

# Verona Pharma



## Breathtaking science

Developing respiratory drugs

to improve health and quality of life

JMP Securities Life Science Conference  
New York City, June 2018

[www.veronapharma.com](http://www.veronapharma.com)

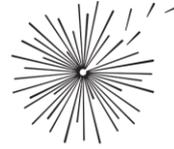


# Forward-Looking Statements

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 the Company’s 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.

This presentation also contains estimates, projections and other information concerning the Company’s business and the markets for the Company’s product candidate, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research, or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, the Company obtained this industry, business, market and other data from reports, research surveys, clinical trials studies and similar data prepared by market research firms and other third parties, from industry, medical and general publications, and from government data and similar sources.



# Verona Pharma



**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: Substantial Unmet Need For New Maintenance and Acute Treatments



Verona Pharma

## 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
- Improve quality of life
- Improve lung function
- Reduce breathlessness
- Prevent exacerbations

Note: U.S. only data

## Acute (Hospital)



1.5M  
ER visits

20%  
Rehospitalized  
within 30 days  
of discharge

20%  
Die within 1 year  
of hospitalization

687K  
Hospitalizations

129K  
Deaths annually

Number of COPD patients and hospitalizations remains high

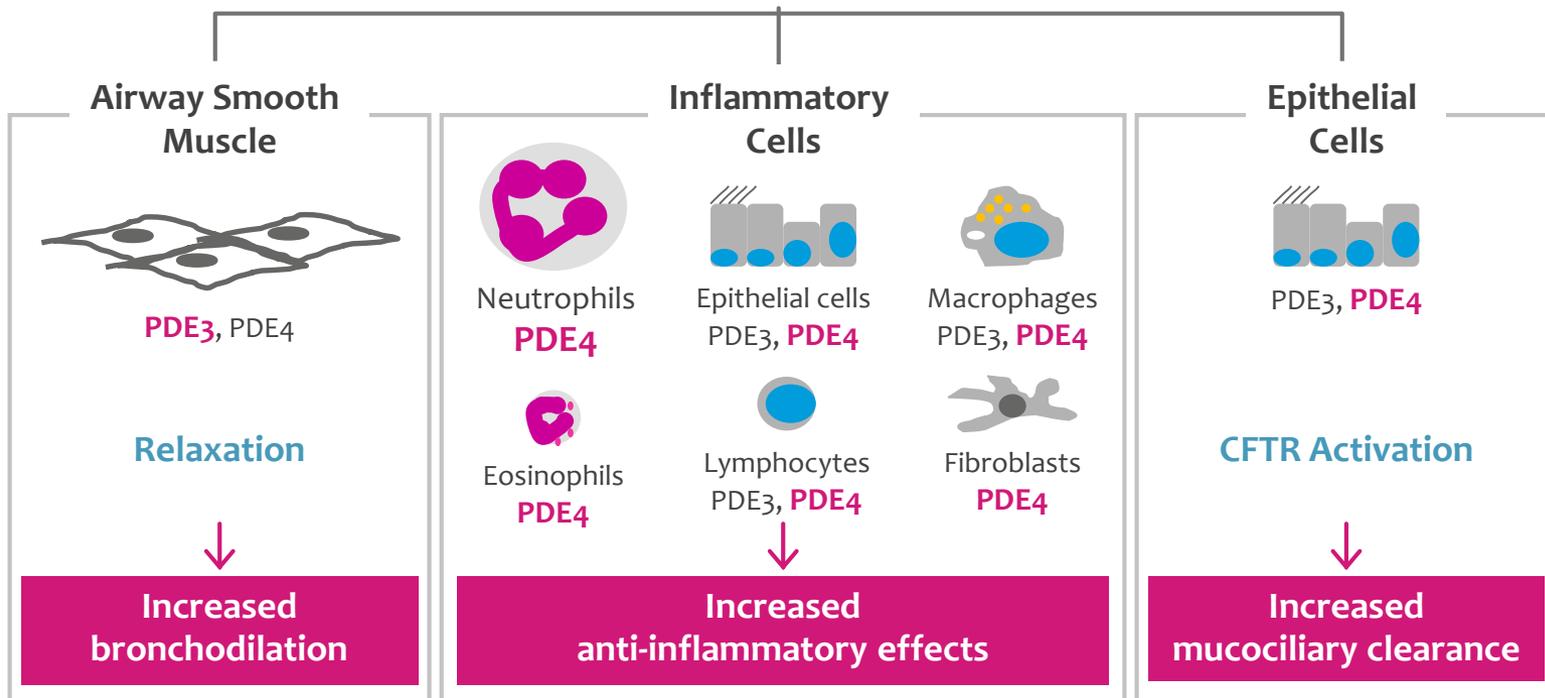
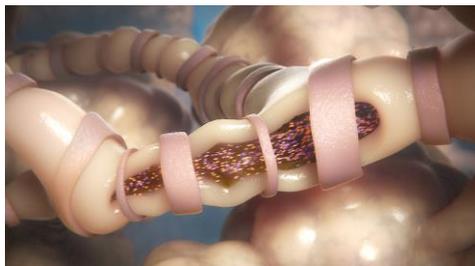
# RPL554 First-in-Class Candidate: Bronchodilator and Anti-inflammatory in a Single Compound



Verona Pharma

**RPL554**  
Dual PDE3 and PDE4 enzyme inhibitor

Impacts 3 Key Mechanisms in Respiratory Disease:

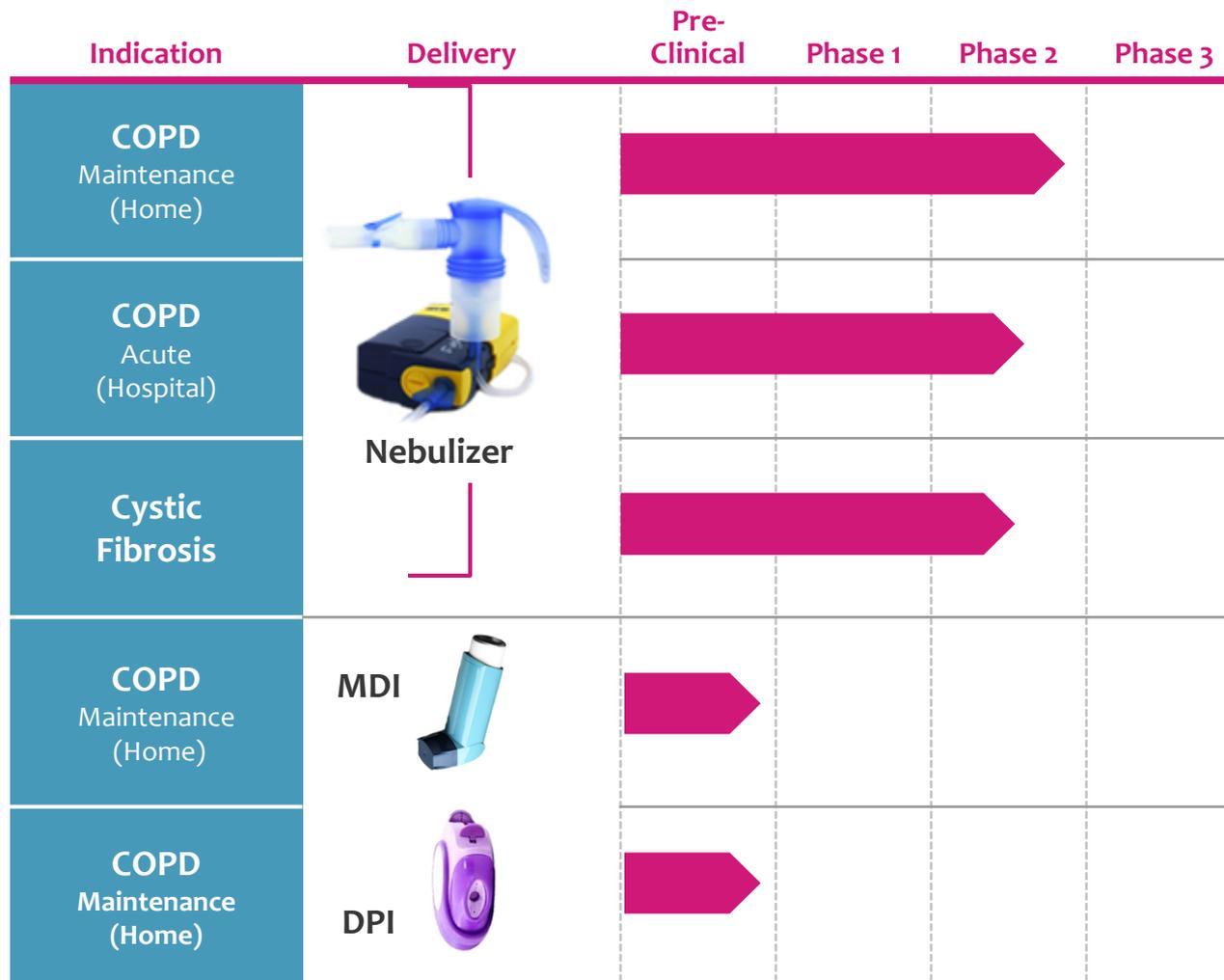


# RPL554: Robust Product Pipeline

Completed twelve Phase 1 and 2 clinical trials and enrolled >730 subjects

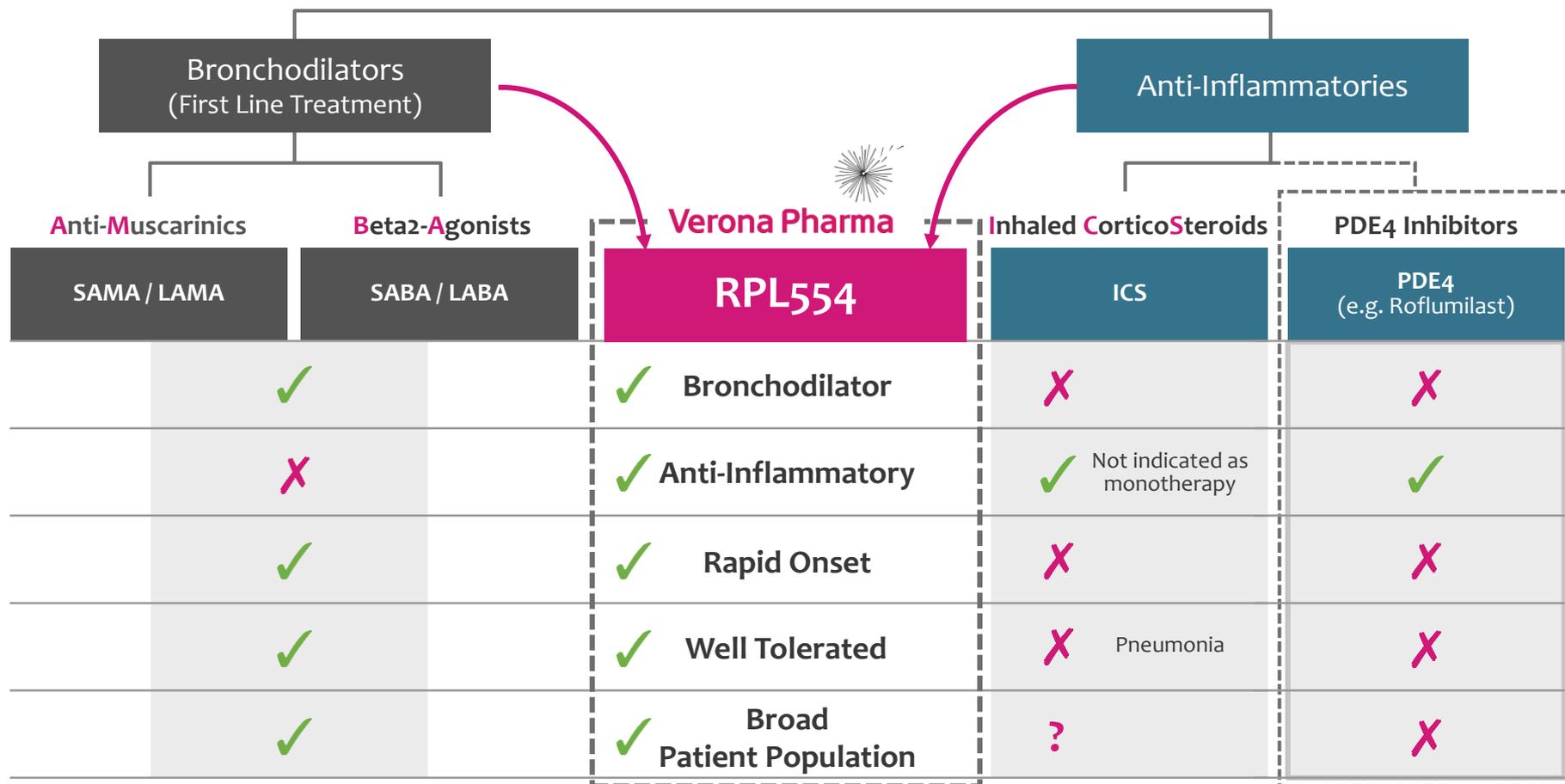


Verona Pharma



RPL554 has potential application in other significant