



Second-Quarter 2017 Financial Results

July 26, 2017

Agenda

Introduction

Michael Partridge, VP Investor Relations

Business Update

Jeff Leiden, M.D., Ph.D., Chairman, President and CEO

Second-Quarter Financial Results and 2017 Financial Guidance

Ian Smith, Executive Vice President, COO and CFO

Q&A

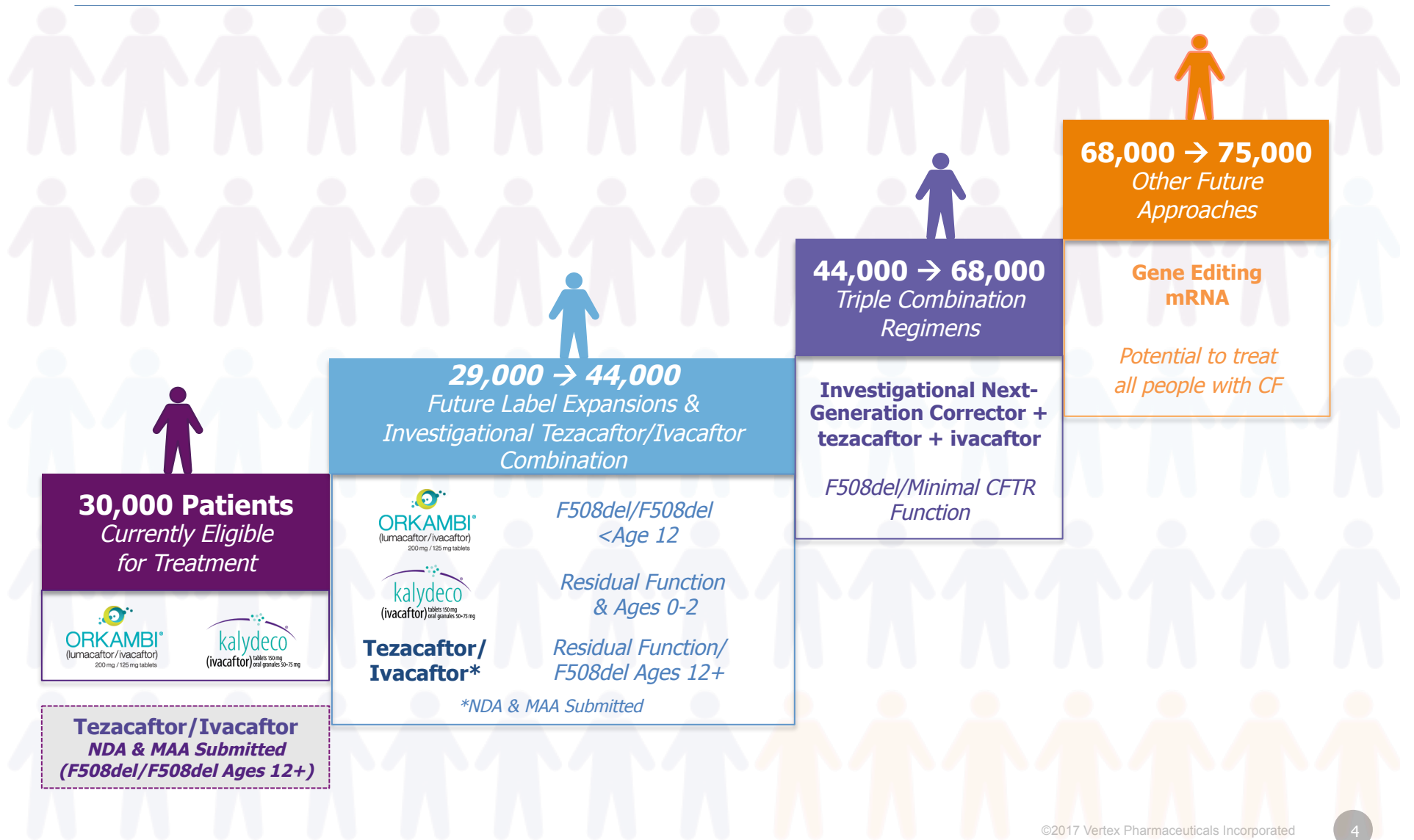
Stuart Arbuckle, Executive Vice President, Chief Commercial Officer

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 KALYDECO and ORKAMBI and the ongoing discovery, development and commercialization of Vertex's product candidates, and (ii) information regarding the Company's financial guidance for 2017. 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, that the Company's expectations regarding its 2017 revenues and expenses may be incorrect (including because one or more of the Company's assumptions underlying its expectations may not be realized), 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, and the risks and uncertainties listed in the Company's July 26, 2017 press release and 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 (i) stock-based compensation expense, (ii) revenues and expenses related to business development transactions including collaboration agreements and consolidated variable interest entities and (iii) other adjustments. 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 business development activities, but includes \$160.0 million in R&D expense related to the upfront payment that Vertex expects to incur in the third quarter of 2017 for the rights to CTP-656 and other assets acquired from Concert Pharmaceuticals. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the Company's July 26, 2017 press release.

