



Verona Pharma



Breathtaking science

Developing respiratory drugs

to improve health and quality of life

H.C. Wainwright
Global Life Sciences Conference
April 2018

www.veronapharma.com



Forward-Looking Statements

This presentation contains “forward-looking” statements that are based on the beliefs and assumptions and on information currently available to management of Verona Pharma plc (together with its consolidated subsidiaries, the “Company”). All statements other than statements of historical fact contained in this presentation are forward-looking statements. Forward-looking statements include information concerning the initiation, timing, progress and results of clinical trials of the Company’s product candidate, the timing or likelihood of regulatory filings and approvals for any of its product candidates, and estimates regarding the Company’s expenses, future revenues and future capital requirements. In some cases, you can identify forward-looking statements by terminology such as “may,” “will,” “should,” “expects,” “plans,” “anticipates,” “believes,” “estimates,” “predicts,” “potential” or “continue” or the negative of these terms or other comparable terminology.

Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause the Company’s actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. These risks, uncertainties and other factors include those under “Risk Factors” in our annual report on Form 20-F filed with the Securities and Exchange Commission (the “SEC”) on February 27, 2018, and in its other reports filed with the SEC. Forward-looking statements represent the Company’s beliefs and assumptions only as of the date of this presentation. Although the Company believes that the expectations reflected in the forward-looking statements are reasonable, it cannot guarantee future results, levels of activity, performance or achievements. Except as required by law, the Company assumes no obligation to publicly update any forward-looking statements for any reason after the date of this presentation, or to conform any of the forward-looking statements to actual results or to changes in its expectations.



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Clinical-stage biopharma focused on developing & commercializing **innovative therapeutics** for treatment of **respiratory diseases** with significant **unmet need**

Inhaled dual inhibitor of
enzymes PDE₃ and PDE₄

RPL554

Current Focus:
COPD and CF

Potential first novel class of bronchodilator in decades
Bronchodilator + anti-inflammatory agent in single compound

COPD: A Substantial Unmet Need For New Maintenance and Acute Treatments



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Maintenance (Home)



24M living with COPD in U.S.

- 15M diagnosed and under treatment
- Approximately 2M severe/very severe

Treatment goals:

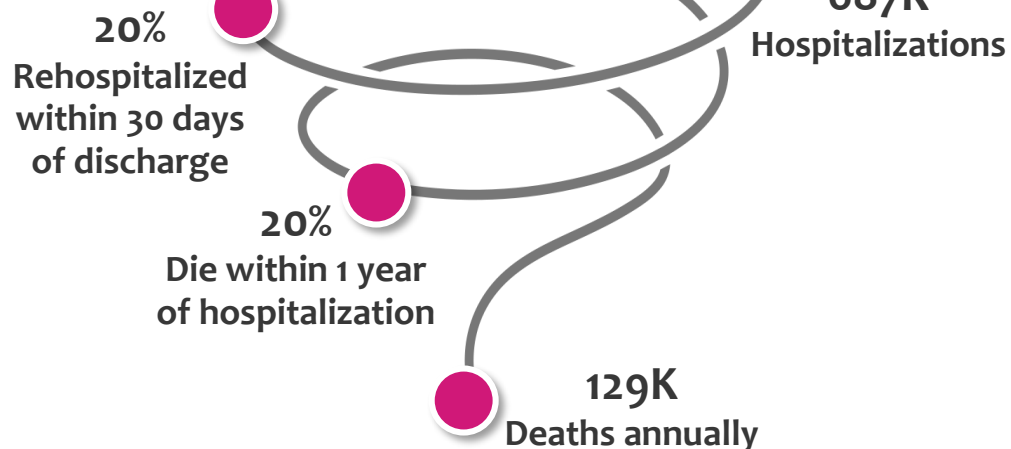
- Symptom control and relief
- Improved quality of life
- Improved lung function
- Prevent exacerbations

Note: U.S. only data

Acute (Hospital)