respiratory diseases such as asthma.

# RPL554: Potential to Address Limitations of Current Therapies

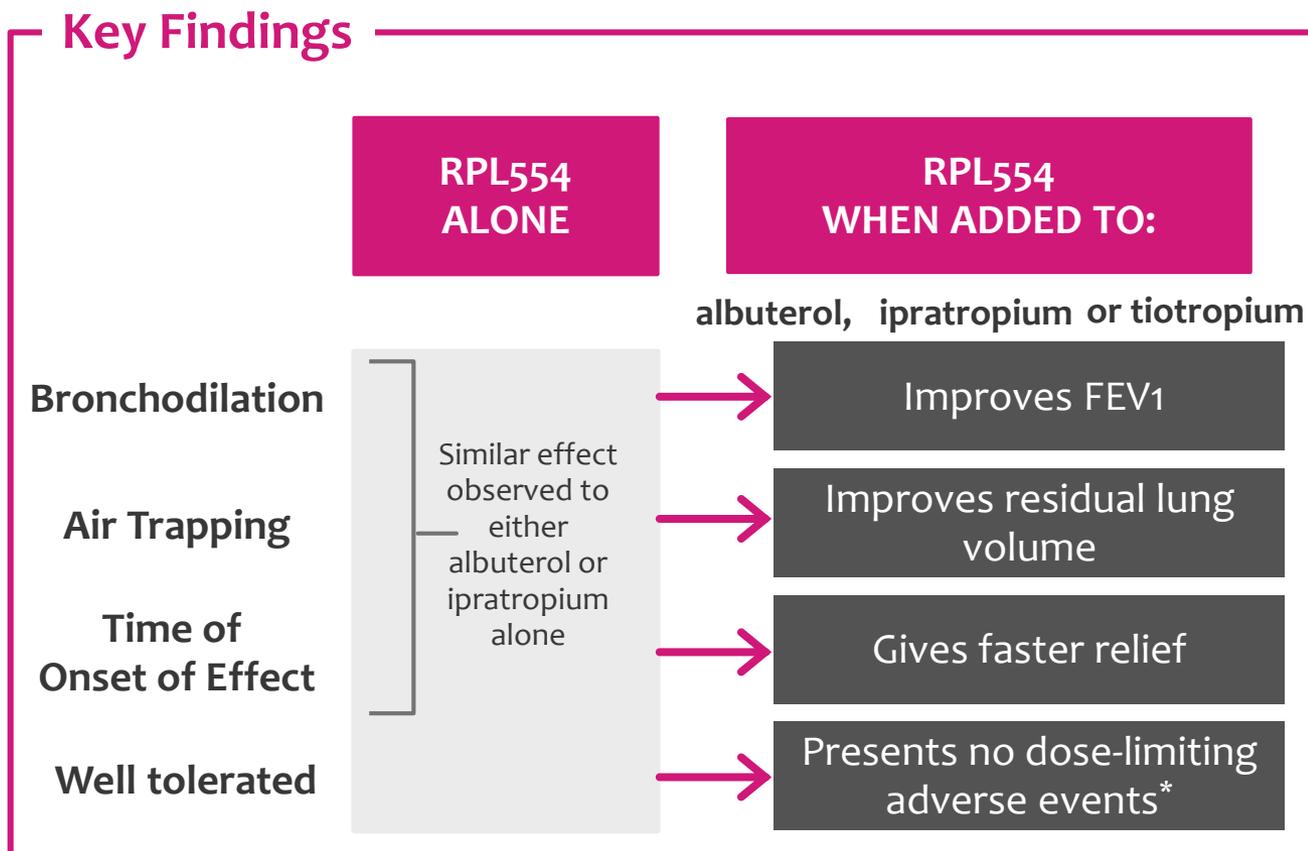


New therapies are required for patients with progressive disease and symptoms despite current treatment options

