



# Verona Pharma



## Breathtaking science

Developing respiratory drugs to  
improve health and quality of life



**Cowen Health Care Conference**  
**Boston, MA**  
**March 2019**

**Nasdaq VRNA**  
**[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 of its product candidate, 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.

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## Verona Pharma

First-in-class product candidate for respiratory disease  
Effective and well tolerated in 13 Ph1 and 2 trials

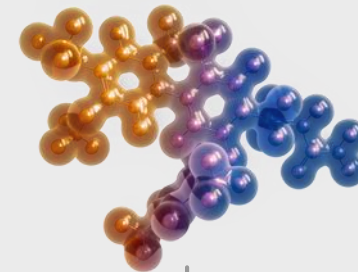
### COPD

Chronic Obstructive Pulmonary Disease



In US: 2 million uncontrolled patients  
despite current treatment

### Novel Drug Candidate Ensifentrine (RPL554)



Inhaled PDE<sub>3</sub>  
and PDE<sub>4</sub>  
Inhibitor

Bronchodilator **AND** Anti-inflammatory Agent  
... in a Single Compound

FDA EoP2 meeting planned 1Q20 - Ph3 program 18-24 months

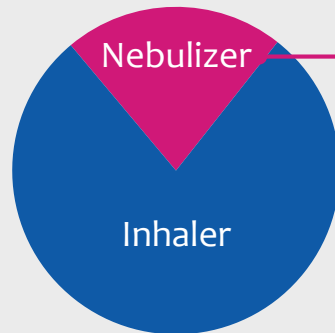


# Verona Pharma

## Ensifentrine - Substantial Commercial Opportunity in COPD

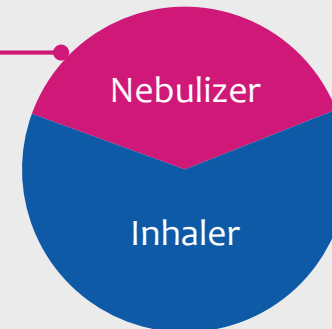
Add-on to Single Bronchodilator

US est. \$5-6B\*



Add on to Double/Triple Therapy

US est. ~\$2-4B\*



Attractive Medicare Part B Reimbursement

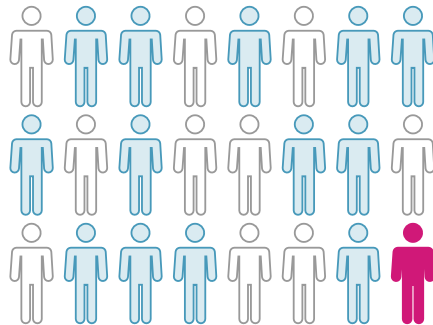
In US: 2 million patients need additional therapy

	Nebulizer	DPI/MDI
Est. % of Patients	10-20%	80-90%
Est. Total Patients	280,000	1.72M
Avg. Annual WAC Price	\$12,000	\$4,800

