



# Verona Pharma



## Breathtaking science

Developing respiratory drugs

to improve health and quality of life



June 2017



# 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 final prospectus filed with the Securities and Exchange Commission (the “SEC”) on April 28, 2017 relating to the Company’s Registration Statement on Form F-1 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, to conform any of the forward-looking statements to actual results or to changes in its expectations.



# Verona Pharma



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










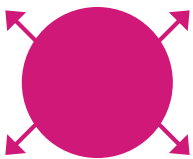


Inhaled dual inhibitor of  
enzymes PDE3 and PDE4

**RPL554**

Current Focus:  
COPD and CF

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

# RPL554: Rich Product Pipeline

Indication	Delivery	Pre-Clinical	Phase 1	Phase 2	Phase 3	Addressable Market Opportunity
COPD Maintenance (Home)	 Nebulizer					
COPD Acute (Hospital)						
Cystic Fibrosis						
COPD Maintenance (Home)	 Inhaler					
Asthma						



# Experienced Team

## Management

**Jan-Anders Karlsson, PhD**  
Chief Executive Officer



**Piers Morgan, MA, ACA**  
Chief Financial Officer



**Kenneth Newman, MD, MBA**  
Chief Medical Officer



**Richard Hennings, BSc**  
Commercial Director



**Peter Spargo, PhD**  
SVP CMC



**Claire Poll, LLB**  
Legal Counsel



## In Management and Board's prior lives...

involved in successful development / commercialization of many of the drugs used to treat COPD including





# Financial Overview

Listed on AiM (VRP.L) and NASDAQ (VRNA)

**Cash and Cash Equivalents**  
(as of December 31, 2016)

\$51.1M<sup>2</sup>

**April 2017 NASDAQ IPO**  
(including over-allotment option)

\$90M NASDAQ IPO

**Operating Expenses**

\$9.5M<sup>1</sup>  
(12 Months Ended 12/31/16)

**Market Cap**  
(fully diluted)

\$242M<sup>2,3</sup>  
ADS ratio 1:8 ordinary shares

Exchange rates used (U.S. dollars per pound sterling):

<sup>1</sup>Average for 2016 £1.3467

<sup>2</sup>May 30, 2017 \$1.2850

Share price and shares used:

<sup>3</sup>125.8m shares fully diluted (16.1m ADSs); share price 150p (\$15.40 / ADS)



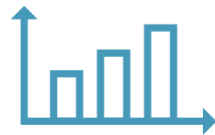
# Clear Success Drivers



**Large COPD patient population** with significant **unmet medical need** and **little** treatment **innovation** in decades



**Significant  
Market opportunity**



**Robust clinical data and validated endpoints**

FEV<sub>1</sub> in 8 P1 and P2a trials

**P2b planned –**  
4 weeks; 400 subjects



**Clear, efficient  
pathway to  
Approval**



**Significant potential  
demand** from  
patients and physicians  
for new medications



**Strong potential for  
Adoption**



Broad application:  
**“pipeline in a product”**  
Global commercialization  
rights



**Significant outlook for  
Growth**



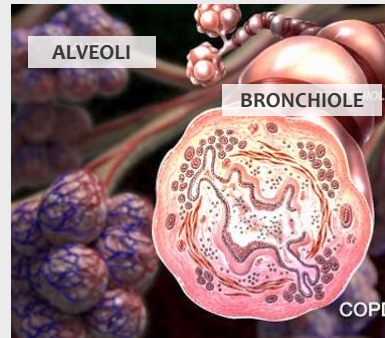
Verona Pharma

# COPD: Devastating Disease, Affecting Many



CAUSES

Smoking  
Air pollution



Inflames and narrows  
airways (bronchioles) and  
can damage alveoli

PROGRESSIVE  
DISEASE

No cure



“Take a deep breath, blow out 20% –  
now walk around,  
holding the rest forever.”  
COPD Sufferer



**Affects Many**

**3<sup>rd</sup>** leading cause of death  
**210M** global sufferers  
**24M** U.S. sufferers



**Very Costly**

**Many** exacerbations and hospitalizations  
**~\$50B** projected annual medical costs by  
2020 (U.S. alone)  
**\$10B+** annual global sales of COPD drugs

Sources: IMS, CDC



# COPD Sufferers Require Maintenance and Acute Treatment



Verona Pharma

## Maintenance (Home)



### U.S. alone: 24M living with COPD

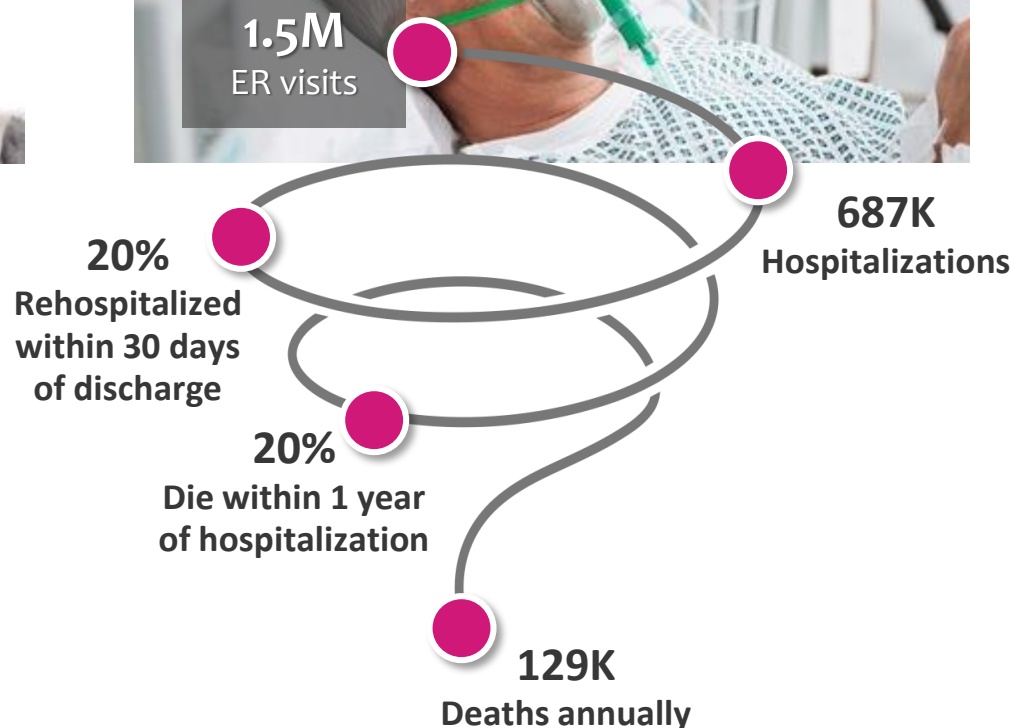
- 15M diagnosed and under treatment
- Approximately 2M severe/very severe