1.5M
ER visits



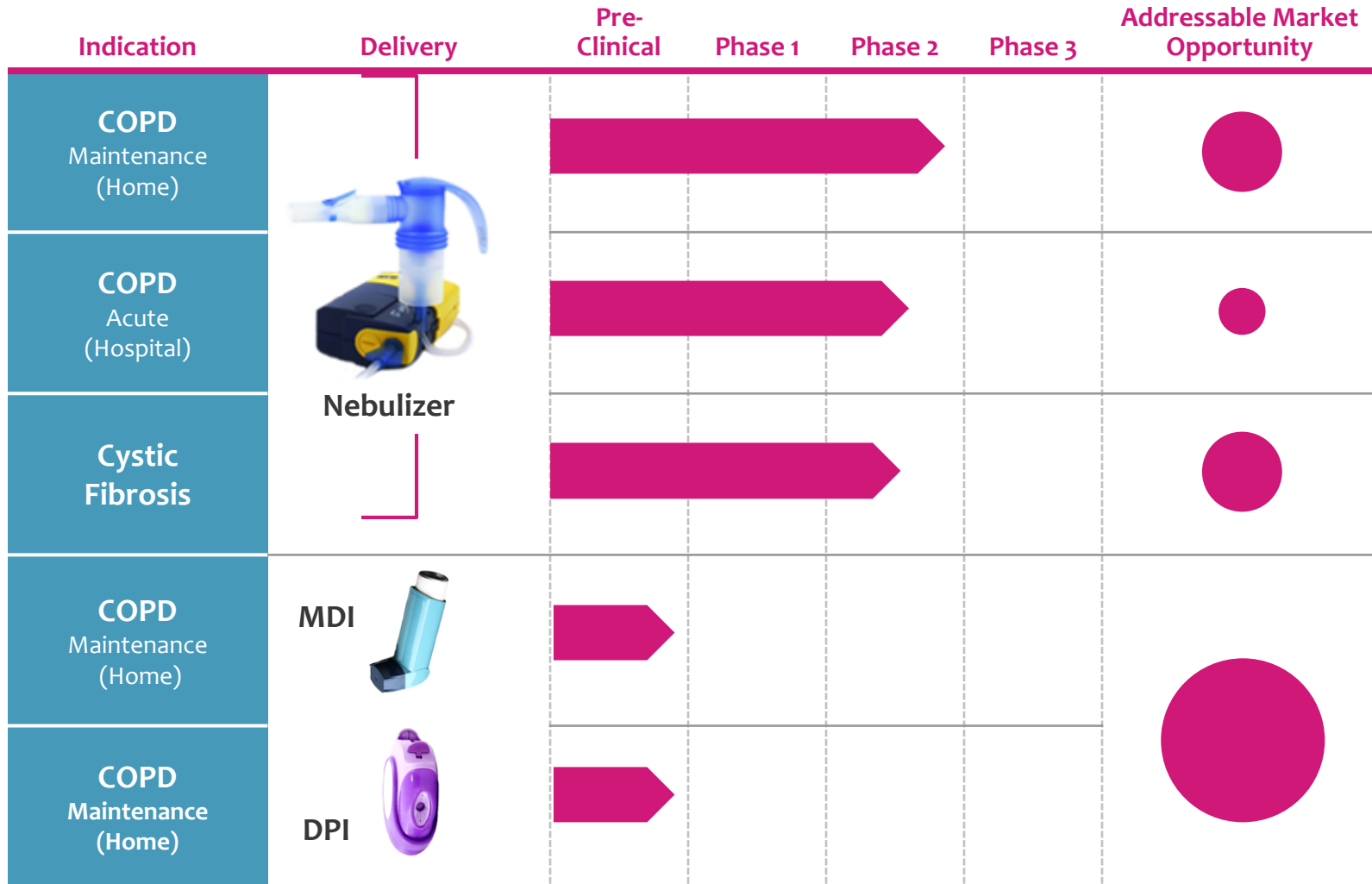
Number of COPD patients and hospitalizations remains high

RPL554: Rich Product Pipeline

12 completed Phase 1 and 2 clinical trials enrolling >730 subjects



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RPL554 also has applications in other significant respiratory diseases such as asthma.

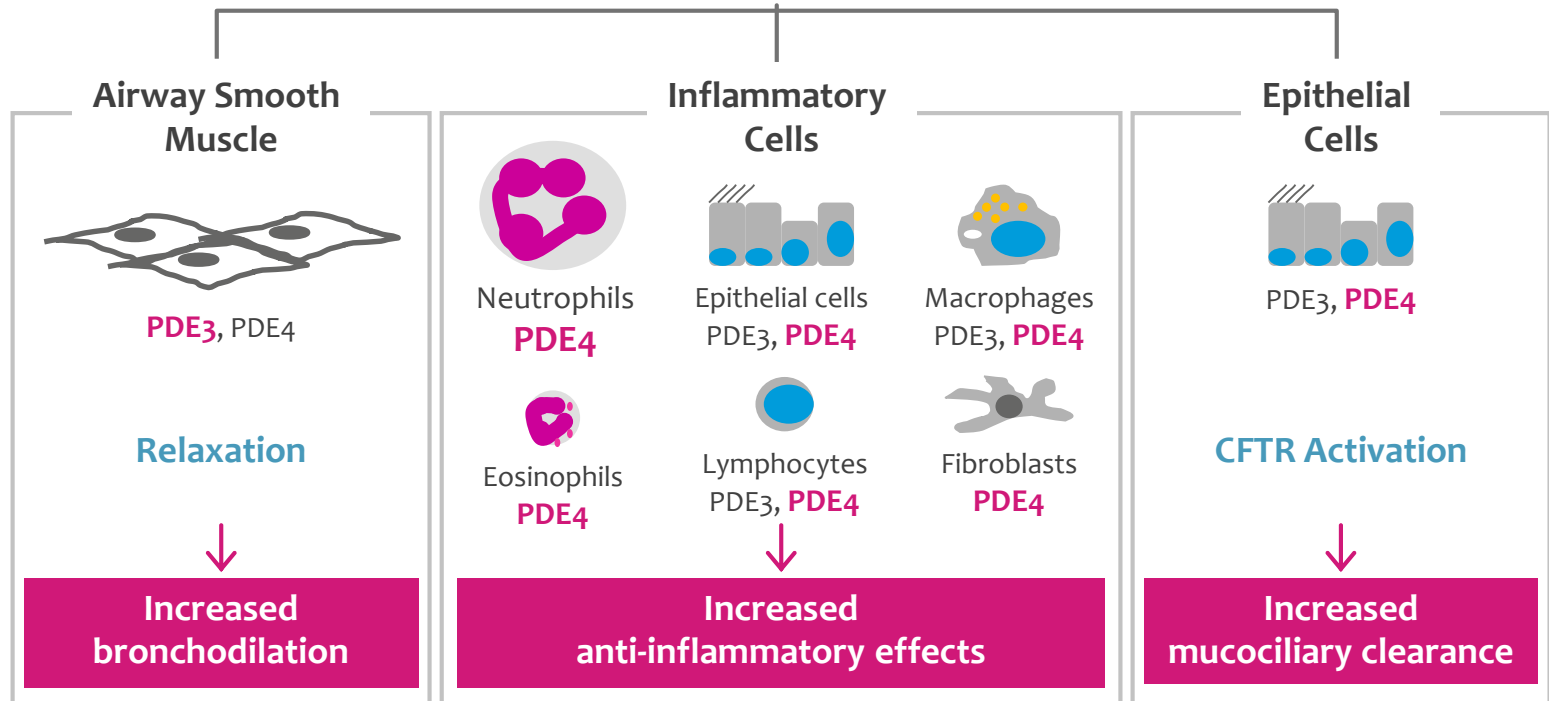
RPL554 First-in-Class Candidate: Bronchodilator and Anti-inflammatory in a Single Compound