# COPD: The Silent Epidemic

## Living with It

24M in US alone



16M  
Diagnosed

2M  
Severe/  
very severe

## In the Workplace

- 70% of COPD sufferers work
- 2<sup>nd</sup> leading cause of disability

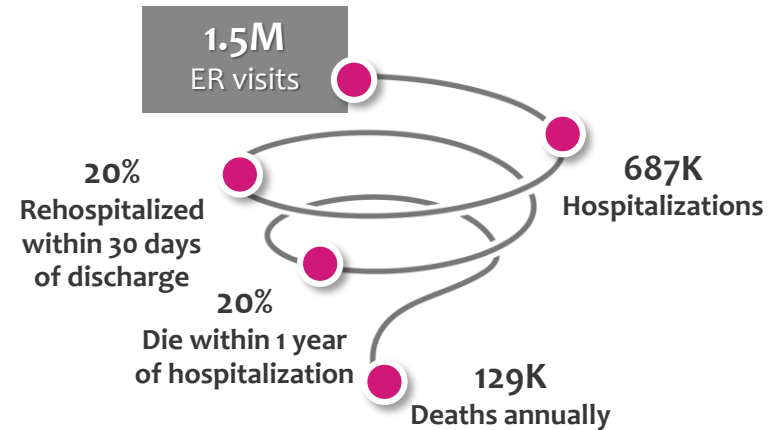
Cost

\$50B/year

Indirect & Direct

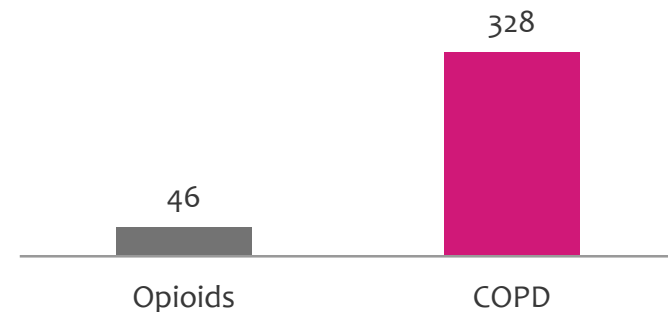
Sources: COPD Foundation; US only

## Dying from It



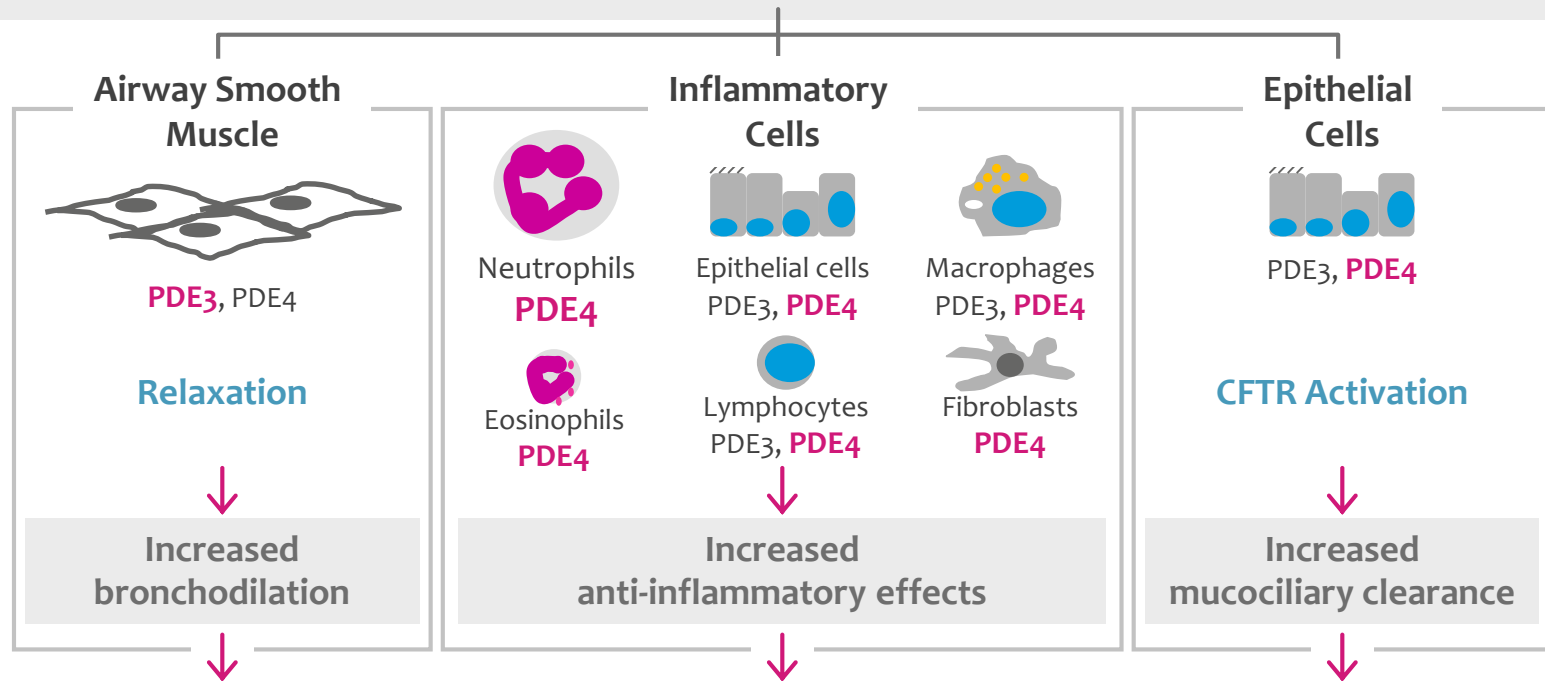
## 3<sup>rd</sup> Largest Chronic Disease Killer