### Treatment goals:

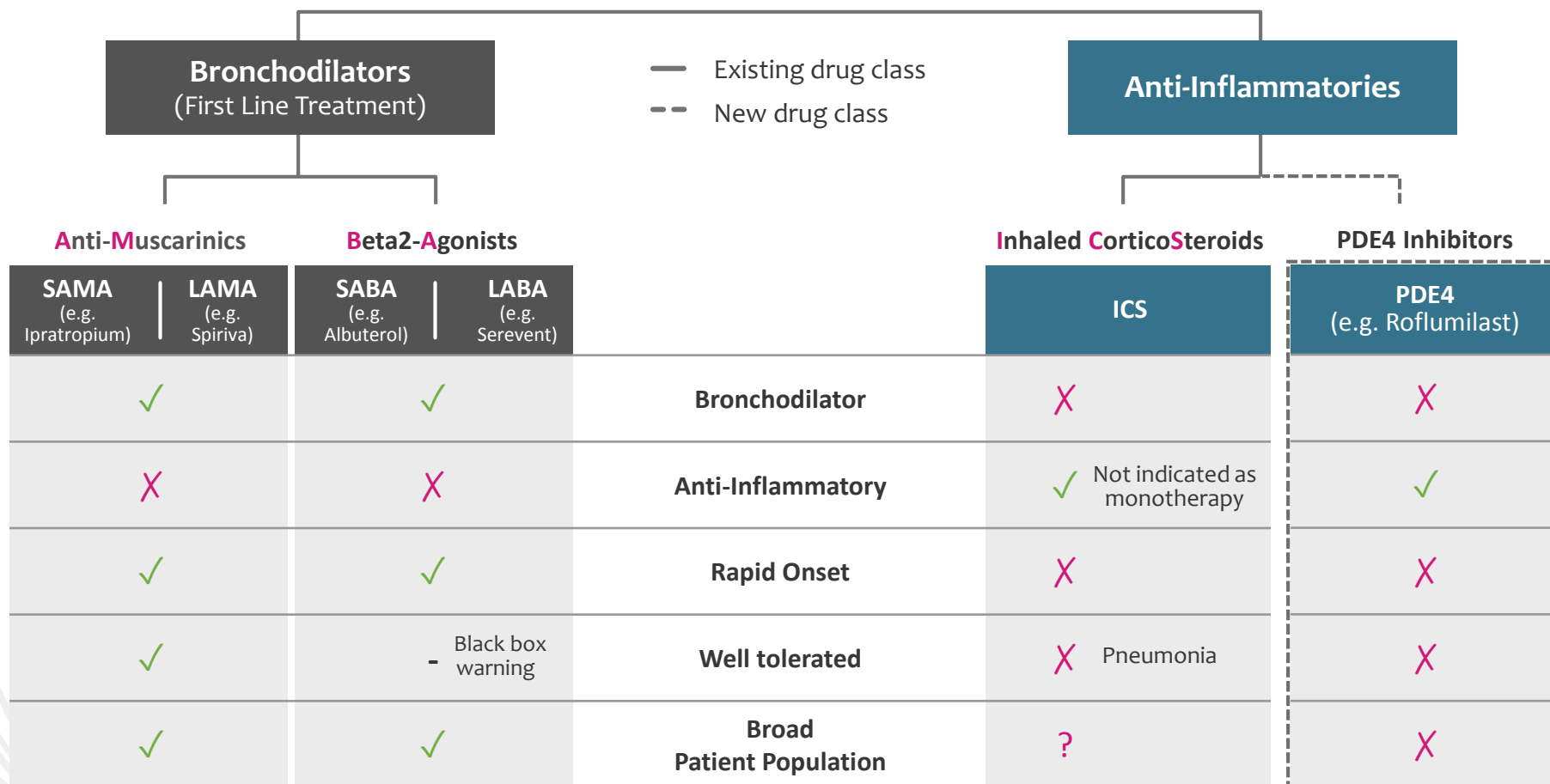
- Improved lung function
- Improved quality of life
- Prevent exacerbations