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



Verona Pharma



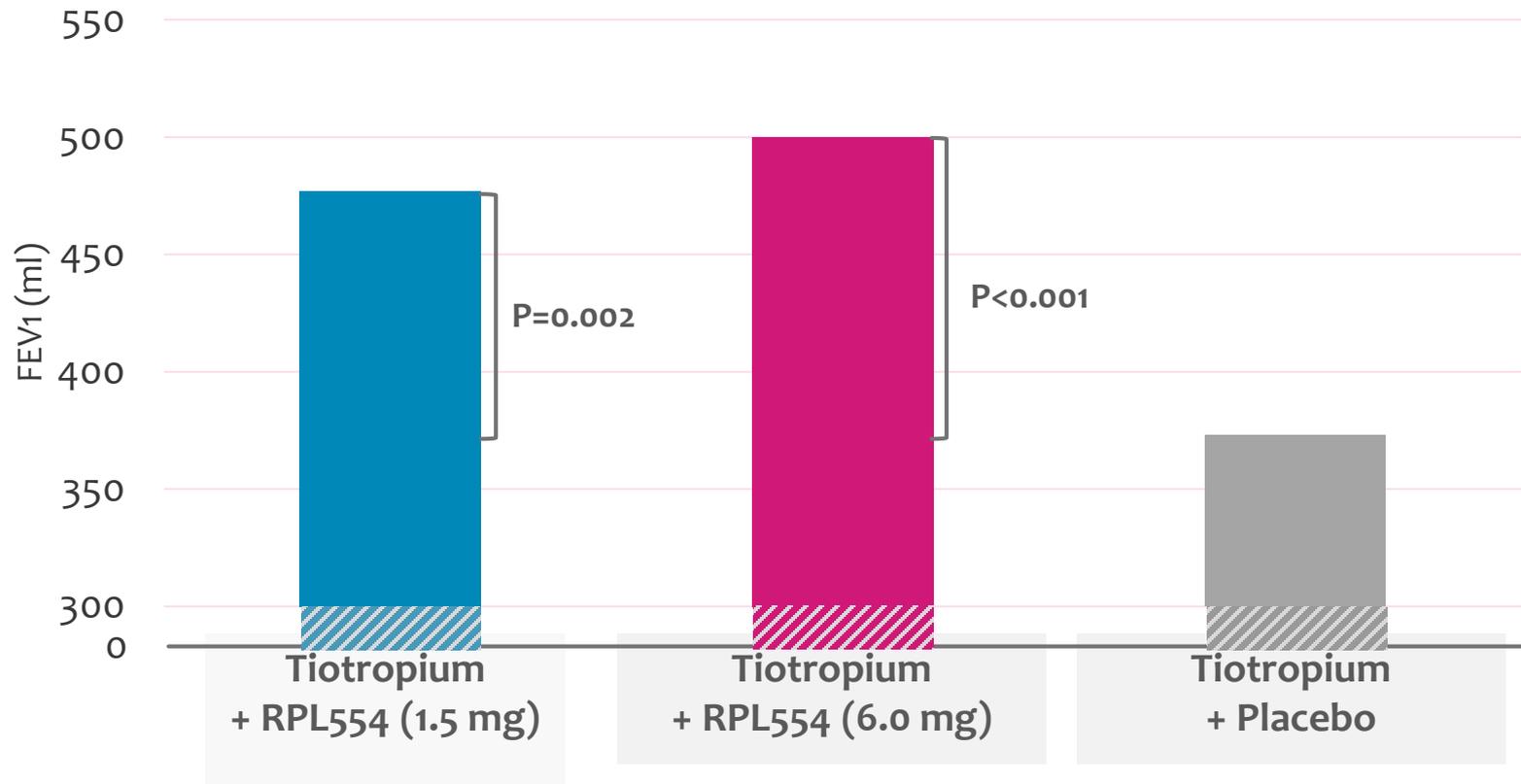
\*in completed clinical trials

Source: Ph2 studies RPL554-009-2015; RPL554-CO-202

# RPL554: Significant Additional Bronchodilator Response when Inhaled on Top of Tiotropium (Spiriva)

## Peak Change from Baseline in FEV<sub>1</sub> (ml) on Day 3

N=27-28



Source: Phase 2 study RPL554-CO-202  
P values vs placebo

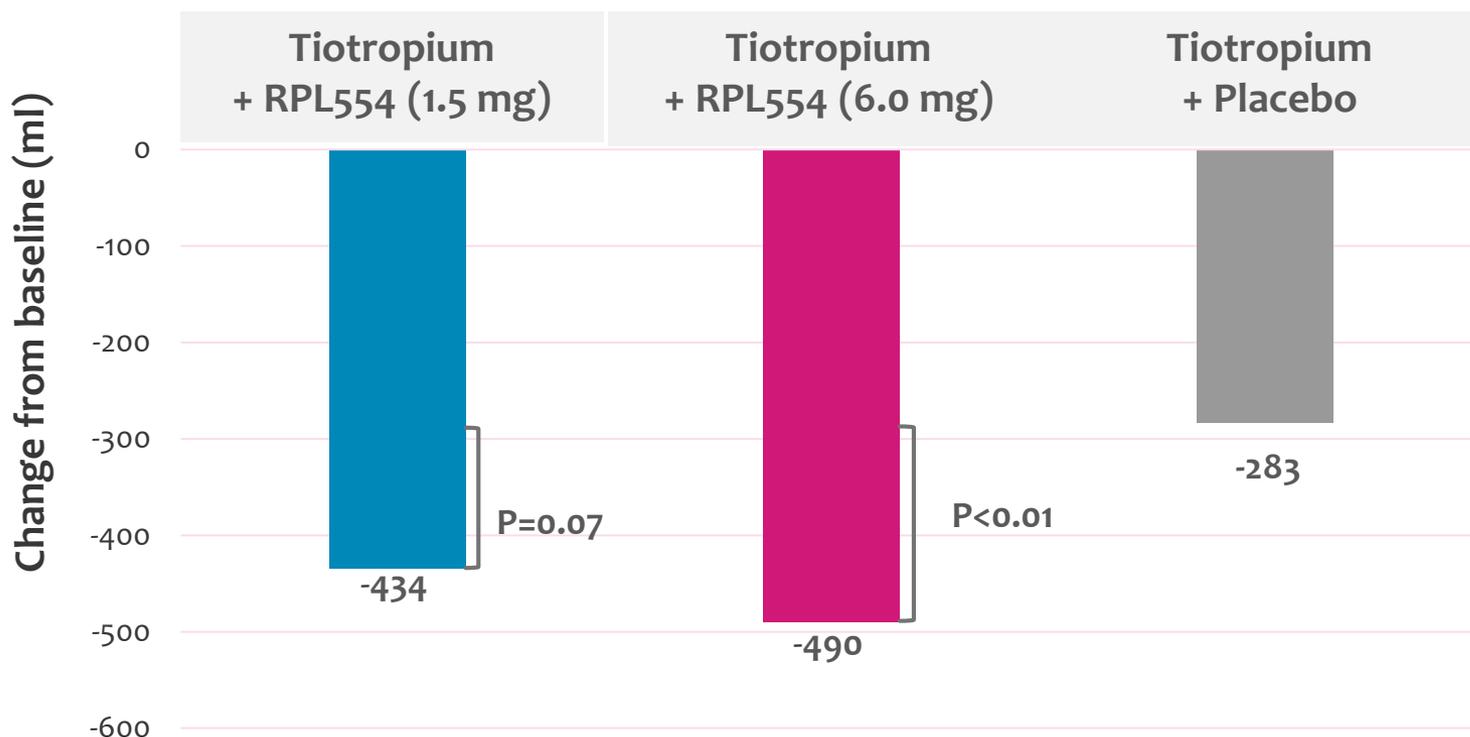
# RPL554: Marked Reduction in Hyperinflation, Residual Volume (RV, air trapping) as Compared to Tiotropium Alone