Deaths/Day



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

## Ensifentrine: Dual PDE3 and PDE4 Enzyme Inhibitor

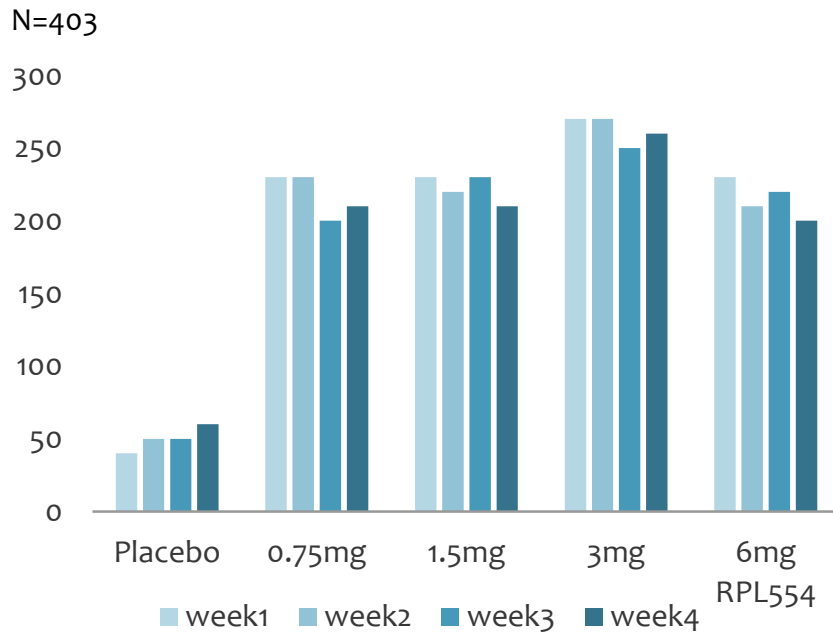


Improved lung function, symptom reduction and improved quality of life

# 4 Week Phase 2b: Rapidly Improved Lung Function and Progressive Symptom Relief as Single Bronchodilator

## Lung Function

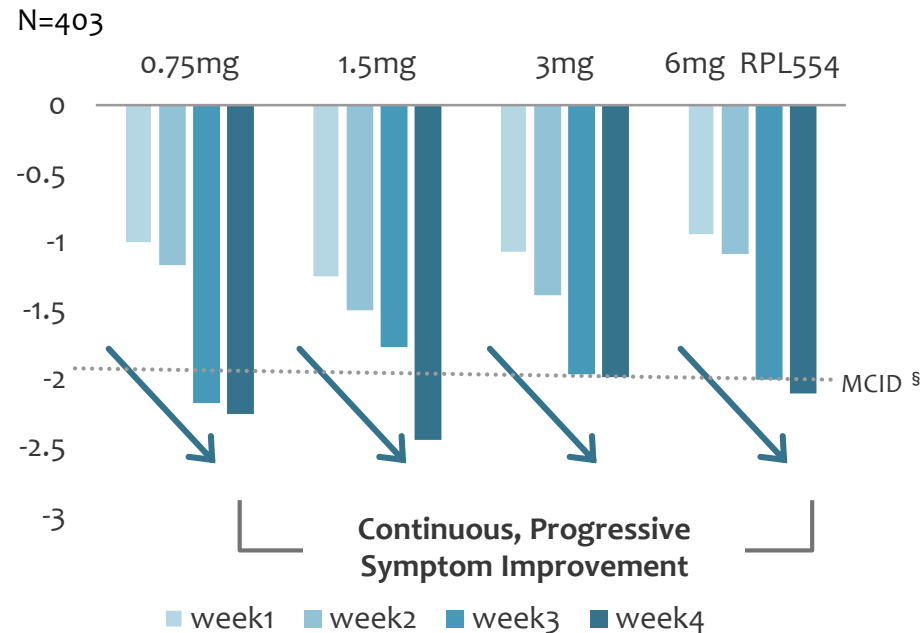
Peak Change FEV<sub>1</sub> (mL) (p<0.001)\*



\*Peak Change from Day 1 in Baseline in FEV<sub>1</sub> (mL) on Day 28, Week 4, Primary endpoint was met; ensifentrine only bronchodilator in these patients

## Symptom Relief

Total Score E-RS: COPD by Week, p<0.02\*\*

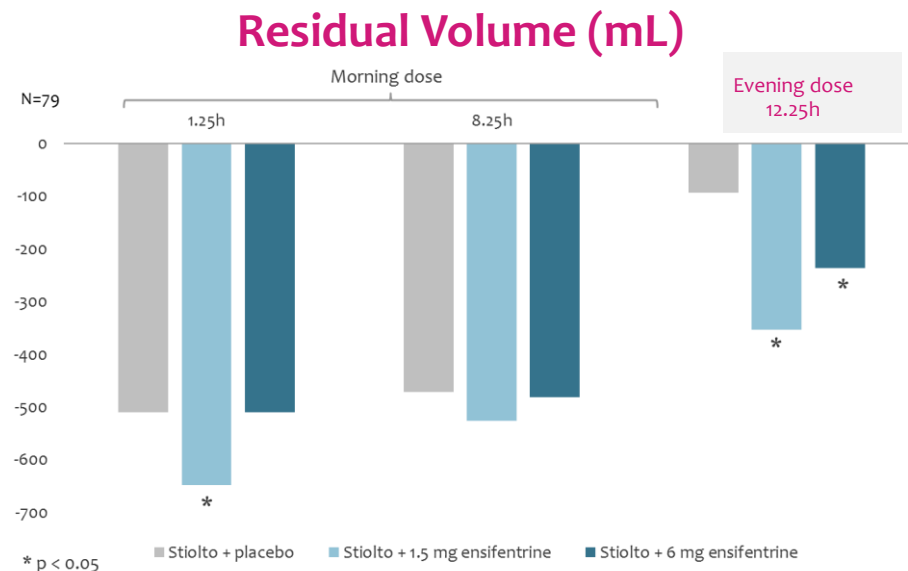
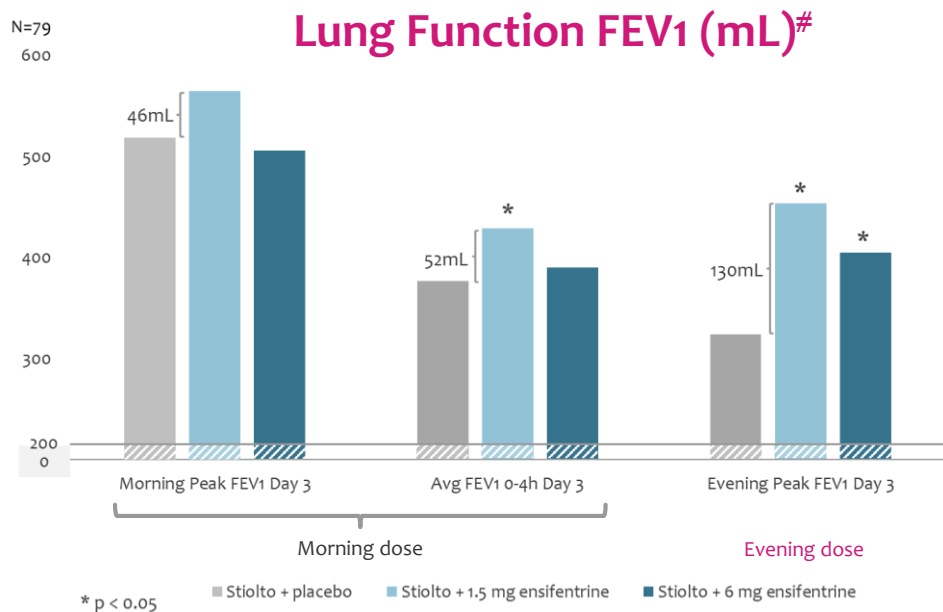