Note: U.S. only data

## Acute (Hospital)



# Current Therapies: Little Innovation and Many Limitations

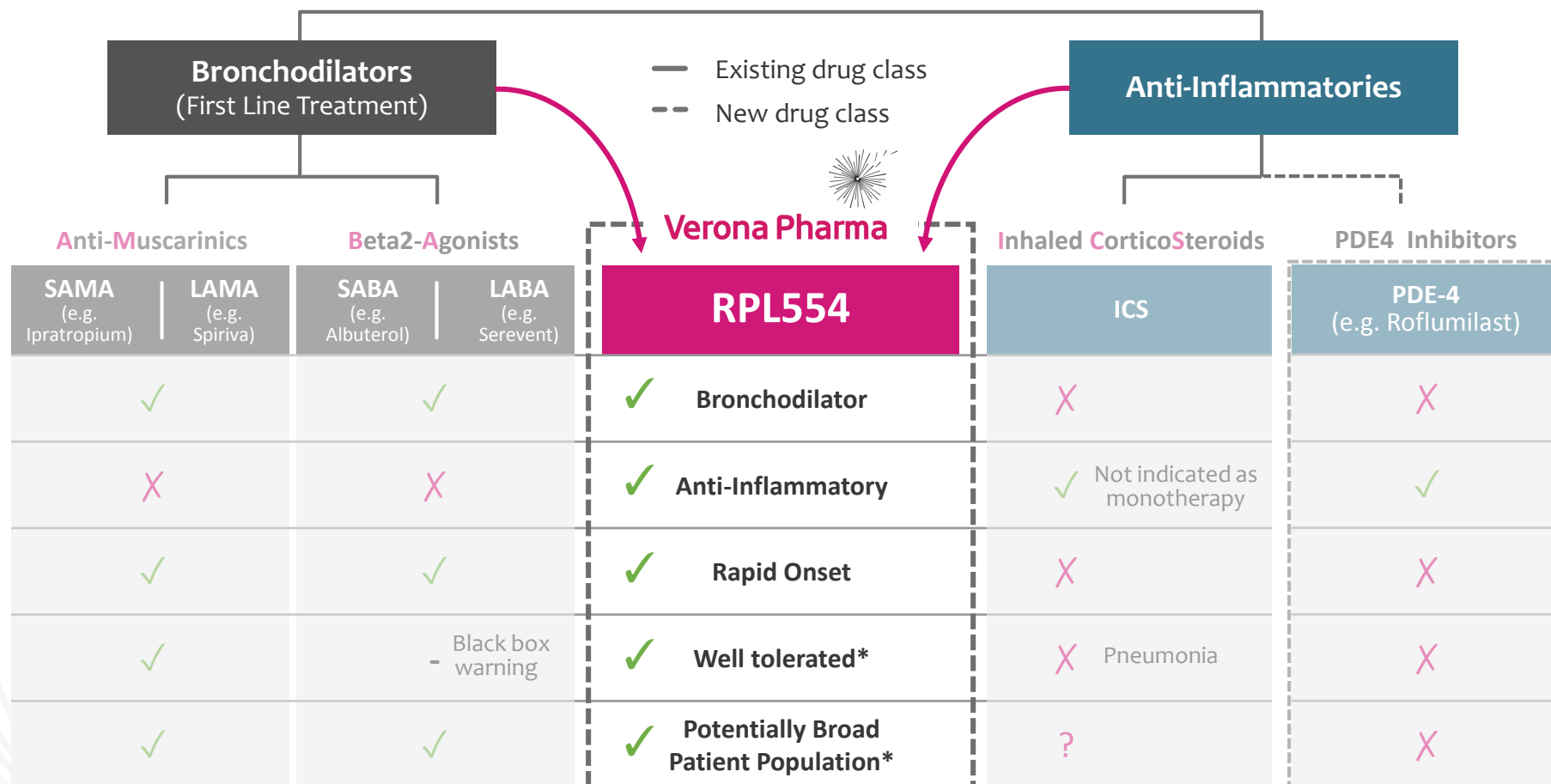


Many patients treated with approved COPD drugs/combinations do not experience significant improvements in quality of life and continue to suffer from significant symptoms

# RPL554: Potential to Address Limitations of Current Therapies



Verona Pharma

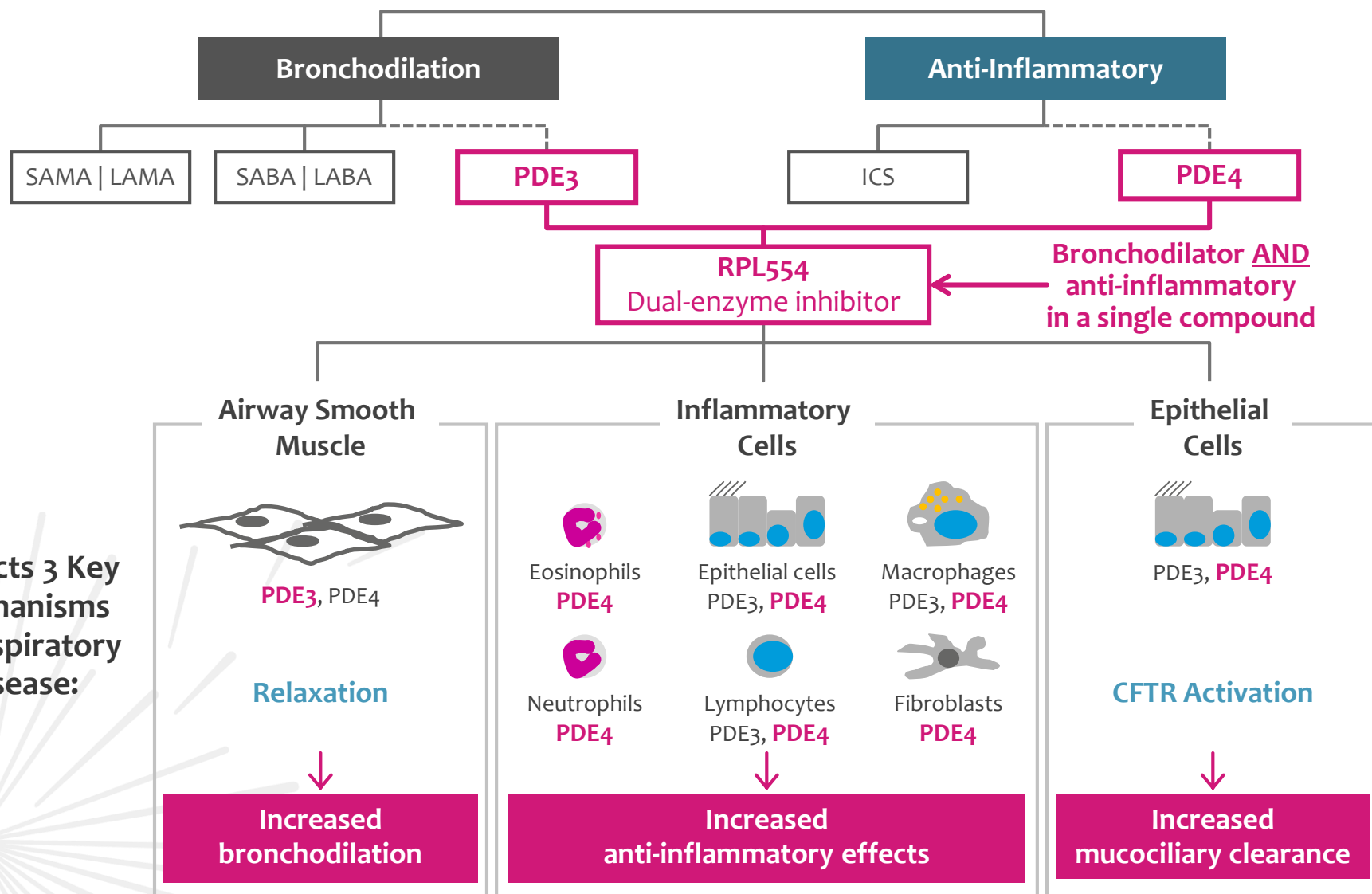


\*In clinical trials to date (RPL554 not yet approved)

