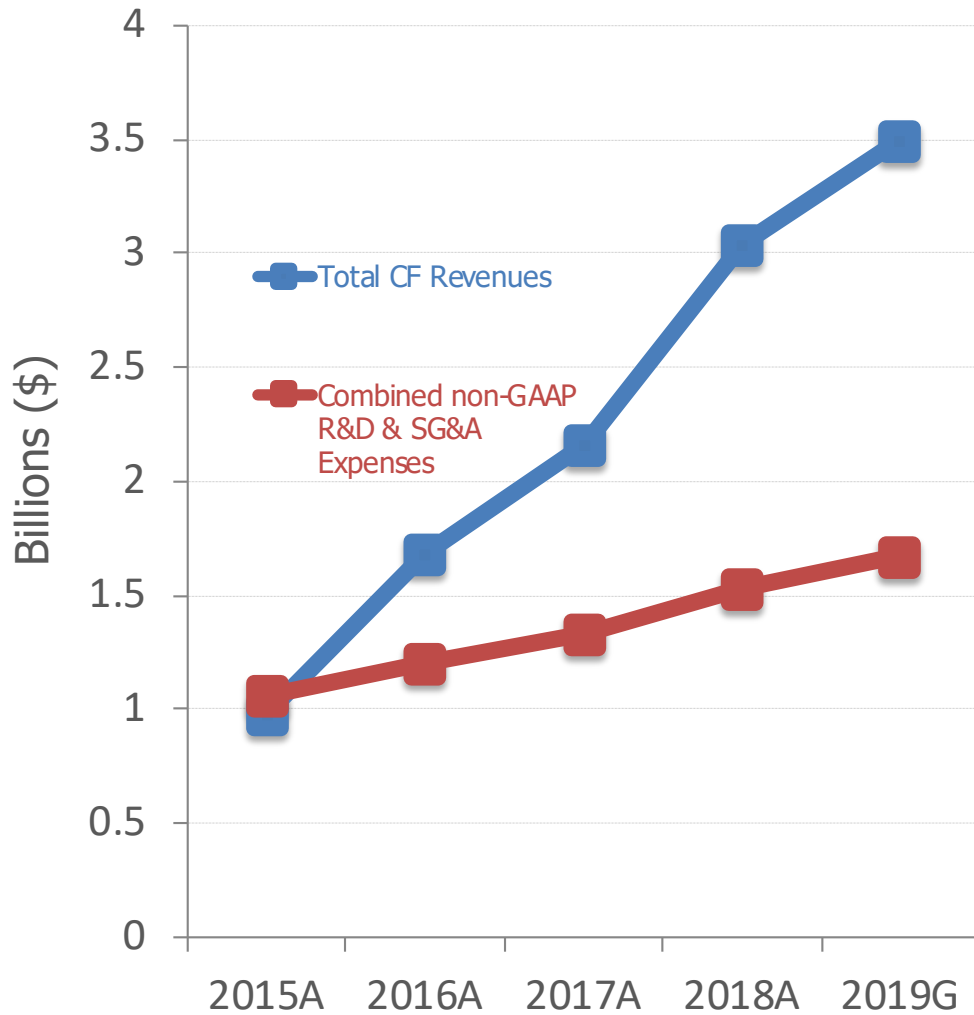
# Full-Year and Q4 2018 Financial Highlights

	FY 2017	FY 2018	Q1 18	Q2 18	Q3 18	Q4 18
<i>(\$ in millions except per share data and percentages)</i>						
Total CF product revenues	<u>\$2.17B</u>	<b><u>\$3.04B</u></b>	<u>\$638</u>	<u>\$750</u>	<u>\$783</u>	<b><u>\$868</u></b>
KALYDECO	845	<b>1.01B</b>	250	253	246	<b>259</b>
ORKAMBI	1.32B	<b>1.26B</b>	354	311	282	<b>315</b>
SYMDEKO		<b>769</b>	34	186	255	<b>294</b>
Combined non-GAAP R&D and SG&A	<u>1.33B</u>	<b><u>1.53B</u></b>	<u>360</u>	<u>388</u>	<u>379</u>	<b><u>400</u></b>
Non-GAAP operating income	564	<b>1.11B</b>	208	260	295	<b>348</b>
Non-GAAP operating margin	26%	<b>37%</b>	33%	35%	38%	<b>40%</b>
Non-GAAP net income	495	<b>1.06B</b>	196	244	282	<b>337</b>
Non-GAAP net income per share - diluted	\$1.95	<b>\$4.08</b>	\$0.76	\$0.94	\$1.09	<b>\$1.30</b>
Cash, cash equivalents & marketable securities (year-end)	\$2.1B	<b>\$3.2B</b>				