\*\* Placebo corrected

§ Minimal clinically important difference

**Bronchodilation and anti-inflammatory effect improved lung function and relieved symptoms - may potentially lead to reduction in COPD exacerbations**

# Phase 2: Improvement in Both FEV<sub>1</sub> and Residual Volume on Day 3 When Inhaled on Top of Two Bronchodilators



<sup>#</sup> FEV<sub>1</sub> (mL) Change from Baseline on Day 3  
 Day 3 Peak FEV<sub>1</sub>, primary endpoint was not statistically significant

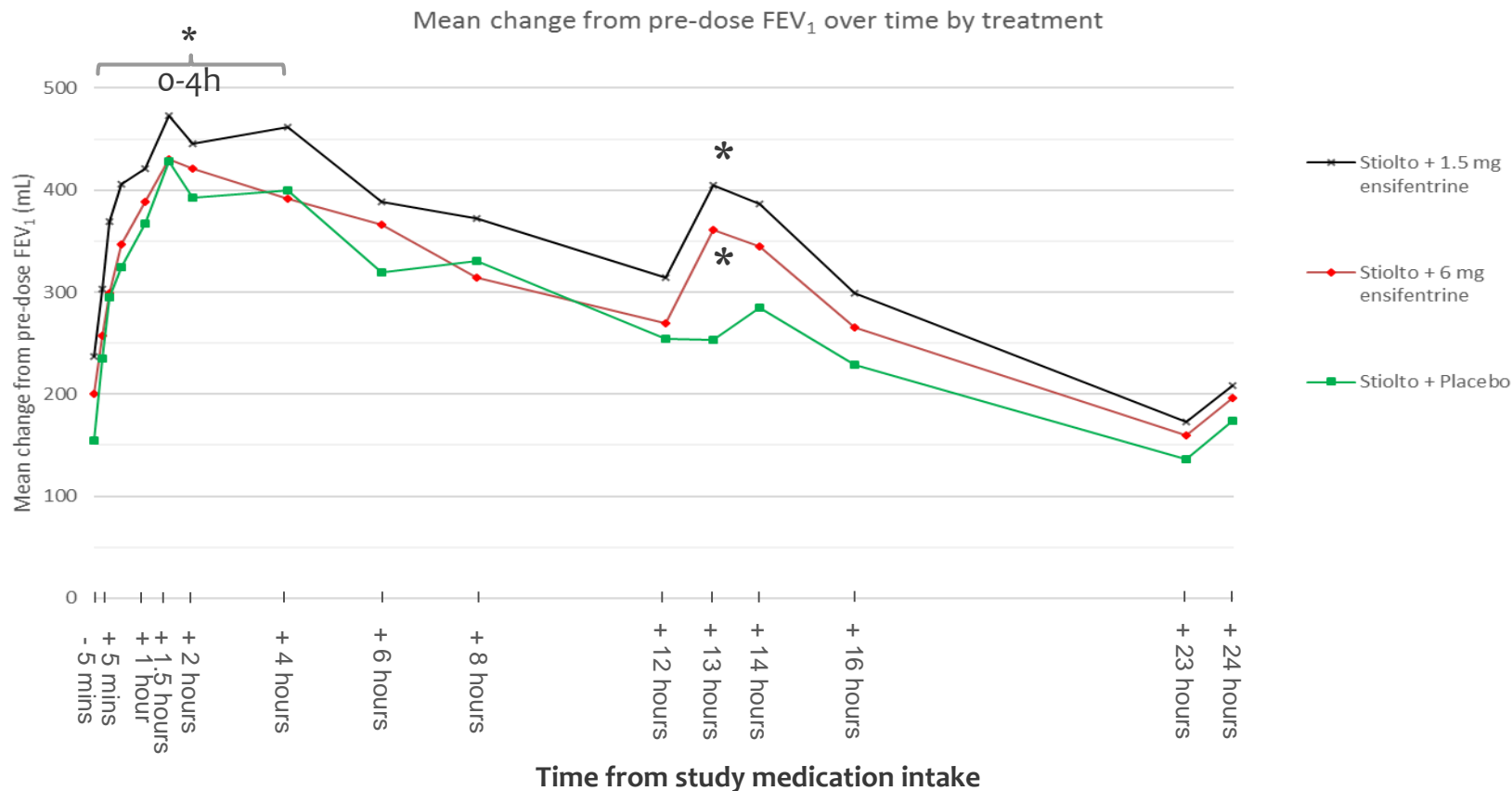
28% of patients used triple therapy (LAMA, LABA, ICS)

Further **improved bronchodilation** and **reduction in residual volume** (air trapping)  
 - may lead to **symptom improvement** in patients already on dual and triple therapy



# Phase 2, Day 3: 24-hour Spirometry (FEV<sub>1</sub>) Profile when Added-on to Dual/Triple Therapy

N=79



~50 to 130 mL improvement in FEV<sub>1</sub> with 1.5 mg dose over Stiolto<sup>®</sup> over 24h