Verona Pharma

## Reduction in Hyperinflation (ml) on Day 2

N=27-28



Reduction of hyperinflation is typically correlated with improvement of shortness of breath

Source: RPL554-CO-202  
P values vs placebo

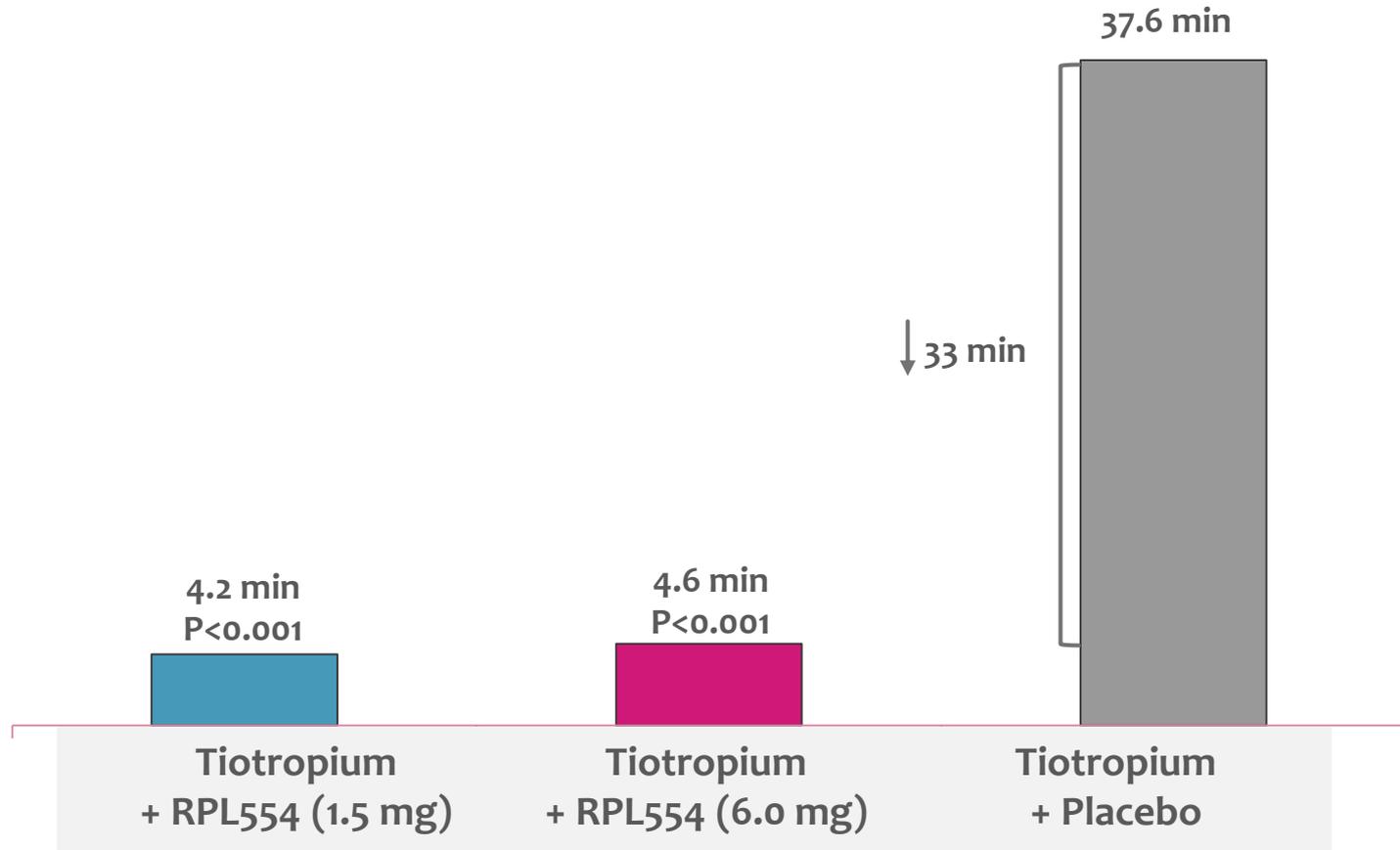
# RPL554: Combination Increases Speed of Onset of Bronchodilator Effect