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



Verona Pharma



Impacts 3 Key Mechanisms in Respiratory Disease:



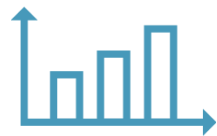
# Clear Success Drivers



**Large COPD patient population** with significant **unmet medical need** and **little** treatment **innovation** in decades



**Significant  
Market opportunity**



**Robust clinical data and validated endpoints**

FEV<sub>1</sub> from 8 P1 and P2a trials

**P2b planned –**  
4 weeks; 400 subjects



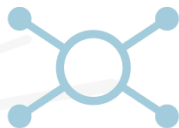
**Clear, efficient  
pathway to  
Approval**



**Significant potential demand** from patients and physicians for new medications



**Strong potential for  
Adoption**



Broad application:  
**“pipeline in a product”**  
Global commercialization rights



**Significant outlook for  
Growth**

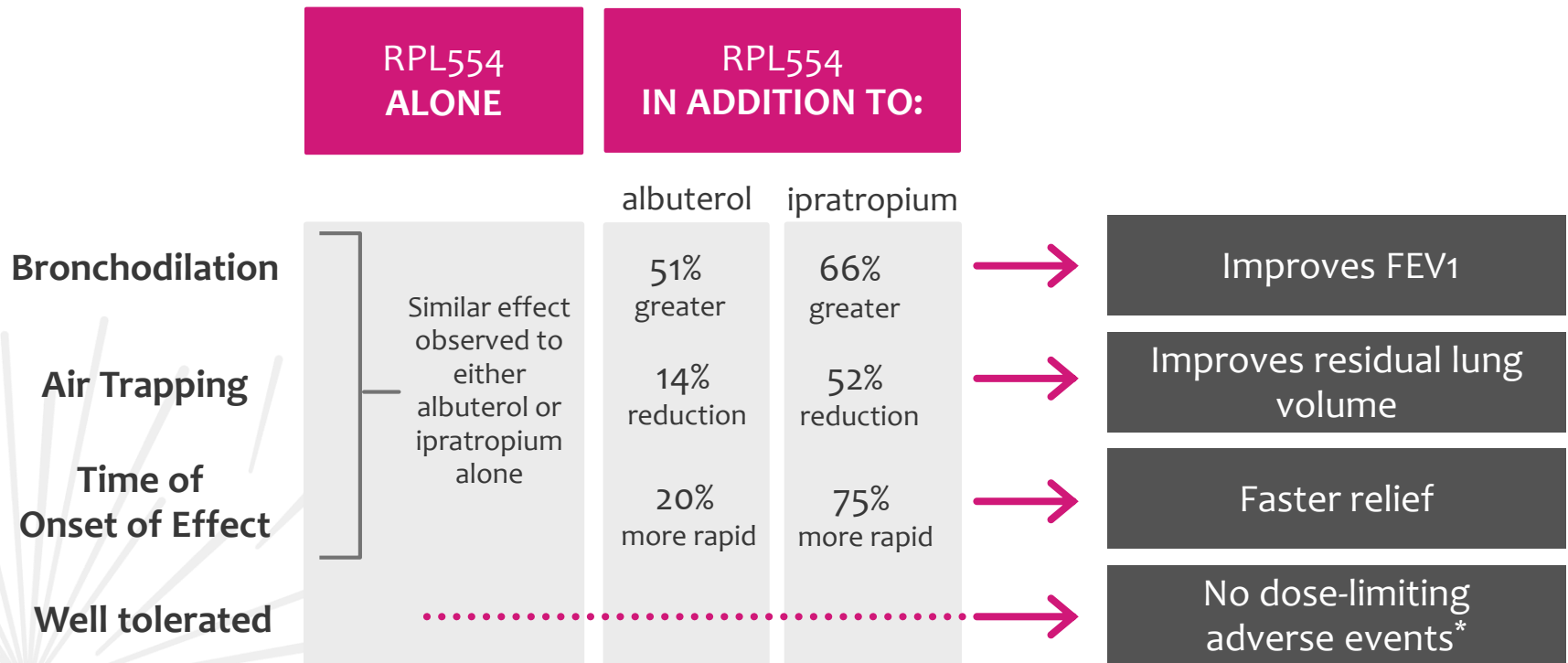