# Nebulized Ensifentrine: Systematic Phase 2 Program Informing Phase 3 Design

Focus on well-established regulatory endpoints



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Establish activity + profile in Ph2 → A. Potential Pivotal studies: Design and endpoints based on Ph2 to increase chance of positive outcome

Standalone  
(Dose Ranging)  
400 pts

Bronchodilator + anti-inflammatory  
Completed 2018

Add-on to  
Single Therapy  
(2 Ind. P2 Studies)

Bronchodilator  
Completed 2017

Add-on to  
Single Therapy  
(Dose Ranging)

Bronchodilator +  
anti-inflammatory\*

Add-on to  
Double/Triple  
Therapy

Bronchodilator  
Completed Jan 2019

A. Potential Pivotal studies: Design and endpoints based on Ph2 to increase chance of positive outcome

1 x study, 6 mo duration  
1 x study 6 mo duration w. 6 mo safety extension

-  
None or single bronchodilator  
background

-  
Lung function (FEV<sub>1</sub>), symptom improvement,  
explore exacerbations in pooled data

B. Planned positioning study for physicians and payors

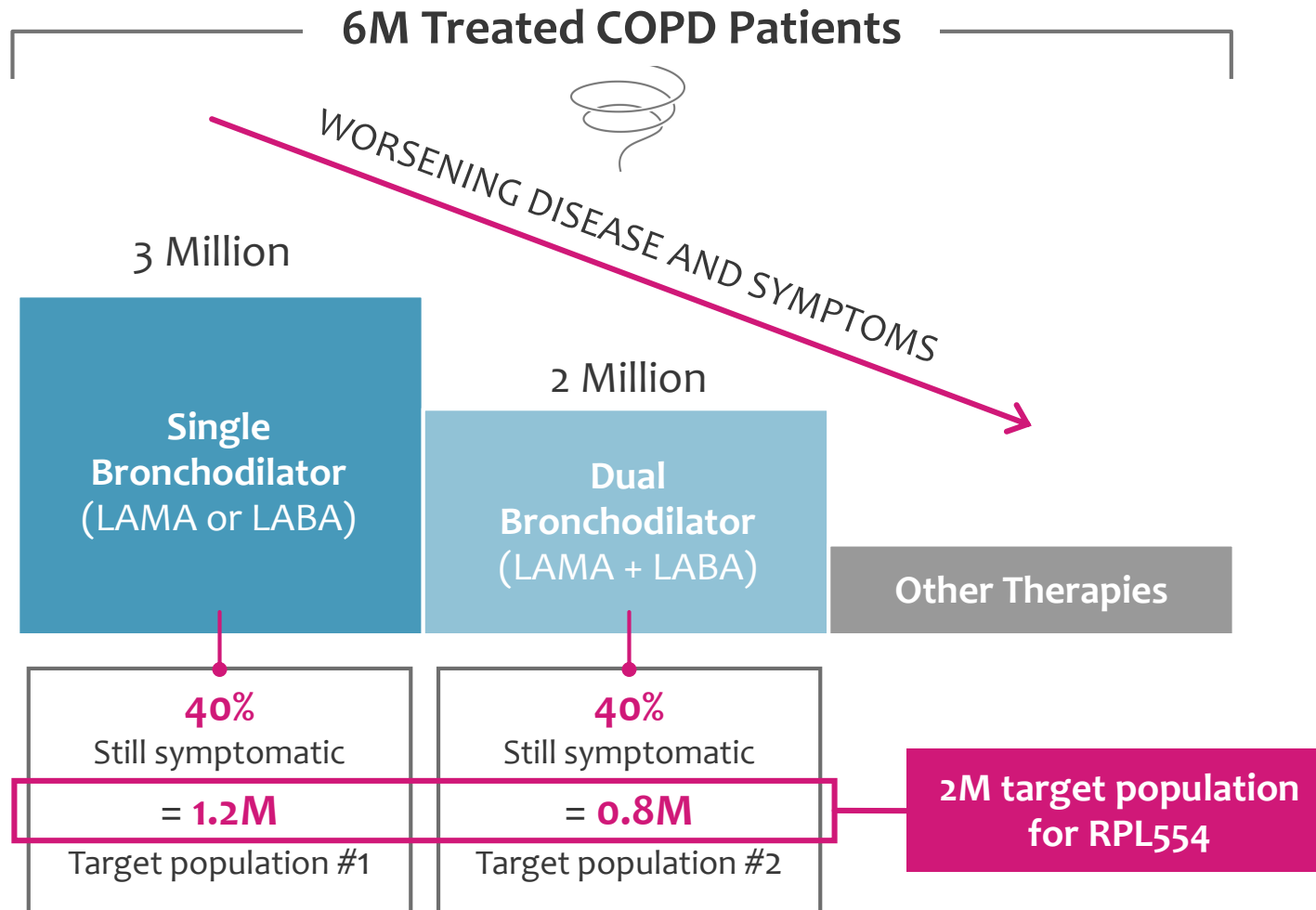
Add-on treatment to single and  
dual bronchodilators in COPD