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RPL554
Dual PDE3 and PDE4 enzyme inhibitor

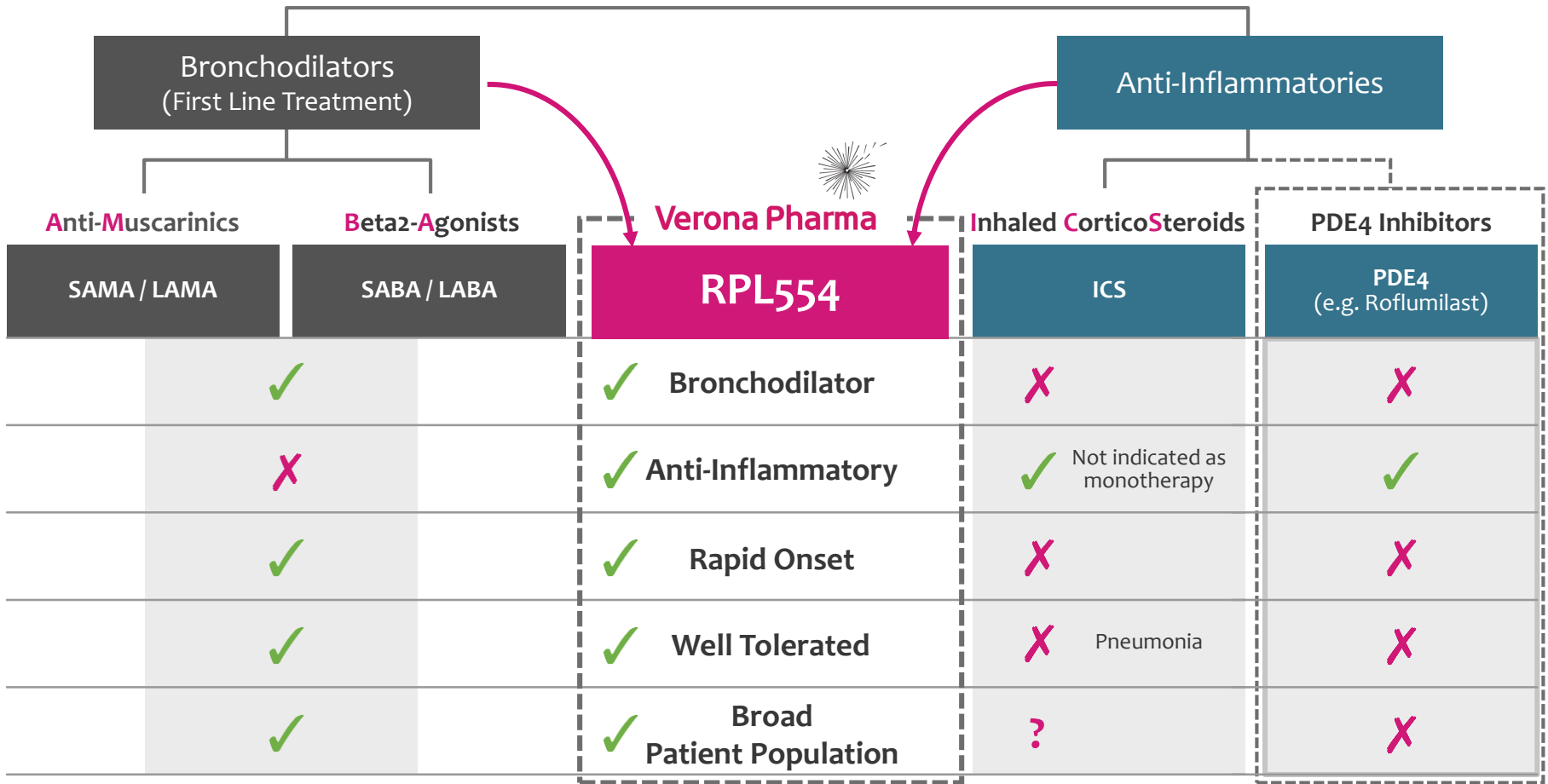
Impacts 3 Key Mechanisms in Respiratory Disease:



RPL554: Potential to Address Limitations of Current Therapies



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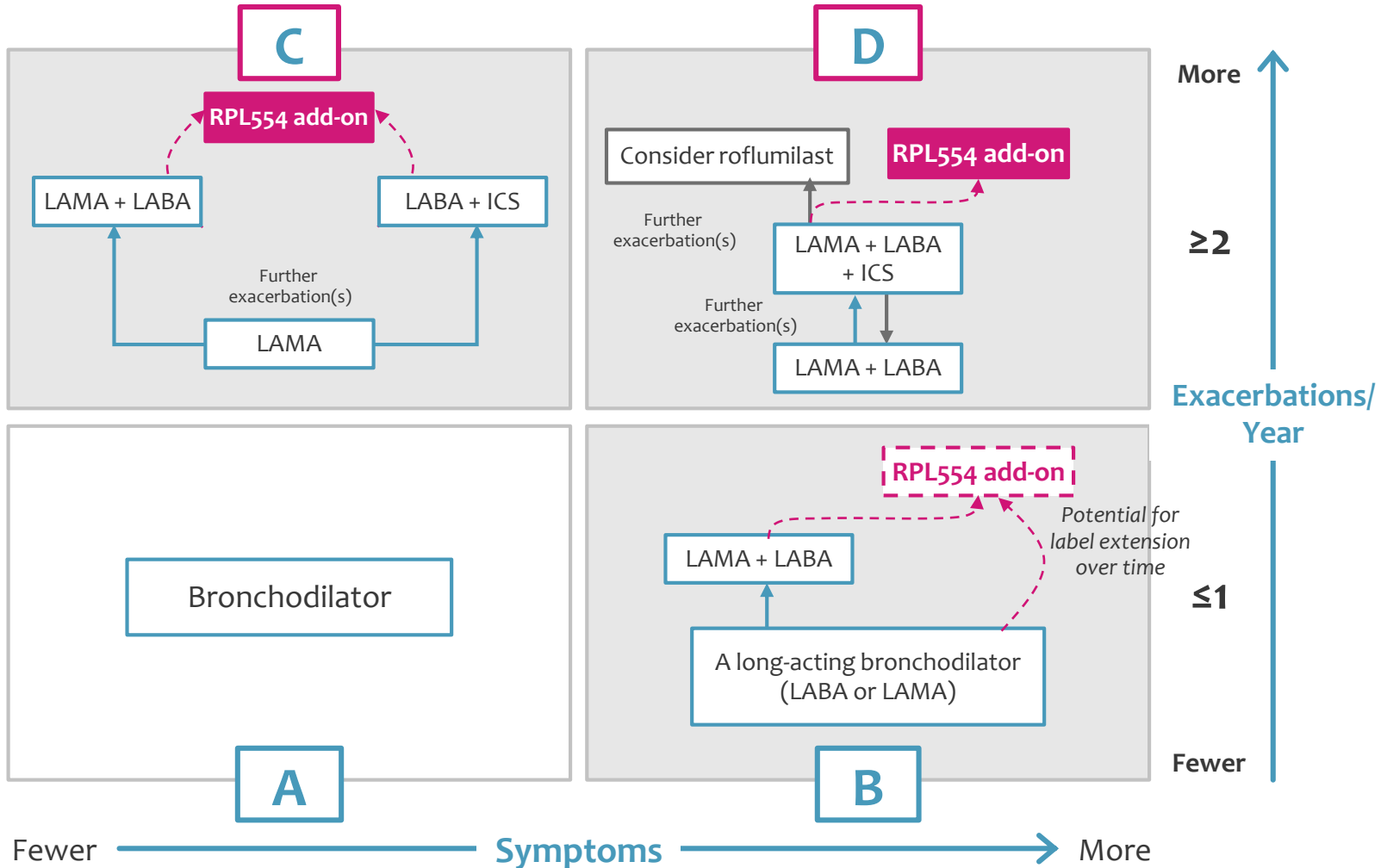
Eventually patients will have “exhausted” all available drug therapies

RPL554: Potential to Improve Standard of Care Treatment for More Severe Patients



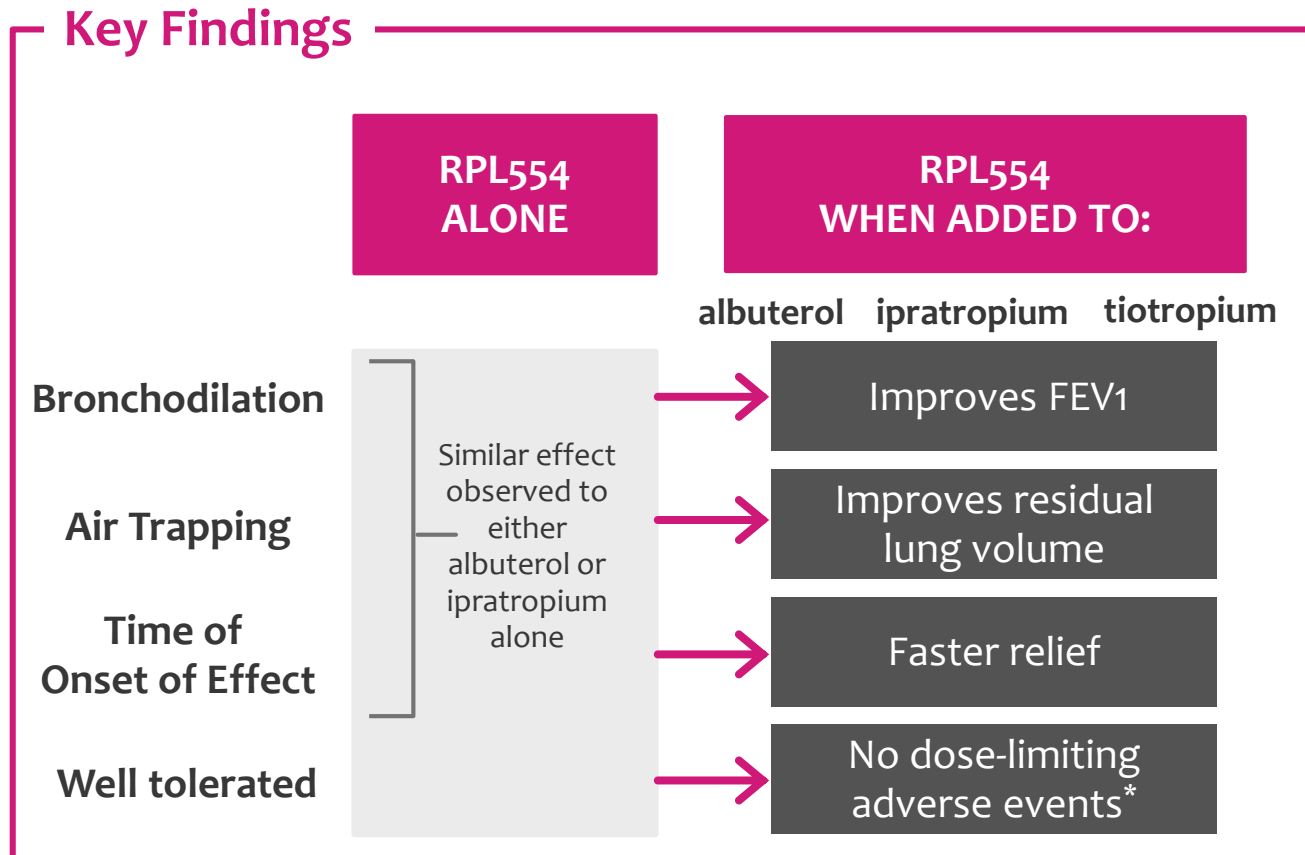
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Based on GOLD COPD Guidelines 2017