# RPL554: Significantly De-Risked

Phase 1 and 2a: 8 Clinical Trials, 282 Subjects

## Key Findings

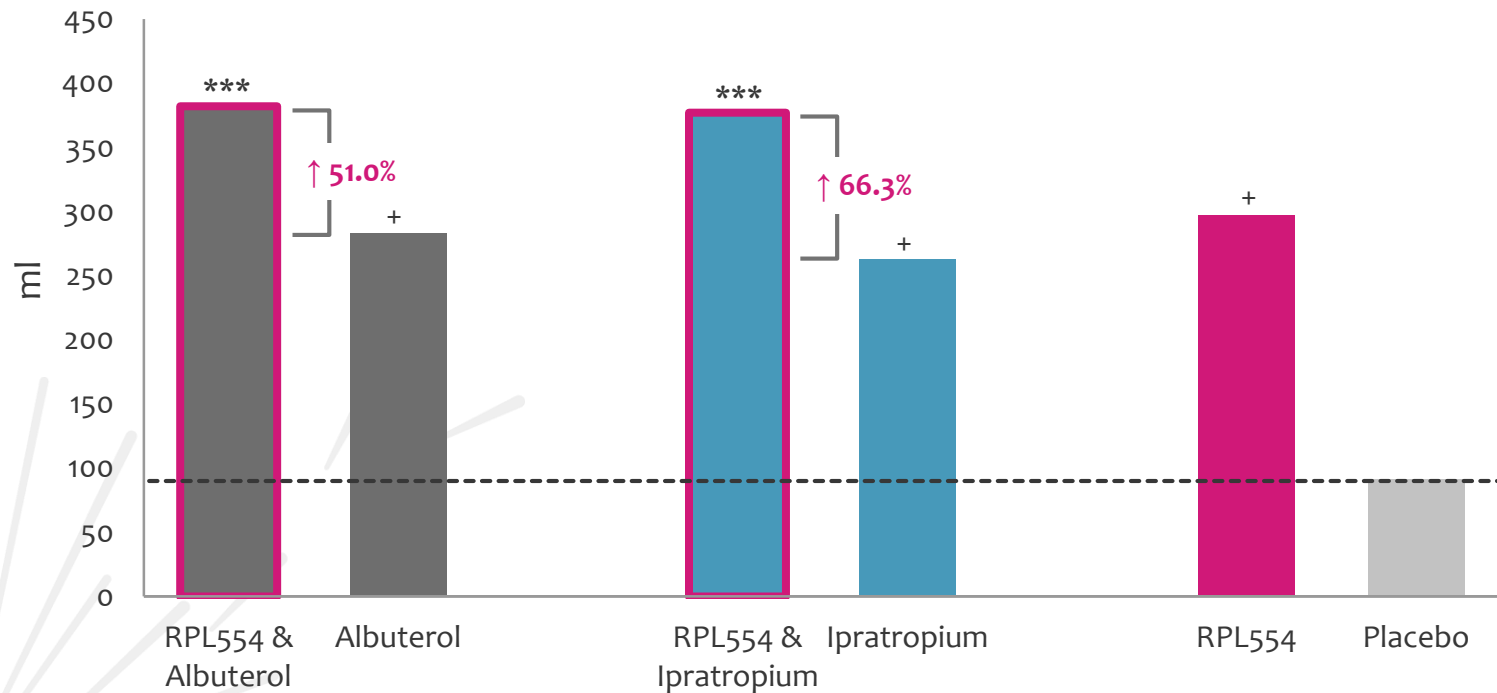


\*in completed clinical trials

# RPL554: Significantly Improves Lung Function in COPD Patients

## Peak Change from Baseline in FEV<sub>1</sub>(L)

N=36



Source: RPL554-009-2015

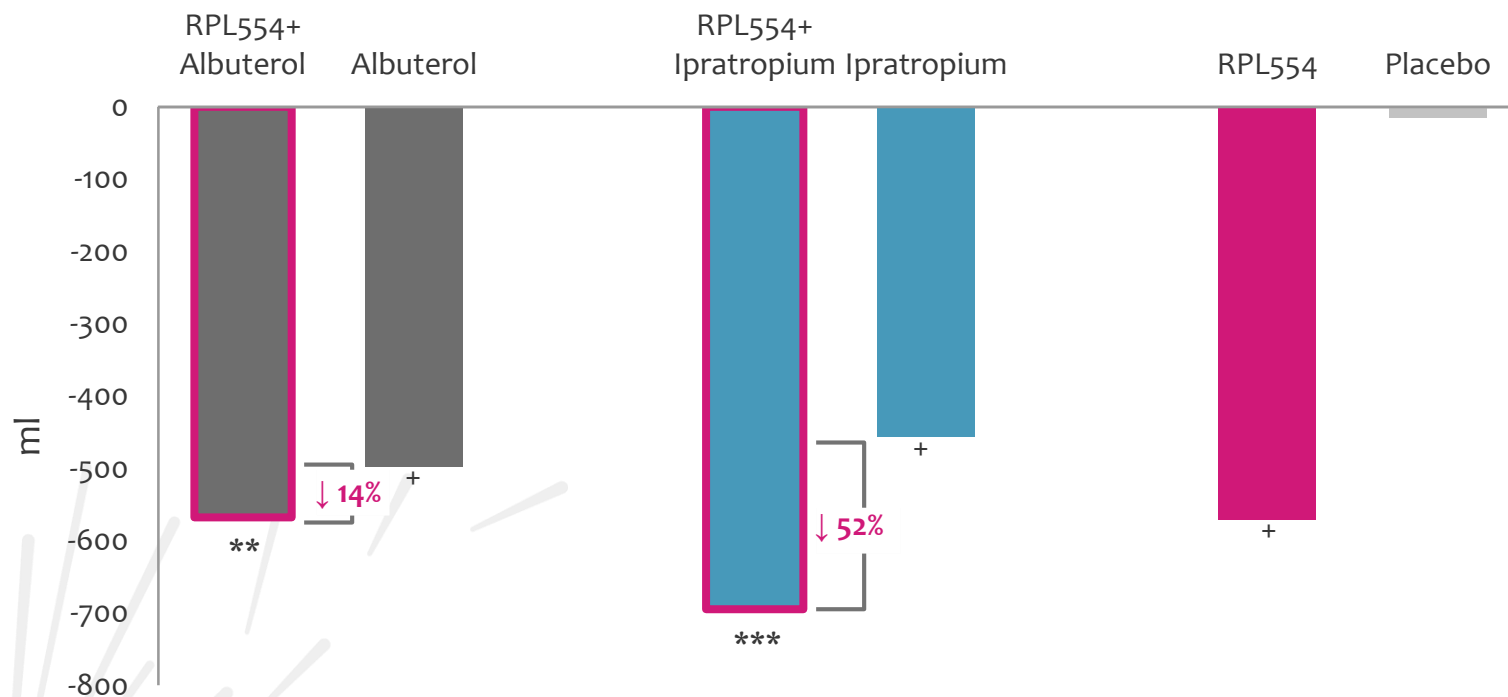
+ p<0.001 vs placebo

\*\*\* p<0.001 vs. albuterol or ipratropium alone

# RPL554: Reduces Residual Lung Volume (Air Trapping) in COPD Patients

## Change from Baseline in Residual Volume at 1 Hour

N=36



**Reduction in Hyperinflation Is Typically Correlated with Improvement in Shortness of Breath**

Source: RPL554-009-2015

+ p<0.001 vs. placebo

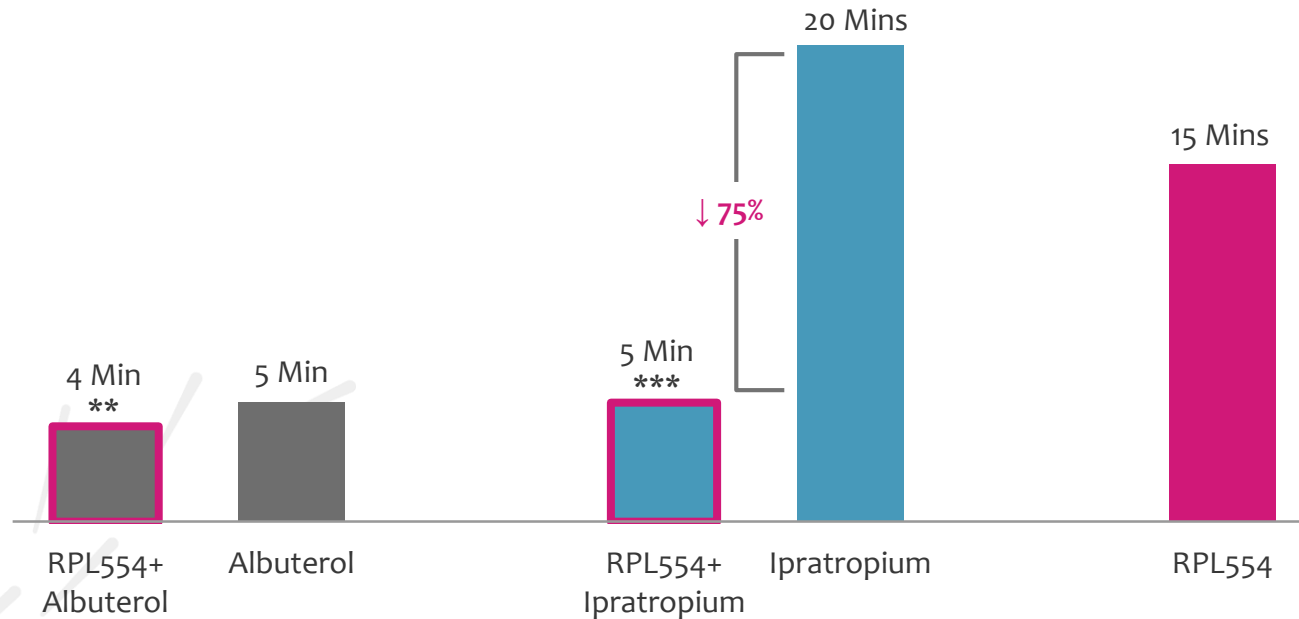
\*\* p=0.016 vs. Albuterol alone at 1 hour timepoint

\*\*\* p=0.001 vs. Ipratropium alone at 1 hour timepoint

# RPL554: Faster Onset of Bronchodilation in COPD Patients

Time to Onset (10% improvement in FEV<sub>1</sub>) (mins)

N=36



## Reinforces Potential of RPL554 in Treating Acute Exacerbations of COPD

Source: RPL554-009-2015

\*\* p<0.01 vs. albuterol alone

\*\*\* p<0.001 vs. ipratropium alone



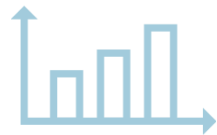
# Clear Success Drivers



**Large COPD patient population** with significant **unmet medical need** and **little** treatment **innovation** in decades



**Significant  
Market opportunity**



**Robust clinical data and validated endpoints**

FEV<sub>1</sub> from 8 P1 and P2a trials

**P2b planned –**  
4 weeks; 400 subjects



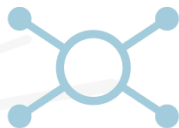
**Clear, efficient  
pathway to  
Approval**



**Significant potential  
demand** from  
patients and physicians  
for new medications



**Strong potential for  
Adoption**



Broad application:  
**“pipeline in a product”**  
Global commercialization  
rights