End of Phase 2 Meeting  
with FDA, target 1Q 2020

\*Expected to begin in 2Q 2019, results expected in 4Q 2019



# Opportunity for Ensifentrine: 40% of US COPD Patients Symptomatic Despite Current Treatment



1. Mahler et al., Eur Respir J. 2014. 2. Bateman et al., Eur Respir J. 2013 Dec

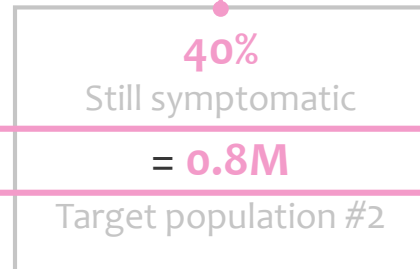
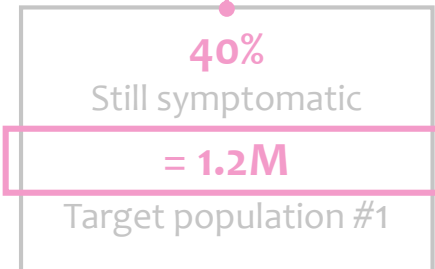
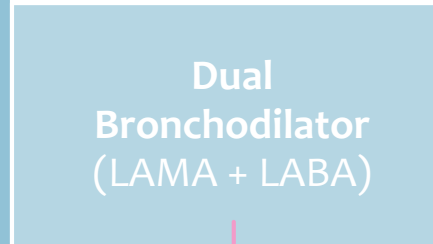
3. Mullerova H et al., American Journal of Respiratory and Critical Care Medicine . 4. Vestbo J, et al., The Lancet, Vol 389, p. 1919-1929; May 13, 2017

# Substantial, Addressable US Commercial Opportunity for Nebulized Ensifentrine

3 Million



2 Million



2M target population for ensifentrine

Nebulizer use

Nebulizer use

Est. % of Patients	10%	20%
Est. Total Patients	120,000	160,000
Avg. Annual WAC Price	\$12,000	\$12,000

280,000 target for nebulized ensifentrine

Attractive Medicare Part B Reimbursement

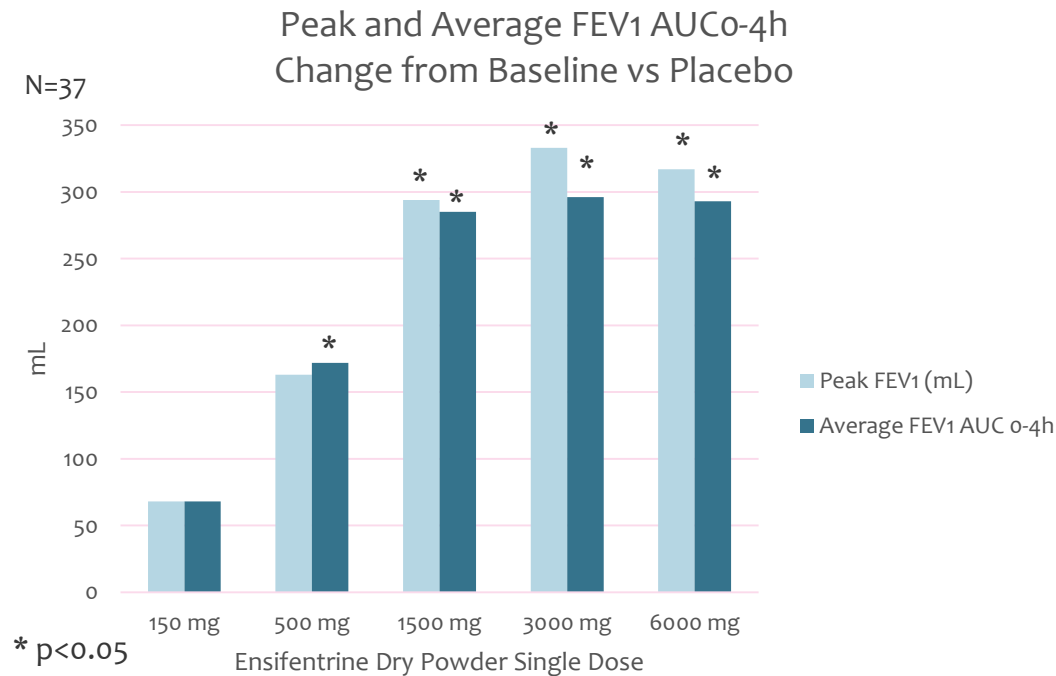
# Positive Interim Phase 2 Data with Ensifentrine Dry Powder Inhaler Formulation in COPD

Data from first of two-part clinical trial



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Dose-dependent, significant and clinically meaningful bronchodilator response



## Inhaler usage for maintenance therapy (US estimates)

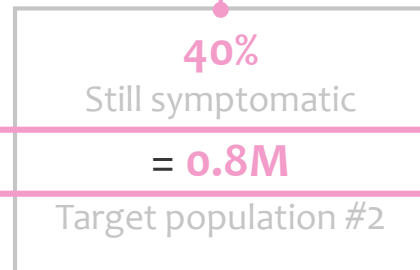
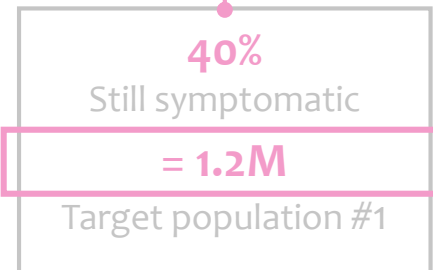
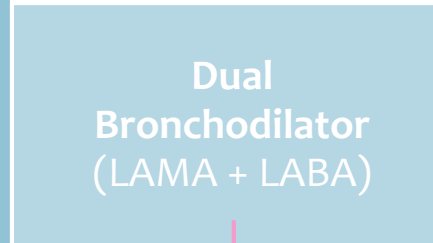
- ~80-90% mild to very severe COPD patients ~ 5.5 million
- **DPI formulation could dramatically expand clinical utility and commercial potential**