RPL554: Significantly De-Risked Add-on Effect Reproduced in Independent Study



*in completed clinical trials
Source: RPL554-009-2015; RPL554-CO-202



Four Week Phase 2b Study: Moderate to Severe COPD Top-Line Data Released March 26, 2018

Trial Description:

- Phase 2b randomized, double blind, placebo controlled, dose ranging study
- Assess nebulized RPL554 in patients with moderate to severe COPD
- Outpatient setting
- No background bronchodilator therapy (stable ICS regimen can be maintained)

Patient Population:

- 403 moderate-to-severe COPD patients, diagnosed >12 months previously
- Males and females, age 40-75

Location:

- Approximately 45 centers in Western & Eastern Europe

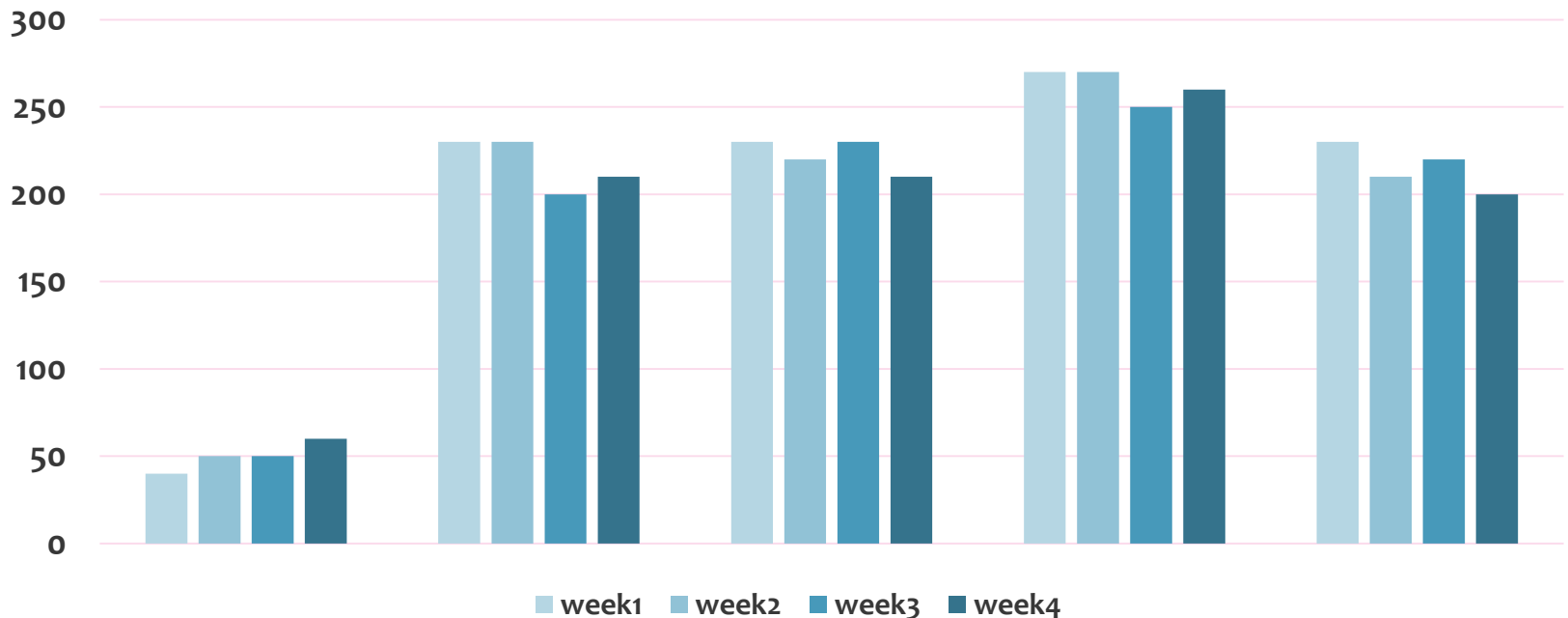
RPL554 Dosage:

- Five arms, twice daily dosing with RPL554 at 0.75 mg, 1.5 mg, 3 mg, 6 mg or placebo

Significant, Clinically Meaningful Bronchodilator Response Maintained over Four Weeks

Peak Change from Day 1 in Baseline in FEV₁ (mL) on week 4 (p<0.001)