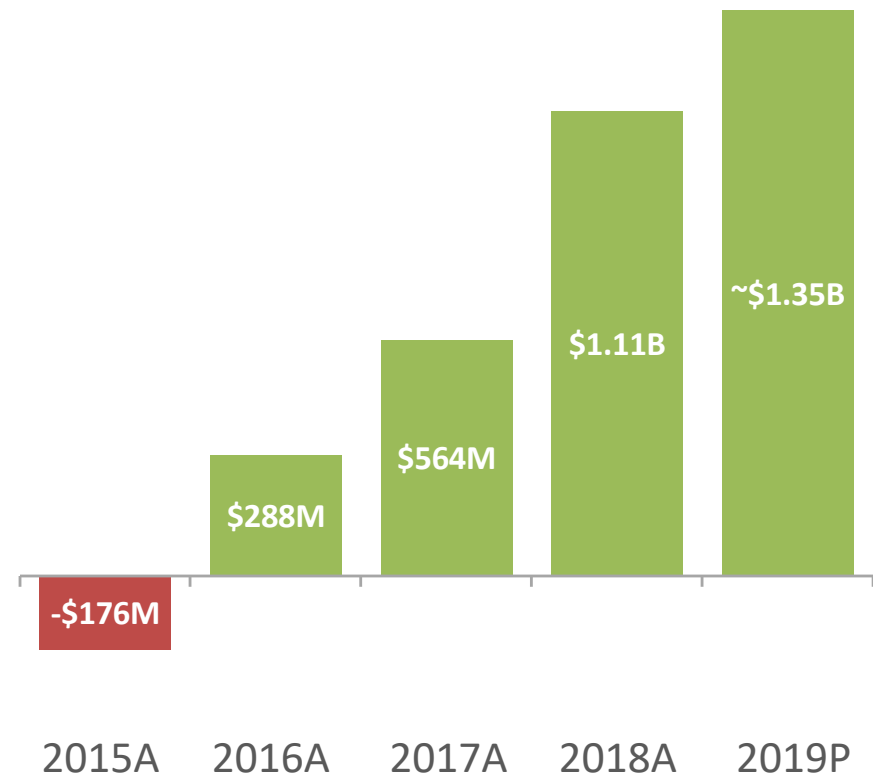
## Notes

- An explanation of non-GAAP financial measures and reconciliation of combined non-GAAP R&D and SG&A expense, non-GAAP net income and non-GAAP net income per share is included in the company's Q4 2018 press release dated February 5, 2019
- Reconciliation of non-GAAP operating income and non-GAAP operating margin to corresponding GAAP measures is included in the appendix of this presentation

# Significant Growth in Revenue Driving Operating Income Expansion



## Non-GAAP Operating Income



**Notes**

- 2019 Total CF Revenues (guidance: \$3.45– \$3.55B) and combined non-GAAP R&D and SG&A expenses (guidance: \$1.65 - \$1.70B) graphed to reflect the midpoint of guidance ranges.
- 2019 projected Operating Income based on the midpoint of the guidance ranges and assumes a 13.5% cost of sales; not intended as financial guidance.



# 2019 Financial Guidance

	FY 2018 Actuals	FY 2019 Guidance	FY 2019 Guidance Commentary
Total CF Product Revenues	\$3.04B	<b>\$3.45 - \$3.55B</b>	Revenue guidance primarily based on the full year impact of: <ul style="list-style-type: none"> <li>• SYMDEKO and SYMKEVI launch</li> <li>• Label expansions for approved CF medicines</li> <li>• Reimbursement agreements achieved in 2018</li> </ul>
Combined Non-GAAP R&D and SG&A	\$1.53B	<b>\$1.65 - \$1.70B</b>	Year-over-year increase based on: <ul style="list-style-type: none"> <li>• CF development efforts, incremental investments to support potential launch of a triple combination regimen and investment to support expansion of pipeline into additional disease areas</li> </ul>
Combined GAAP R&D and SG&A	\$1.97B	<b>\$2.0 - \$2.15B</b>	
GAAP and Non-GAAP Effective Tax Rate		<b>21% - 22%</b>	<ul style="list-style-type: none"> <li>• The vast majority of the company's tax provision will be a non-cash expense until NOLs are fully utilized</li> </ul>

*Note: An explanation of non-GAAP financial measures and reconciliation of non-GAAP combined R&D and SG&A expense is included in the company's Q4 2018 press release dated February 5, 2019*



# Full-Year and Fourth-Quarter 2018 Financial Results



February 5, 2019

# Appendix

## Reconciliation of GAAP to non-GAAP Financial Information

	Q4 2017	FY 2017	Q1 2018	Q2 2018	Q3 2018	Q4 2018	FY 2018
<b>GAAP total revenues</b>	\$652	\$2,489	\$641	\$752	\$785	<b>\$870</b>	<b>\$3,048</b>
<b>Non-GAAP total revenues</b>	\$623	\$2,174	\$639	\$751	\$784	<b>\$869</b>	<b>\$3,043</b>
<b>GAAP operating income</b>	126	123	129	173	206	<b>128</b>	<b>635</b>
Stock compensation expense	75	291	78	82	86	<b>79</b>	<b>325</b>
Collaborative and transaction revenues and expenses	(18)	133	1	4	2	<b>141</b>	<b>147</b>
Other adjustments	1	17	0	2	1	<b>1</b>	<b>5</b>
<b>Non-GAAP operating income</b>	184	564	208	260	295	<b>348</b>	<b>1.11B</b>
<b>Operating Margin %:</b>							
GAAP	19%	5%	20%	23%	26%	<b>14%</b>	<b>21%</b>
Non-GAAP	30%	26%	33%	35%	38%	<b>40%</b>	<b>37%</b>
<b>Net income</b>							
GAAP	101	263	210	207	129	<b>1.55B</b>	<b>2.10B</b>
Non-GAAP	158	495	196	244	282	<b>337</b>	<b>1.06B</b>
<b>Net income per share - diluted</b>							
GAAP	\$0.39	\$1.04	\$0.81	\$0.80	\$0.50	<b>\$5.97</b>	<b>\$8.09</b>
Non-GAAP	\$0.61	\$1.95	\$0.76	\$0.94	\$1.09	<b>\$1.30</b>	<b>\$4.08</b>



*Note: All numbers in the above reconciliation table are in millions except per share data and percentages. Totals may not add due to rounding.*