# DPI Ensifentrine: Potential to Substantially Expand US Commercial Opportunity

3 Million



2 Million



2M target population for ensifentrine

	Nebulizer		Inhaler use	
Est. % of Patients	10%	90%	20%	80%
Est. Total Patients	120,000	1.08M	160,000	0.64M
Avg. Annual WAC Price	\$12,000	\$4,800	\$12,000	\$4,800

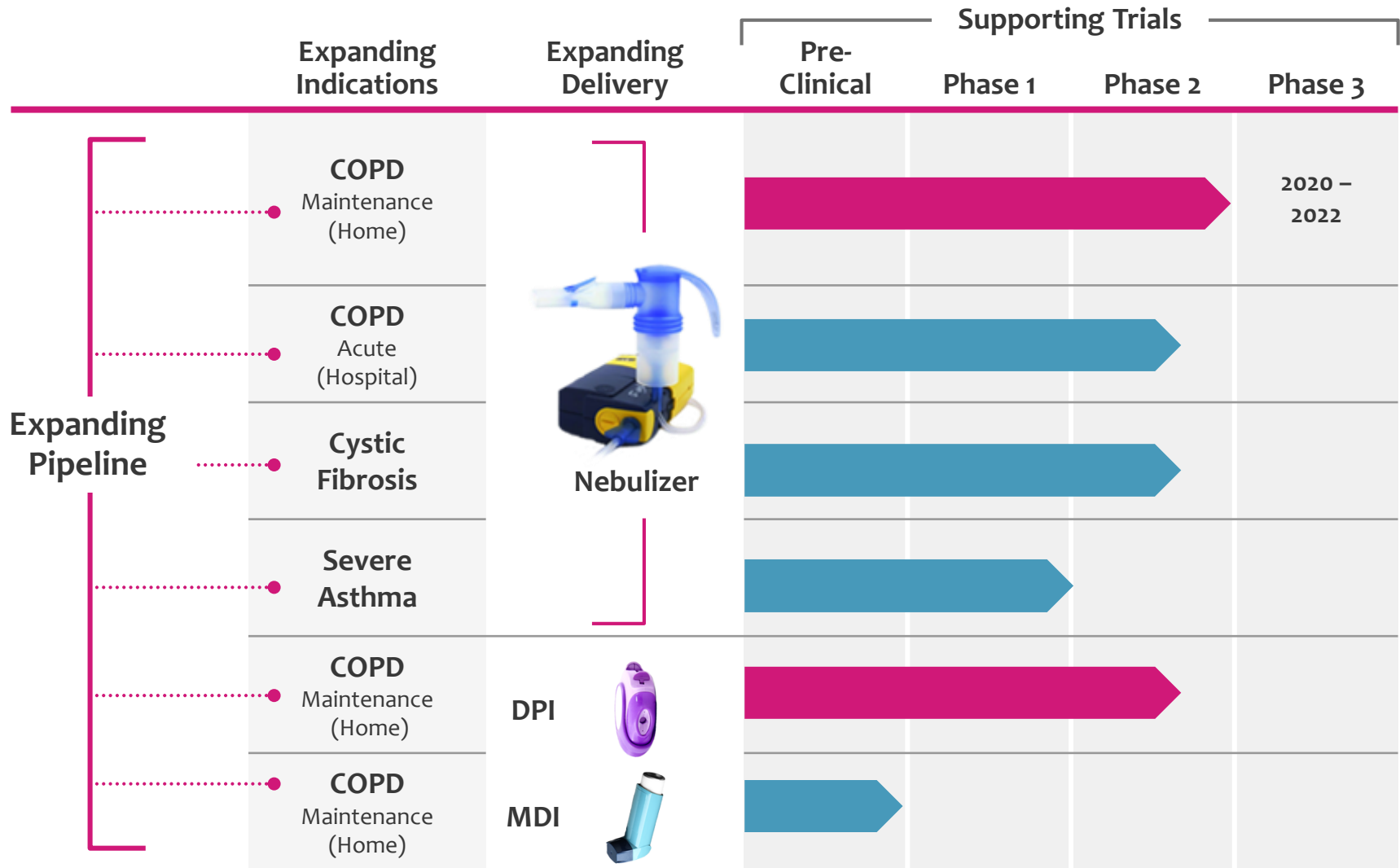
280,000 target for nebulized ensifentrine

1.72 Million target for DPI/MDI ensifentrine

# Ensifentrine Lifecycle: Expanding The Pipeline Over Time



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# Cystic Fibrosis: A Devastating Orphan Disease

## *Ensifentrine: Favorable PK and PD Profile in CF Patients*



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- 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
  - Designed to reduce airway obstruction and inhibit inflammation