Verona Pharma

## Median Time to Onset ( $\geq 10\%$ improvement in FEV<sub>1</sub>; mins) on Day 3

N=27-28



**Reinforces the potential of RPL554 in treating acute exacerbations of COPD**

Source: RPL554-CO-202  
P values vs placebo

# RPL554: Four Week Phase 2b Study in Moderate to Severe COPD



Verona Pharma

## **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:**

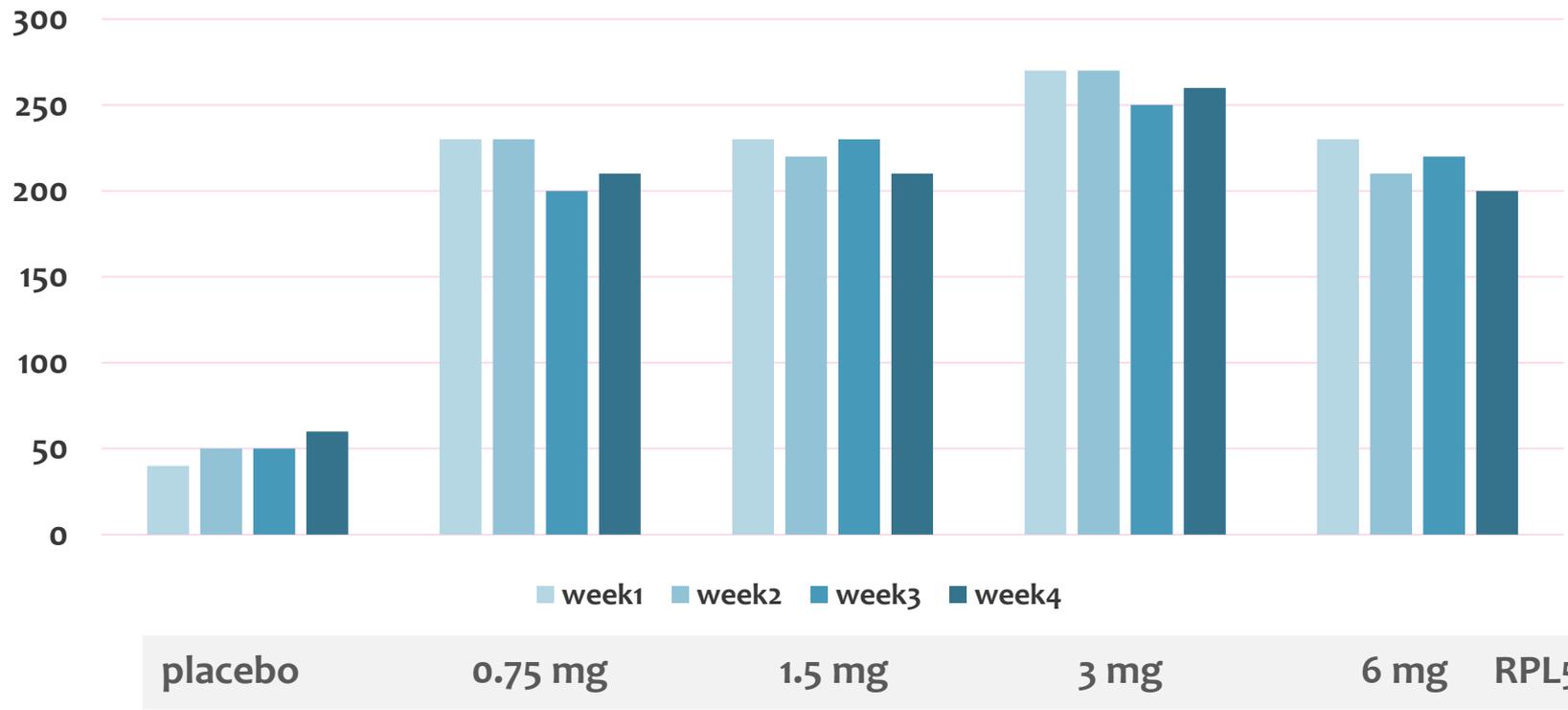
- Approximately 45 centers in Western & Eastern Europe

## **RPL554 Dosage:**

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

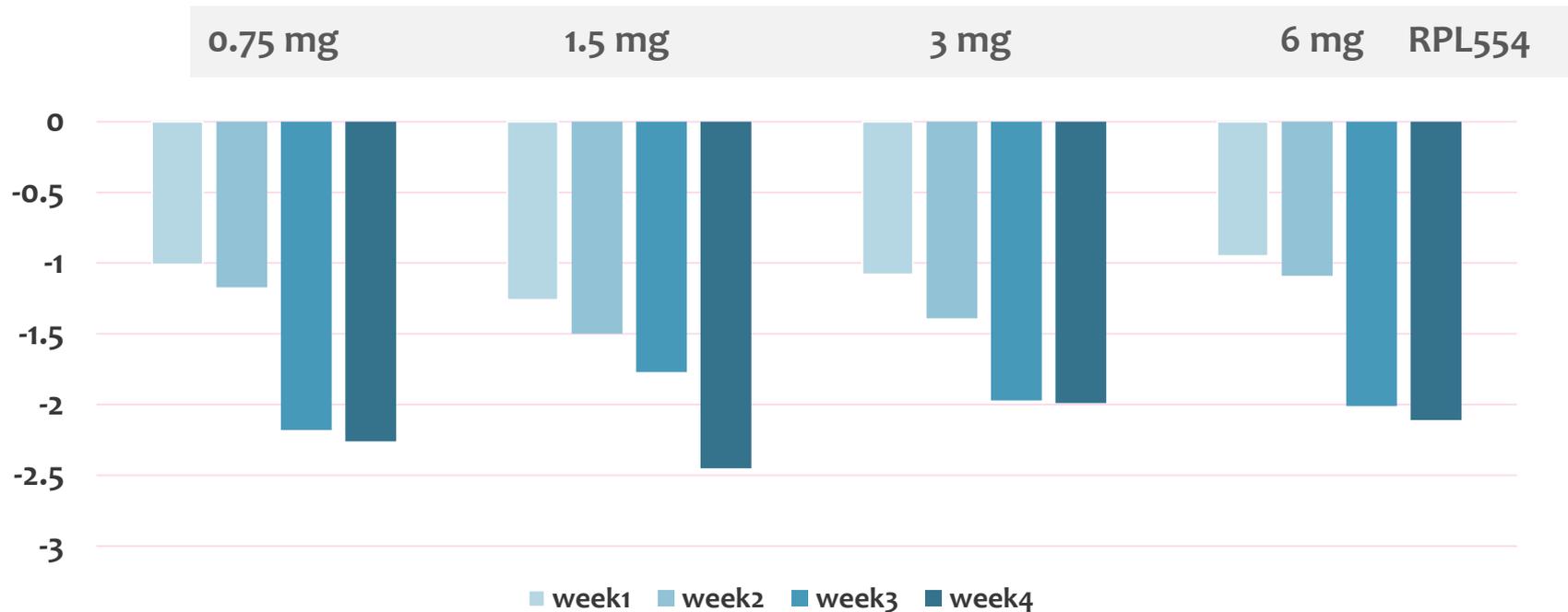
# Significant, Clinically Meaningful Bronchodilator Response Maintained over Four Weeks

Peak Change from Day 1 in Baseline in FEV<sub>1</sub> (mL) on week 4  
(p<0.001)  
N=403