**Significant outlook for  
Growth**

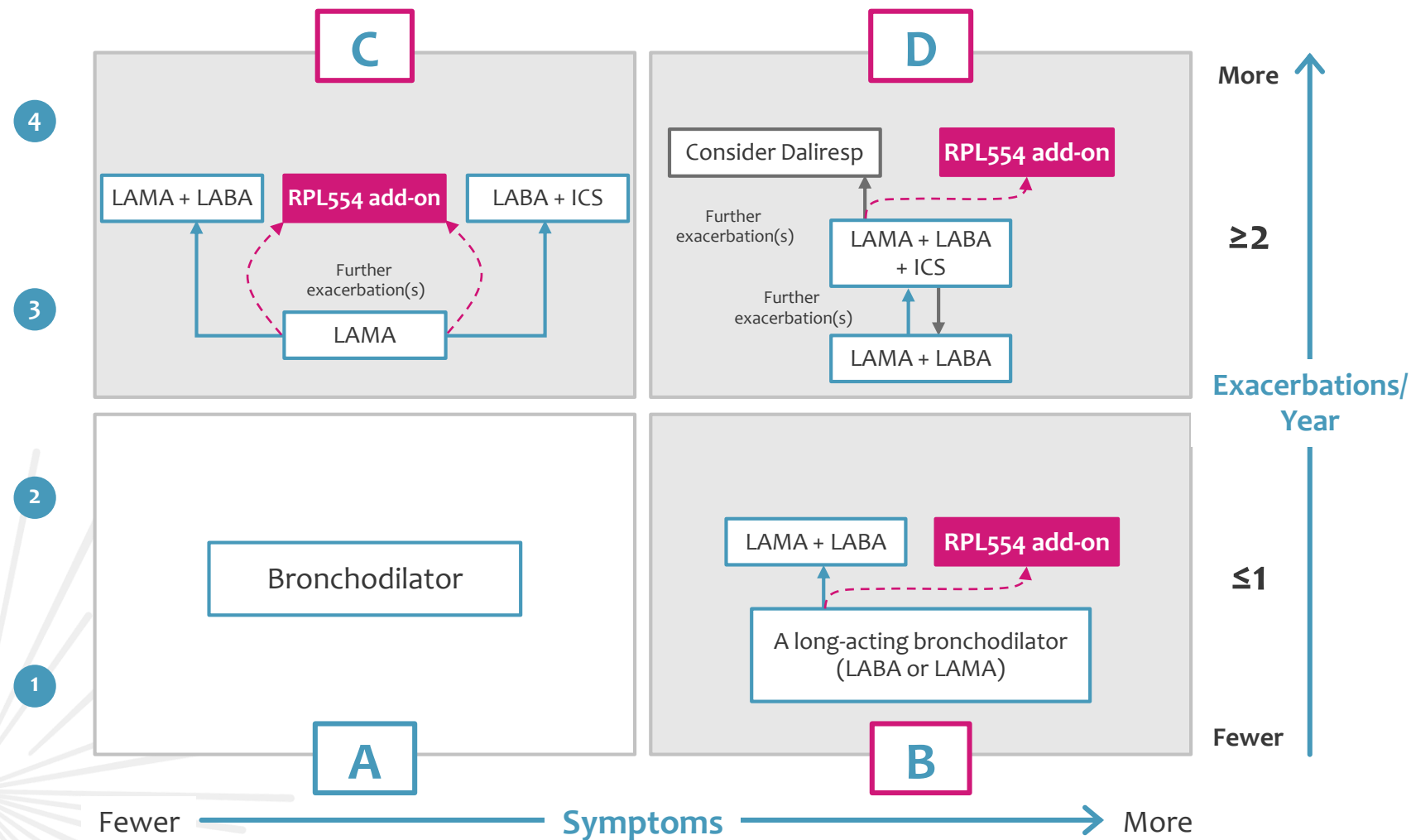


# RPL554: Potential to Improve Standard of Care Treatment for More Severe Patients











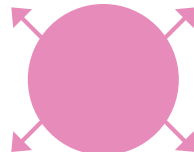

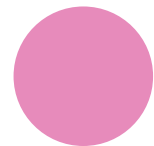


Verona Pharma

Based on GOLD COPD Guidelines 2017



# RPL554 Second Indication: Cystic Fibrosis (CF)

Indication	Delivery	Pre-Clinical	Phase 1	Phase 2	Phase 3	Addressable Market Opportunity
COPD Maintenance (Home)						
COPD Acute (Hospital)						
<b>Cystic Fibrosis</b>	<b>Nebulizer</b> 					
COPD Maintenance (Home)						
Asthma						



# CF: A Devastating Orphan Disease



## Population:

- Most common fatal inherited disease in U.S.
  - Incidence: ~70K globally

## Cause:

- Mutations in gene that encodes CFTR protein

## Symptoms:

- Inability to clear thickened mucus, impaired lung function and persistent lung infection

---

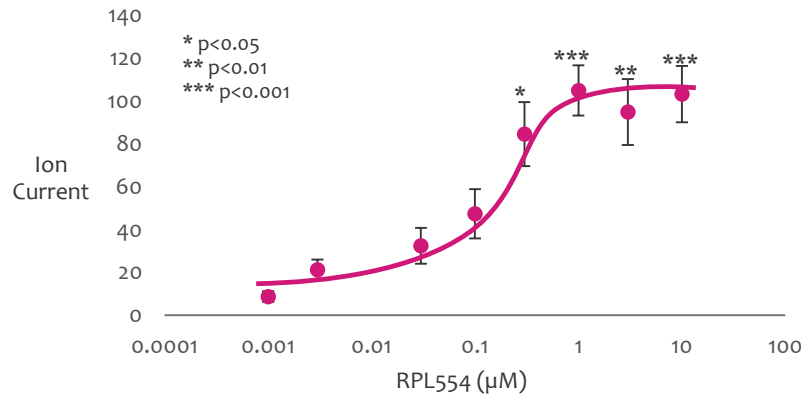
## Consequences:

- Frequent exacerbations and hospitalization
- No cure
- Median age of death – 37 years