N=403

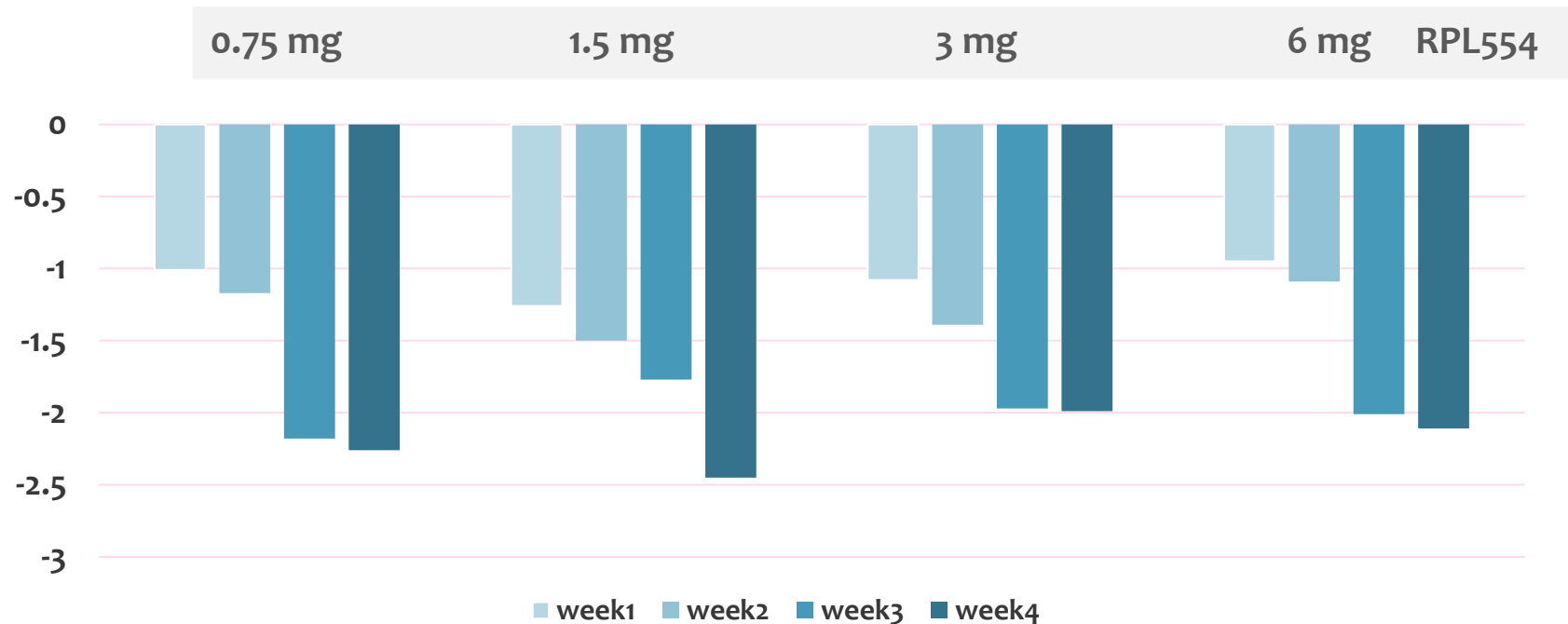


placebo 0.75 mg 1.5 mg 3 mg 6 mg RPL554

Progressive Improvement of COPD Symptoms with all Doses from Weeks 1 to 4

Total score (0-40) E-RS: COPD by week (placebo corrected, $p < 0.02$)

N=403





Effective and Well Tolerated over Four Weeks Treating COPD Patients in Outpatient Setting

Primary endpoint:

- Peak FEV₁; all doses showed statistically significant difference vs. placebo (**p<0.001**)
- Peak bronchodilator effect observed at first dose, sustained over four weeks (**p<0.001**)

Secondary endpoints include:

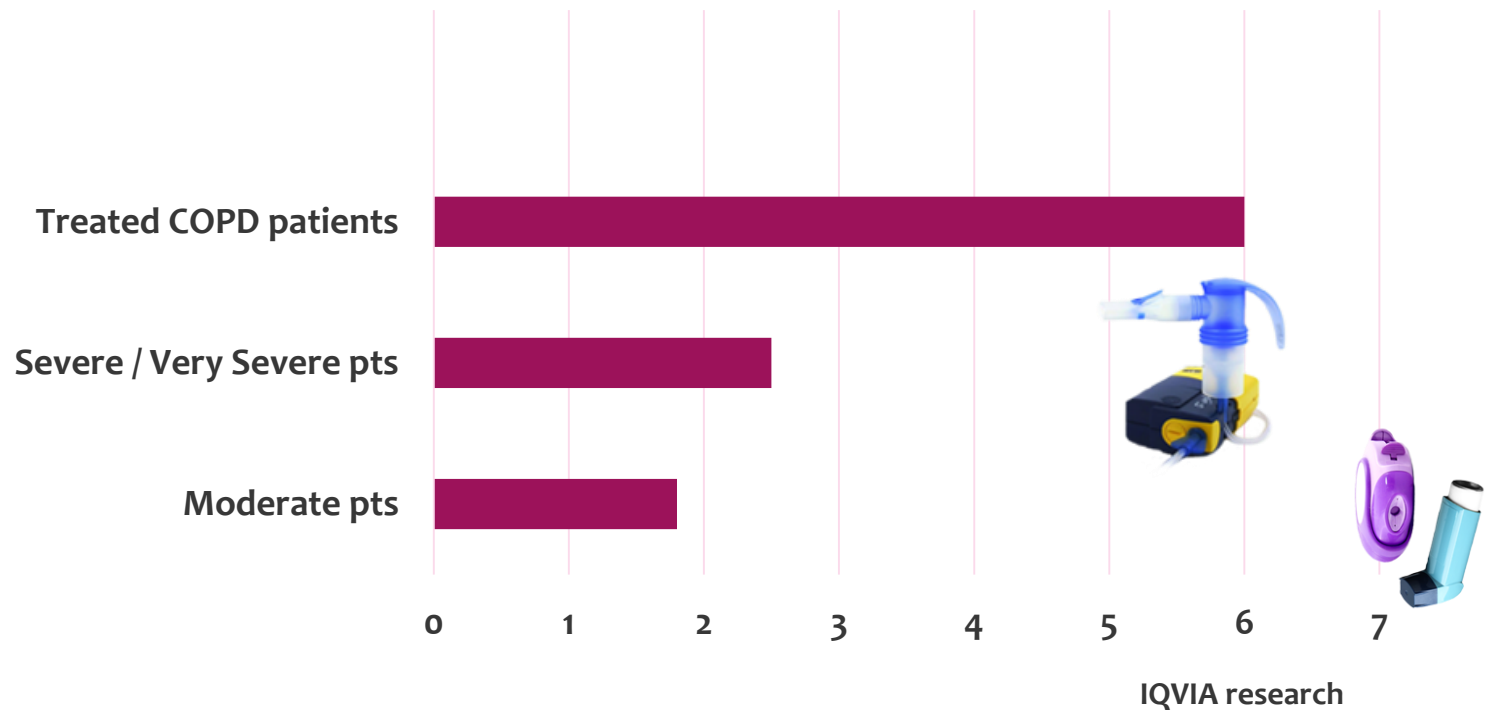
- Statistically significant improvement in average FEV₁ over 12 hours observed at all dose levels at the first dose; sustained over the four weeks of dosing
- Study did not demonstrate consistent improvements in trough FEV₁
- Statistically significant, progressive improvements in daily COPD symptoms, using E-RS (**p<0.02** improvements in all sub domains of EXACT-PRO)
- Well tolerated at all doses; adverse event profile similar to placebo