# RPL554: Rapid and Progressive Improvement of COPD Symptoms with All Doses from Weeks 1 to 4

Total score E-RS\*: COPD by week (placebo corrected,  $p < 0.02$ )  
N=403

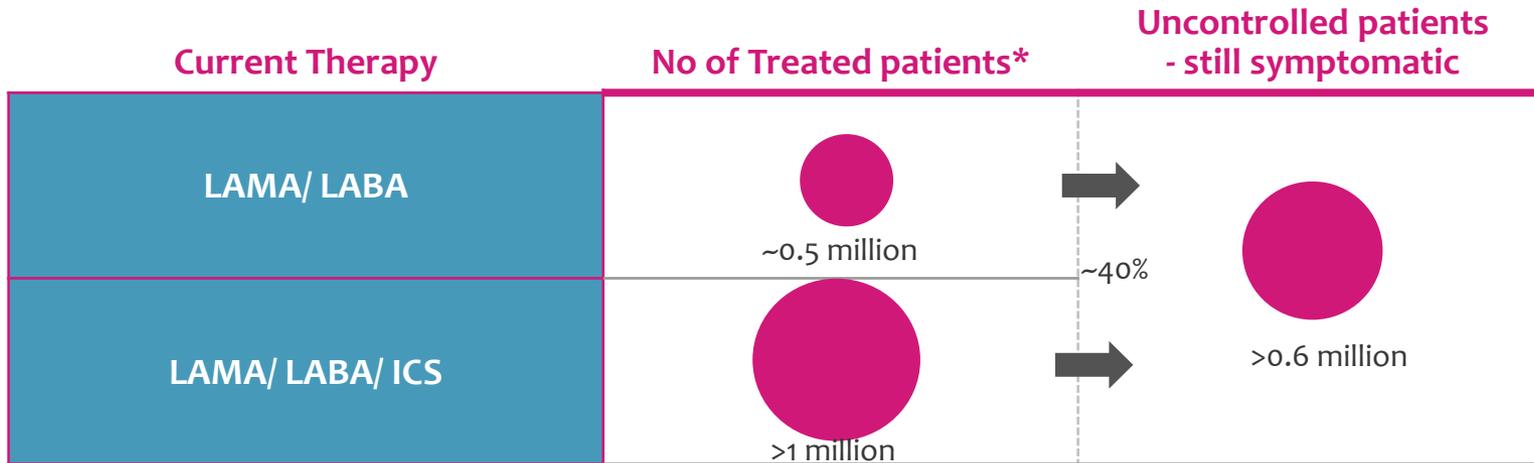


(\*E-RS (EXACT-PRO) - a recognized patient-reported outcome measure for use in clinical studies of COPD)

# Nebulized RPL554 for COPD: > 600,000 Potentially Eligible Patients in U.S.



Verona Pharma



Physicians see the value of a novel MoA in this high unmet need population with very limited alternatives\*

Estimate ~40% of patients receiving dual bronchodilators are uncontrolled\*\*

Source: \*IQVIA claims data analysis Q2 2017 & Verona market research Q1 2018

\*\*Vestbo J, et al. Lancet 2017; 389: 1919-29.

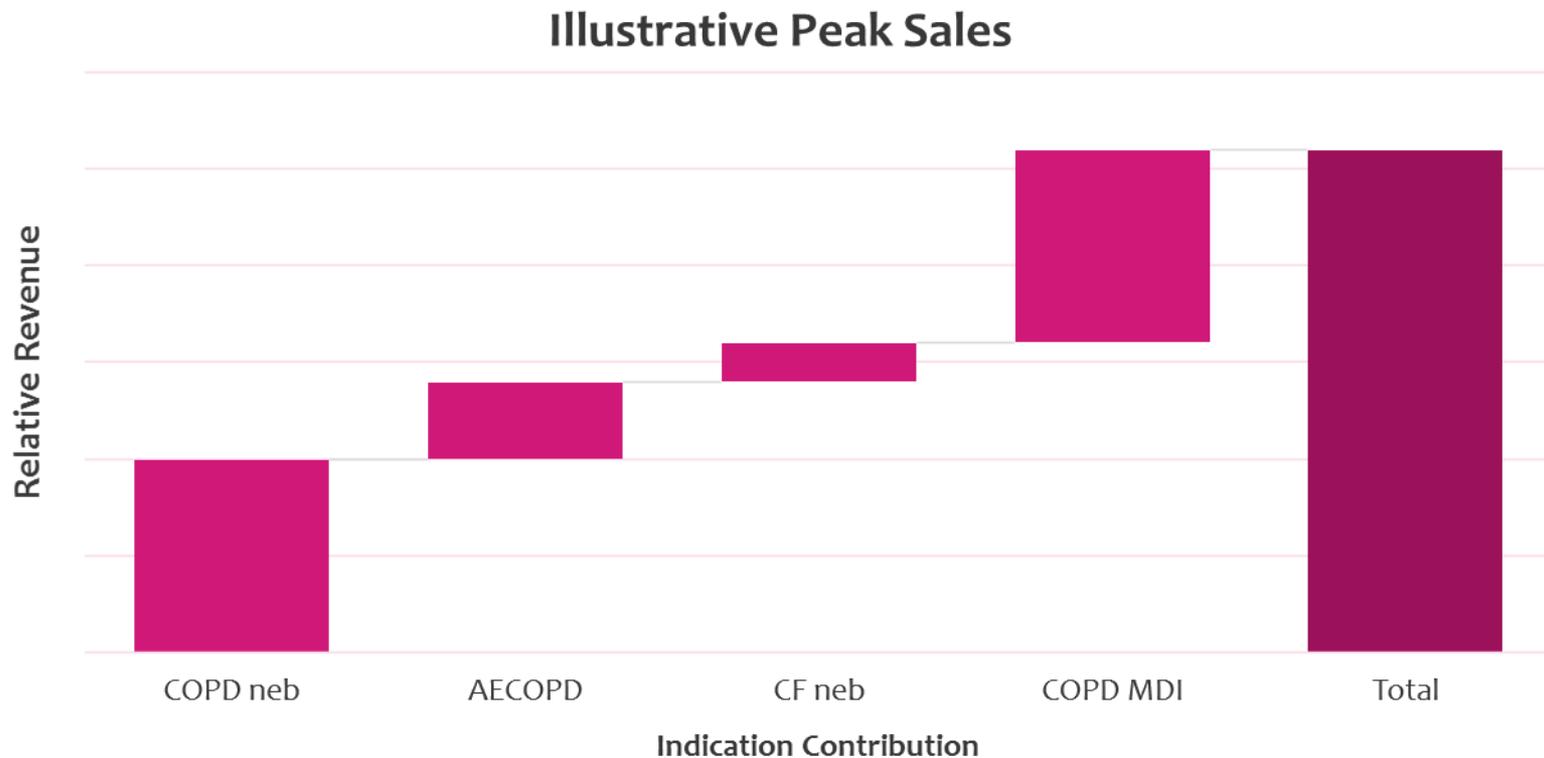
Mullerova H, et al. Am J Respir Crit Care Med 2017; 195: A4986.