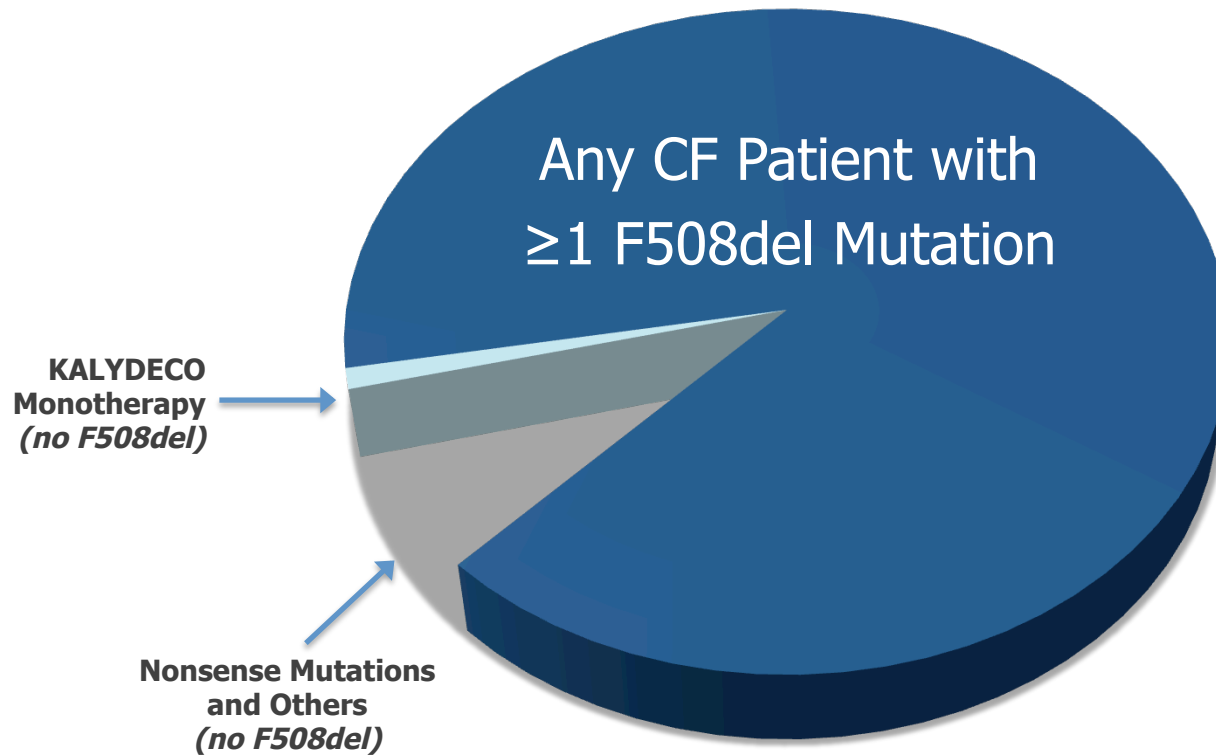
Path to Treating All Patients



1H 2017 Accomplishments

- Received **FDA approval of KALYDECO** for people with CF ages 2 and older with one of 23 residual function mutations
- Obtained **reimbursement for ORKAMBI** in Ireland and Italy for use in people with CF ages 12 and older who have two copies of *F508del* mutation
- Submitted an **NDA and MAA for tezacaftor/ivacaftor** combination for people with CF ages 12 and older who have two copies of *F508del* mutation
- **Positive Phase 2 proof-of-concept data for VX-440 and VX-152** triple combination regimens in people with CF who have at least one *F508del* mutation
- **Positive Phase 1 data for VX-659** triple combination regimen for people with CF who have at least one *F508del* mutation
- Obtained **worldwide development and commercialization rights to CTP-656** and other assets related to treatment of CF

Potential to Treat 90% of CF Patients with Next Generation Triple Combination Regimens



GOAL: Create Medicines that Fundamentally Alter the Progression of CF for All Patients

Q2 2017 Financial Results

	Q2 2017	Q1 2017	Q2 2016
ORKAMBI Revenues	\$324M	\$295M	\$245M
KALYDECO Revenues	\$190M	\$186M	\$180M
Total CF Revenues	\$514M	\$481M	\$426M
Non-GAAP Combined R&D and SG&A Expense	\$333M	\$313M	\$306M
GAAP Net Income (Loss)	\$18M	\$248M	\$(65)M
Non-GAAP Net Income	\$99M	\$101M	\$58M
GAAP Net Income (Loss) Per Diluted Share	\$0.07	\$0.99	\$(0.26)
Non-GAAP Net Income Per Diluted Share	\$0.39	\$0.41	\$0.24
Cash, cash equivalents & marketable securities (quarter-end)	\$1.67B	\$1.41B	\$1.07B

An explanation of our Non-GAAP financial measures and reconciliation of our Non-GAAP combined R&D and SG&A expense is included in our July 26, 2017 press release.

2017 Financial Guidance

	2017 Guidance	Guidance Commentary
ORKAMBI Revenues	\$1.1 - \$1.3B	<ul style="list-style-type: none"> Ongoing performance in currently reimbursed markets 2H17 growth driven by reimbursement agreements in Europe <ul style="list-style-type: none"> France would be the largest contributor if reimbursement is secured in 2017
KALYDECO Revenues	\$740 - \$770M	<ul style="list-style-type: none"> Recent approval in patients with residual function mutations
Total CF Revenues	\$1.84 - \$2.07B	
Non-GAAP Combined R&D and SG&A Expense	\$1.33 - \$1.36B	<ul style="list-style-type: none"> Expected growth in future quarters in 2017 based on: <ul style="list-style-type: none"> Progression of CF portfolio Preparation and acceleration of triple-combination studies Investment in development of CTP-656
GAAP Combined R&D and SG&A Expense	\$1.79 - \$1.92B	<ul style="list-style-type: none"> As above \$160M R&D expense associated with acquisition of CTP-656

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