RPL554: Potential to Improve Standard of Care Treatment for Millions of Patients



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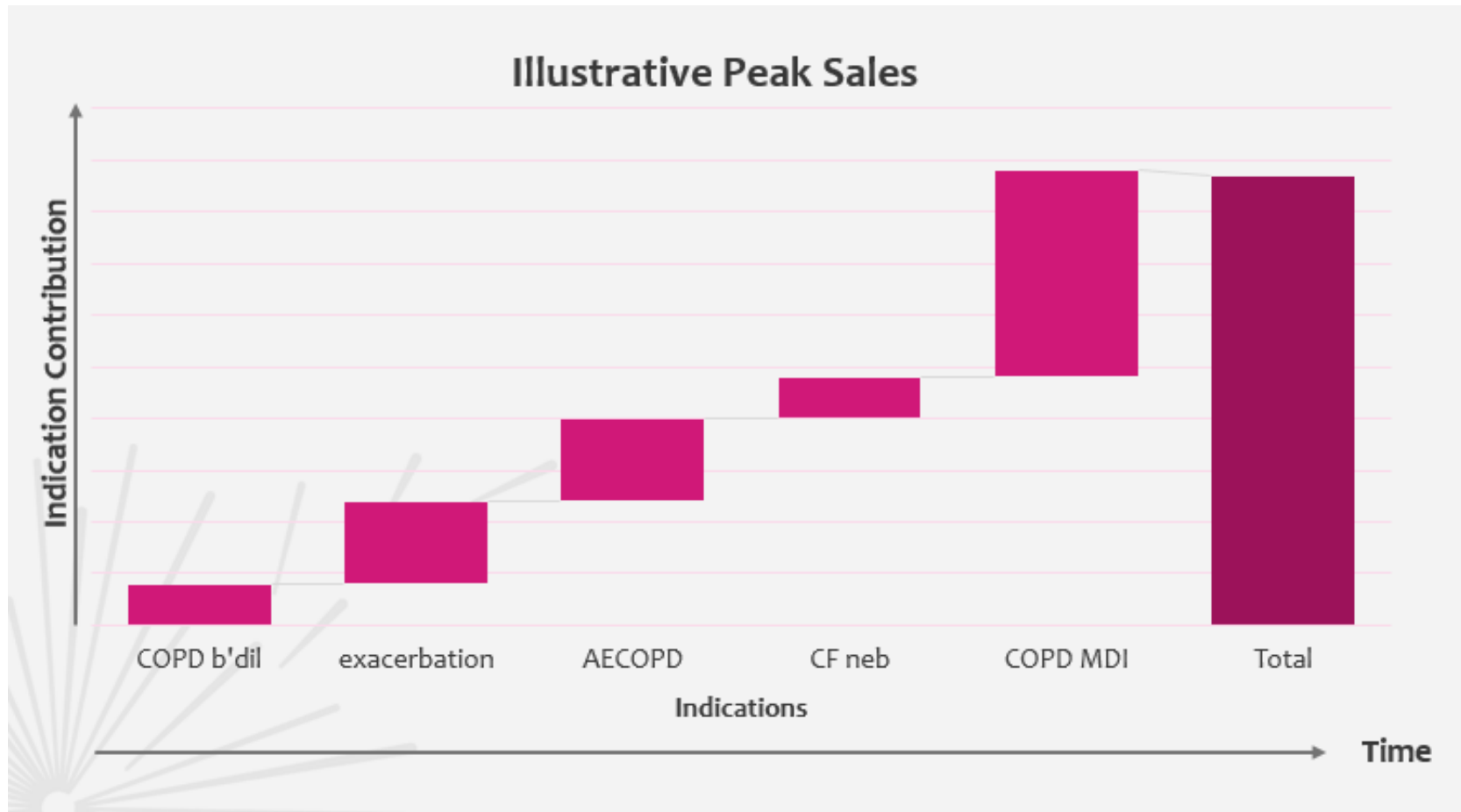
COPD Patients in US (Million)



RPL554: Targeting Multiple Indications Allows Earlier Access to Large Markets



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CF: A Devastating Orphan Disease