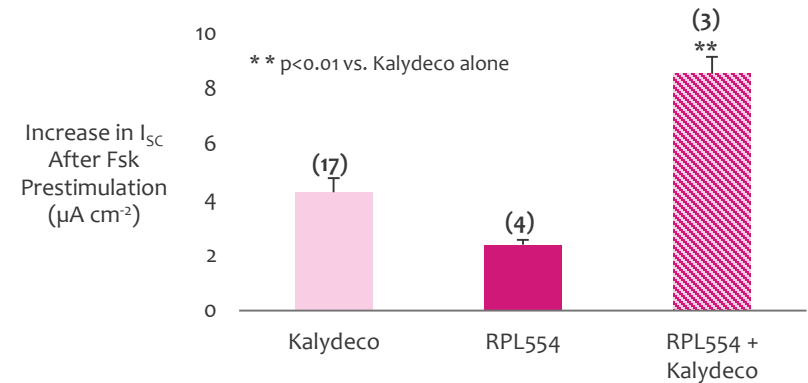


# RPL554 for CF: Pre-Clinical Studies

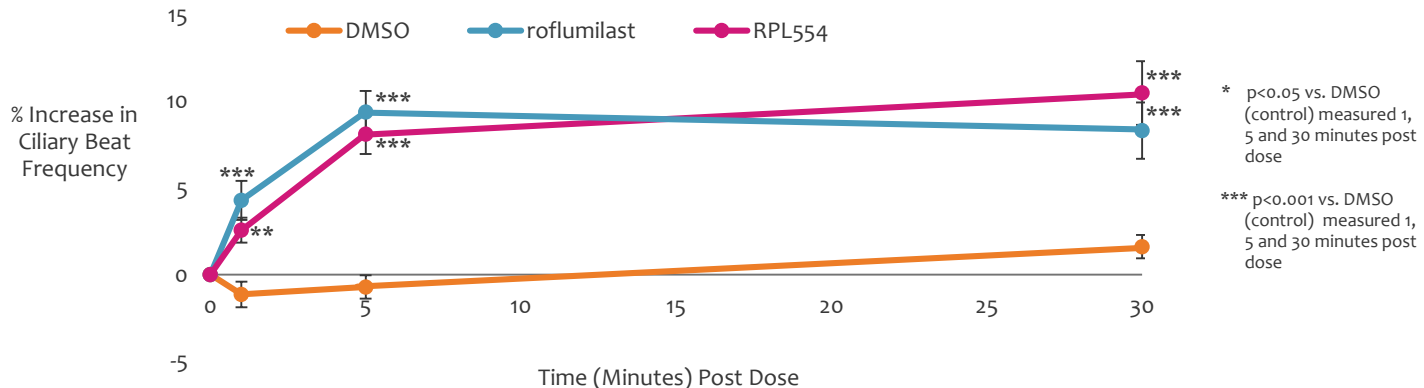
## Stimulation of CFTR



## Additive Effect When Administered with Kalydeco



## Increase Stimulation of Ciliary Beat Frequency



# RPL554: Potential to provide treatment independent of CF mutation status


---

## U.S. alone:

- ~30K patients with CF

## Treatment goals:

- Improved lung function
  - Reduced exacerbations; anti-inflammatory therapy
  - Improved quality of life
- 



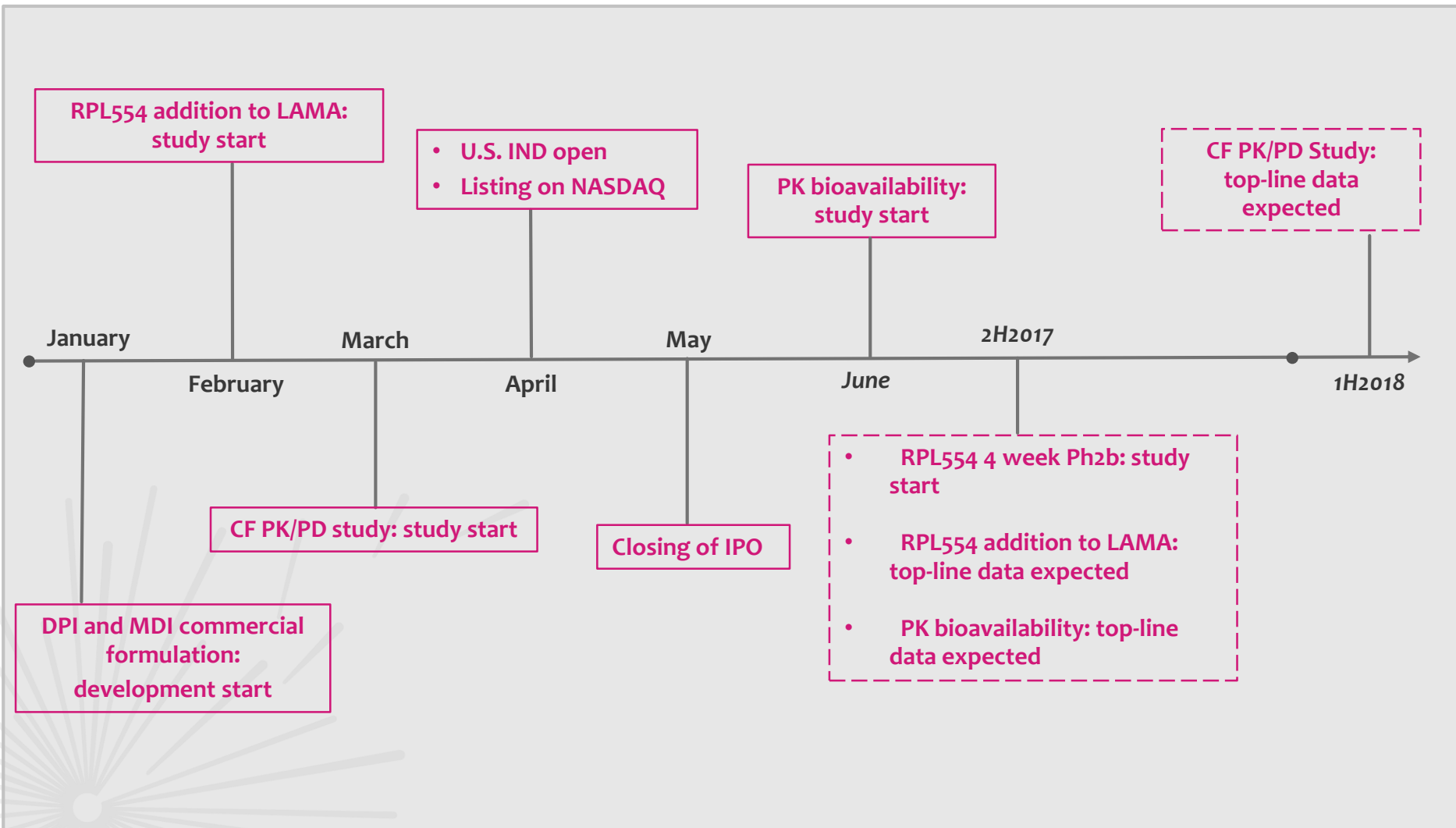
Verona Pharma	
RPL554	
Anti-Inflammatory	✓
CFTR potentiator with different mechanism of action	✓
Bronchodilator	✓
Rapid Onset	✓
Well tolerated*	✓

\*In clinical trials to date (RPL554 not yet approved)





# 2017 Achievements and Upcoming Milestones





# Verona Pharma



## Breathtaking science

Developing respiratory drugs  
to improve health and quality of life



June 2017