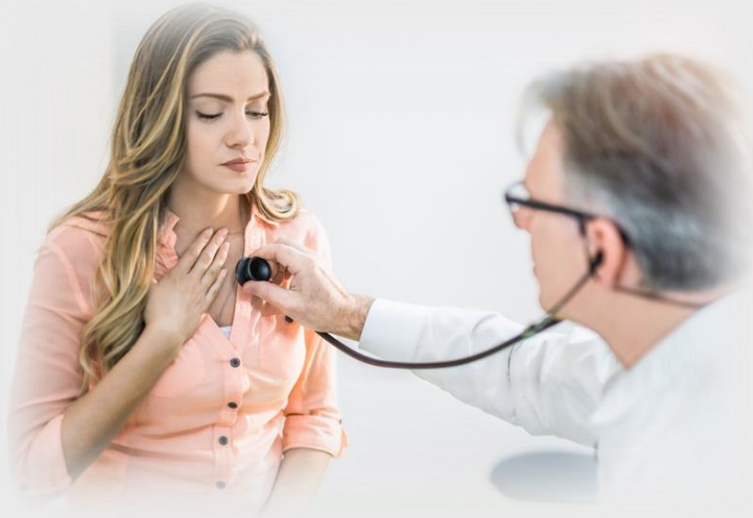


**Pre-clinical Studies Completed and Phase 2a Study Data Reported in March 2018**





# Ensifentrine: Potential in Asthma



- Potential to be an effective bronchodilator in asthma patients
- Clear dose-response relationship and well tolerated in asthmatics in Phase 2a clinical study
- Little effect on heart rate and plasma potassium levels compared to nebulized albuterol

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## Potential Positioning

- Severe asthma, before start of treatment with biologics
- Steroid-sparing

## Potential Device

DPI or MDI inhaler device may be more convenient for asthma patients



# Ensifentrine: Long Patent Runway (until mid-2030s)

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## Robust Patent Portfolio

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- Composition of Matter – granted US, EU, Japan, other; expires 2020
  - Polymorph – granted US, EU, Japan, other; expires 2031
  - Formulations – granted US, EU, other; expires 2035
  - Manufacturing, use, salt forms, combinations: granted and pending in US, EU, and other territories; expires 2031 – 2037
  - Additional IP opportunities being explored
- 

## New Chemical Entity

- US: Market exclusivity up to 5 years post NDA approval
- EU: Market & Data exclusivity up to 10 years post Marketing Authorization

**Verona Pharma has Global Rights**



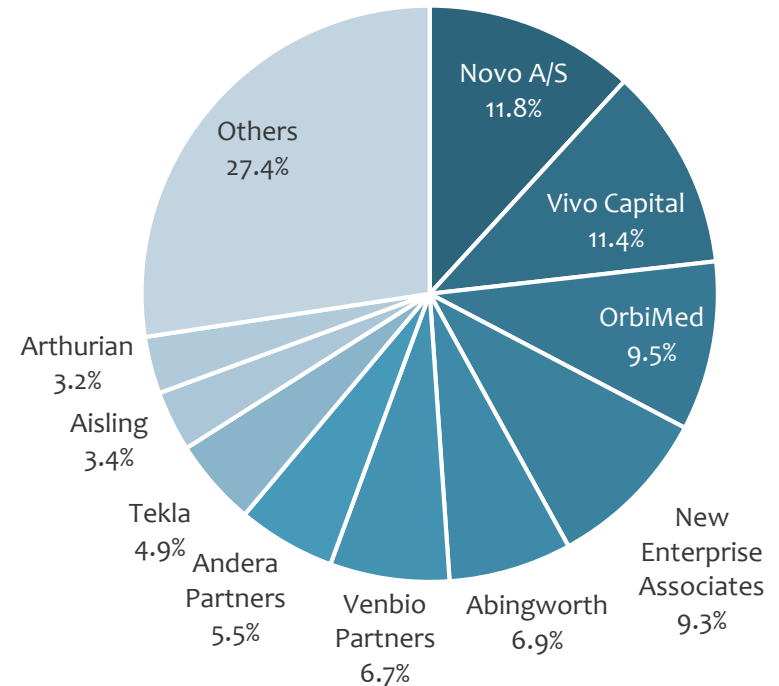


# Well Financed with Major Healthcare Investors

## Financial Overview December 31, 2018

Cash and Cash Equivalents	\$82.6M <sup>1</sup>
Operating Expenses FY18	\$32.7M <sup>1</sup>
Market cap	\$73.7M <sup>2</sup>

## Shareholdings<sup>3</sup>



<sup>1</sup>Exchange rate used (US dollars per pound sterling): December 31, 2018: \$1.2763

<sup>2</sup>Current issued 105.3M shares or 13.2m ADSs, share price \$5.60 on February 28, 2019

<sup>3</sup>As disclosed to the Company in accordance with AIM Rule 26, or through s80 notices and 13F and 13G filings



# 2019: Potential for Multiple Value Inflection Points as Ensifentrine Advances Towards Phase 3

**Nebulized Ensifentrine as Maintenance Treatment of COPD**

Top-line Phase 2 data: RPL554 as add-on to LAMA/LABA

Data from 4 week Phase 2 dose ranging as add-on to single therapy

FDA: EOP2 meeting

Advancing into Phase 3 trials



**Inhaler Ensifentrine as Maintenance Treatment of COPD**

Initial Phase 2 data: DPI studies (single dose)

Top-line Phase 2 data: DPI (multi-dose)

Top-line Phase 2 data: MDI (single dose)



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**Thank you**