- Most common fatal inherited disease in U.S.
- Mutations in gene that encodes CFTR protein
- Inability to clear thickened mucus, impaired lung function and persistent lung infection
- Frequent exacerbations and hospitalization
- No cure
- Median age of death – 37 years
- RPL554 has potential to provide treatment independent of CF mutation status
 - Reduce airway obstruction and inhibit inflammation

Phase 2a study, data reported 2 March 2018

RPL554 Demonstrates Favorable PK and PD Profile in CF Patients



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- Randomized, double blind, cross-over trial comparing 1.5 mg and 6.0 mg doses with RPL554 to placebo in 10 patients with CF
 - Patients displayed a range of CF genotype mutations in the CFTR
-
- Primary endpoint:
 - PK profile was consistent with that observed in patients with COPD, although with lower peak serum levels of RPL554 in CF patients
 - Serum half-life was dose-dependent; 7.5 to 10.1 hours for 1.5 mg and 6 mg, respectively.
 - Secondary endpoints:
 - Statistically significant increase in average FEV₁ in treated patients for 1.5 mg (all $P < 0.01$) and 6 mg (all $P < 0.05$) at 4, 6 and 8 hour time points
 - RPL554 was well-tolerated in this patient group with an adverse event profile consistent with other studies with RPL554

Results support further development in CF



RPL554 IP Summary

Patent Portfolio:

- Composition of Matter – granted US, EU, Japan, other; expires 2020
- Polymorphs – granted US, EU, Japan, other; expires 2031
- Formulations, combinations, salt forms, use, manufacturing: granted and pending in US, EU, and other territories; expires 2031 – 2037
- Additional IP opportunities being explored

Verona Pharma has global rights

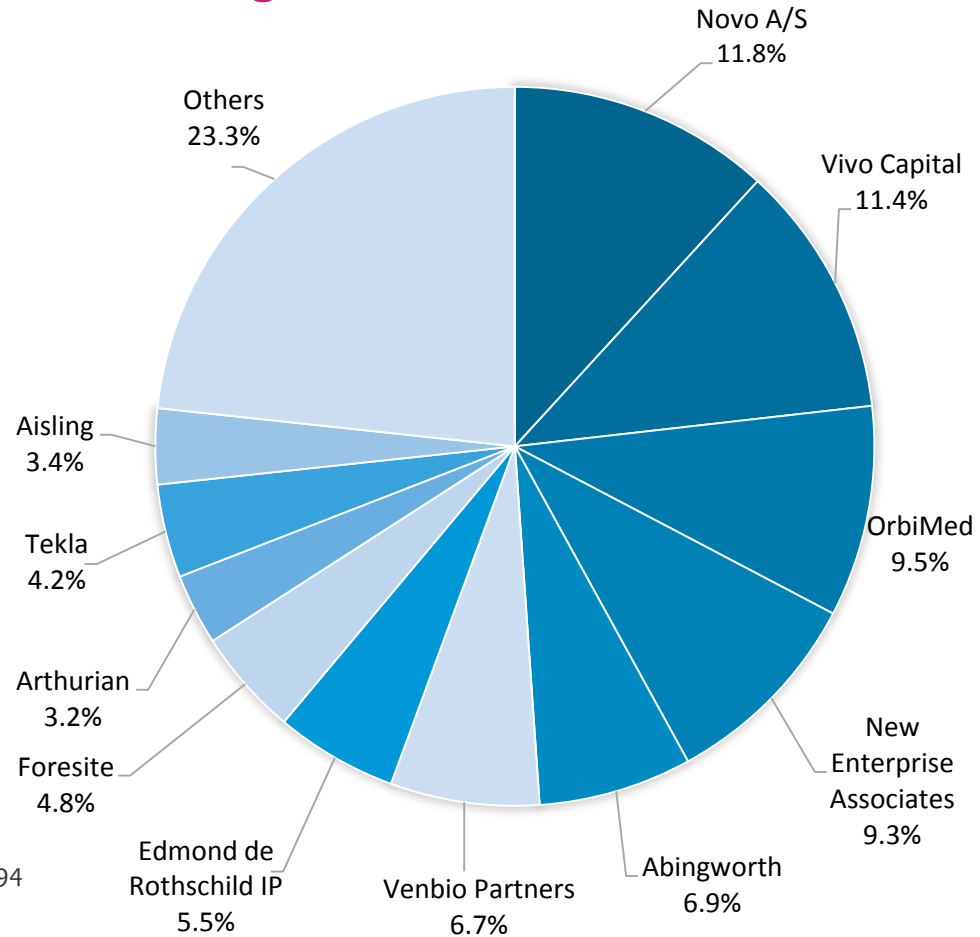


Financial Overview and Shareholder Register

Financial Overview 31 Dec 2017

Cash and Cash Equivalents	\$108.6M ¹
Operating Expenses	\$40.3M ¹
Market cap	\$300M ²

Shareholdings



¹Exchange rate used (US dollars per pound sterling): December 31, 2017 \$1.35294

²Fully diluted 125m shares or 15.6m ADSs, ADS price \$19.07 April 4, 2018



Next Steps – Focused Development in Attractive Market Segments

Key activities leading up to Phase 3 in maintenance treatment in COPD with nebulized formulation of RPL554:

- Positioning study: RPL554 in addition to established combination treatments
- Market research and evaluation of optimal positioning
- “End of Phase 2” meeting to discuss regulatory path
- Pivotal clinical trials in COPD maintenance setting expected to start in 2019

Development of pMDI and DPI formulations of RPL554:

- Expected start of pre-clinical studies 2H 2018

Development as anti-inflammatory treatment in Cystic Fibrosis:

- Following positive Phase 2a results, KOL and regulatory discussion ahead of clinical proof-of-concept study