Mahler, D. A., et al. European Respiratory Journal 2014, 43(6), 1599–609.

# RPL554: Targeting Multiple Indications Allows Earlier Access to Large US Markets



Verona Pharma



# RPL554: Phase 3 Plan for Nebulized RPL554 Development as Maintenance Treatment of COPD



Verona Pharma

- Pivotal trials in **moderate to very severe COPD patients and/or patients with COPD symptoms** who prefer/accept nebulizer treatment
- Position **RPL554 as “add-on” to standard of care**
  - Supported by quantitative market research
- Potential key endpoints: **lung function** (eg. FEV<sub>1</sub>, residual volume) **and/or symptoms**
- Focus on speed and cost in pivotal trials with nebulizer treatment
  - **Two trials (3 to 6 months duration) with 12 months safety data**
  - **Pivotal trials planned to commence in 2019**
  - **NDA filing planned for 2021**



# 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 March 2018**

# RPL554: Demonstrates Favorable PK and PD Profile in CF Patients in Phase 2a Trial



Verona Pharma

- 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 consistent with that observed in COPD patients, 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
  - 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 with an adverse event profile consistent with other studies

**Results support further development in CF**

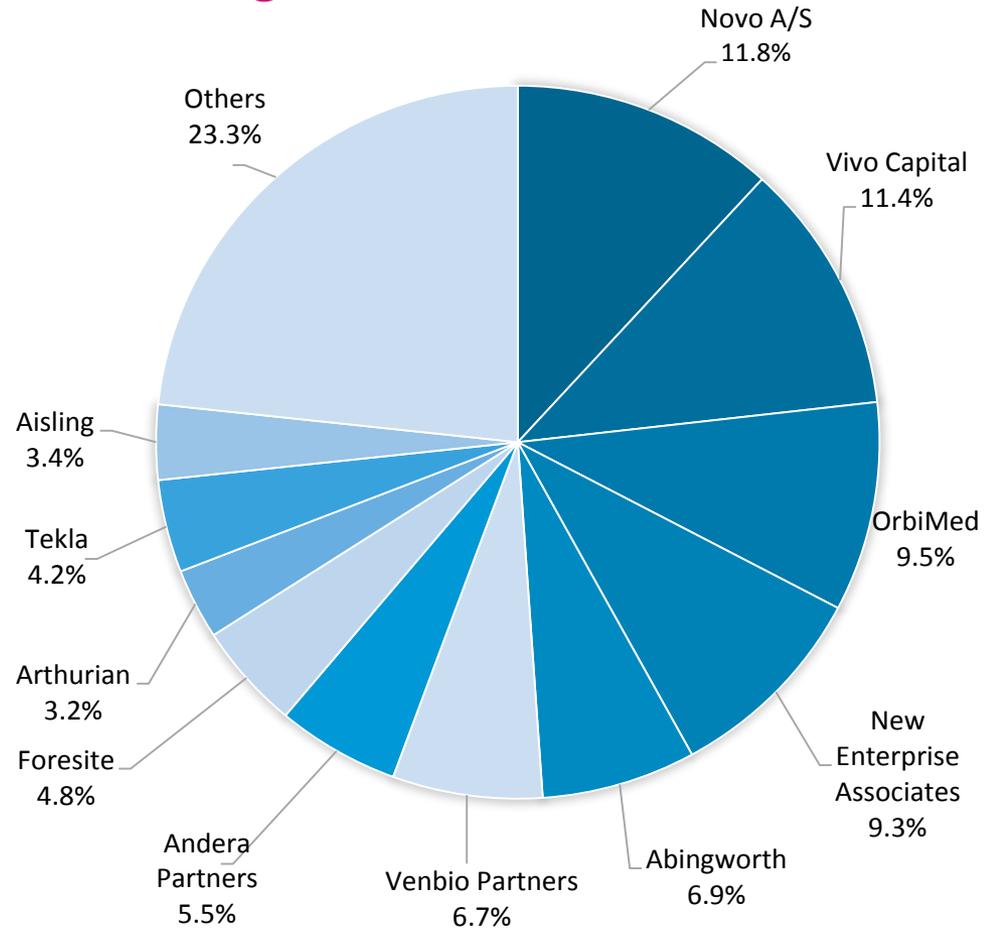


# Well Financed with Major Healthcare Investors

## Financial Overview Mar 31, 2018

Cash and Cash Equivalents	\$101.9M <sup>1</sup>
Operating Expenses 1Q18	\$8.2M <sup>1</sup>
Market cap	\$253M <sup>2</sup>

## Shareholdings



<sup>1</sup>Exchange rate used (US dollars per pound sterling): March 31, 2018 \$1.4027

<sup>2</sup>Fully diluted 125m shares or 15.6m ADSs, ADS price \$16.22 May 11, 2018

# Significant Value Inflection Points over next 12 Months, Ahead of Commencing Phase 3



Verona Pharma

## RPL554 nebulized formulation as maintenance treatment of COPD

- Planned Phase 2a study start: RPL554 as add-on to LAMA/LABA w/wo ICS 3Q 2018
- Phase 2a clinical data read out from “add-on study” above 1H 2019
- Decision on viability of drug product in plastic ampules mid 2019
- Regulatory agreement to proceed into Phase 3 studies 2019

## RPL554 pMDI and DPI formulations (out-licensing)

- Expected completion of pre-clinical studies 2018/19
- Expected start of clinical Phase 2 trials 2018/19
- Clinical data read out DPI/MDI Phase 2 trials 2019

## RPL554 anti-inflammatory treatment in Cystic Fibrosis

- Clinical anti-inflammatory/ Proof-of-concept study TBD