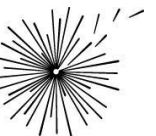




# Developing therapies to improve lung function and symptoms

April 2020

Nasdaq: VRNA | AIM:VRP | [www.veronapharma.com](http://www.veronapharma.com)



**Verona Pharma**

# 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, 2020, 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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# Ensifentrine is a late-stage, first-in-class candidate for unmet respiratory needs

Inhaled PDE3 and PDE4 inhibitor

## Large COPD opportunity:

- Total US sales of \$9.6 billion chronic maintenance COPD therapies<sup>1</sup>
- 1.2M US COPD patients failing despite maximum therapy<sup>2,3</sup>
- About 7,000 physicians prescribe 70% of US nebulized prescriptions<sup>4</sup>

## Unique profile:

- First novel class of bronchodilator in COPD in over 40 years<sup>5</sup>
- Results from 15 clinical trials, including two Phase 2b trials
- Safety profile similar to placebo in trials involving over 1300 subjects

## Pathway to approval:

- Well-validated demonstrated path to US FDA approval
- Expert team has developed and commercialized many leading respiratory products

**Bronchodilator  
and  
anti-inflammatory  
activity**

# Ensifentrine: Differentiated profile as dual bronchodilator and anti-inflammatory

Ensifentrine impacts 3 key mechanisms in respiratory disease

## Airway Smooth Muscle<sup>1-4</sup>



PDE3, PDE4



↑ cAMP

Bronchial relaxation

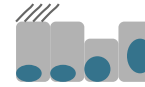


**bronchodilation**

## Inflammatory Cells<sup>5,6</sup>



Neutrophils  
PDE4



Epithelial cells  
PDE3, PDE4



Macrophages  
PDE3, PDE4



Eosinophils  
PDE4



Lymphocytes  
PDE3, PDE4



Fibroblasts  
PDE4

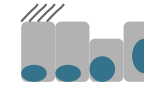
↑ cAMP

↓ Cell proliferation  
survival & activity



**anti-inflammatory effects**

## Epithelial Cells<sup>7,8</sup>



PDE3, PDE4



↑ cAMP  
↑ CFTR activation  
↑ Ciliary function



**mucociliary clearance**

# Ensifentrine improves lung function and symptoms in moderate to severe COPD patients

Improvements shown with or without background therapy

## Summary of Phase 2b data:

- **Lung function:** Statistically significant and clinically meaningful improvements with optimal efficacy observed consistently with the 3 mg dose
- **Symptoms:** Statistically significant and clinically meaningful improvements in symptoms and Quality of Life measures
- **Twice-daily:** Statistically significant and clinically meaningful improvements in average FEV<sub>1</sub> over 12 hours

## Summary of Phase 1 and 2a data:

- **Anti-inflammatory:** Significant reduction in all inflammatory cell types in sputum in LPS challenged healthy subjects (COPD-like inflammation)
- **Lung function and volumes:** Improved when added to background dual/triple therapy

**Well tolerated  
in 15 clinical  
trials in over  
1300 subjects**

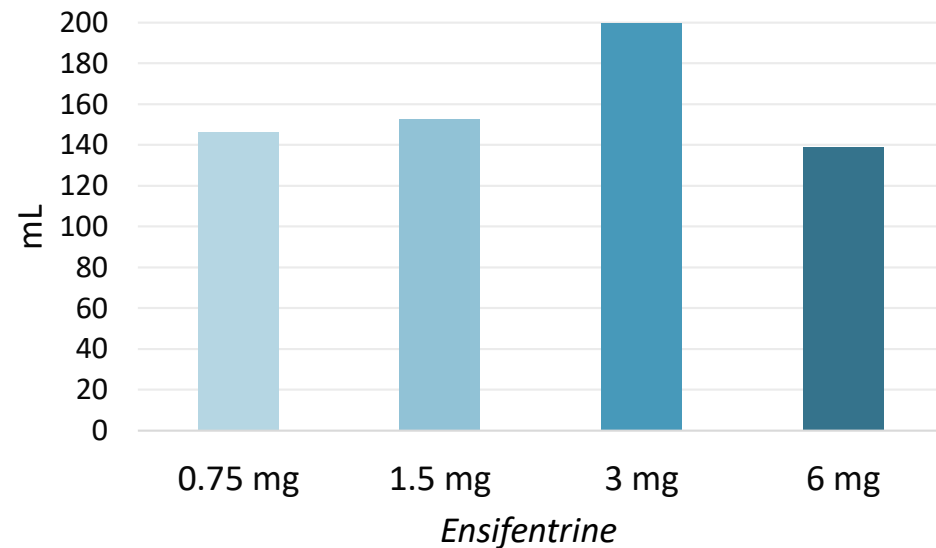
# Ensifentrine monotherapy rapidly improved lung function

## Progressive symptom relief in COPD

### Lung function

Peak Change FEV<sub>1</sub> (mL), p<0.001\* at Week 4

N=403

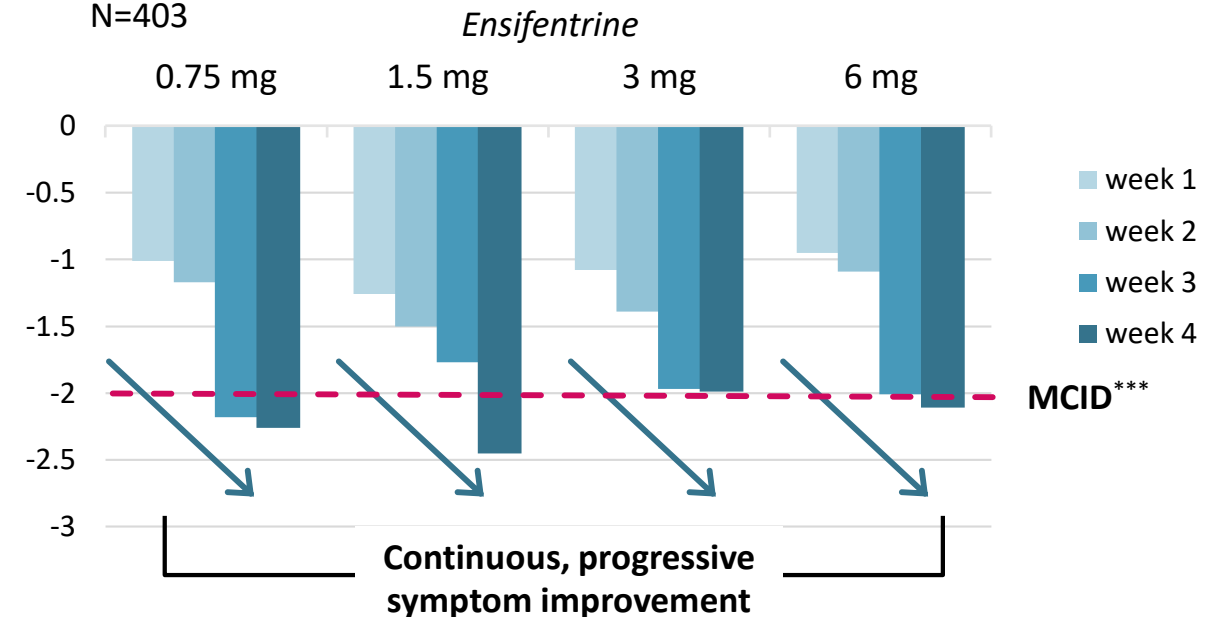


\*Peak Change from Day 1 in Baseline in FEV<sub>1</sub> (mL) on Day 28, Week 4, Primary endpoint met; placebo corrected

### Symptom relief

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

N=403



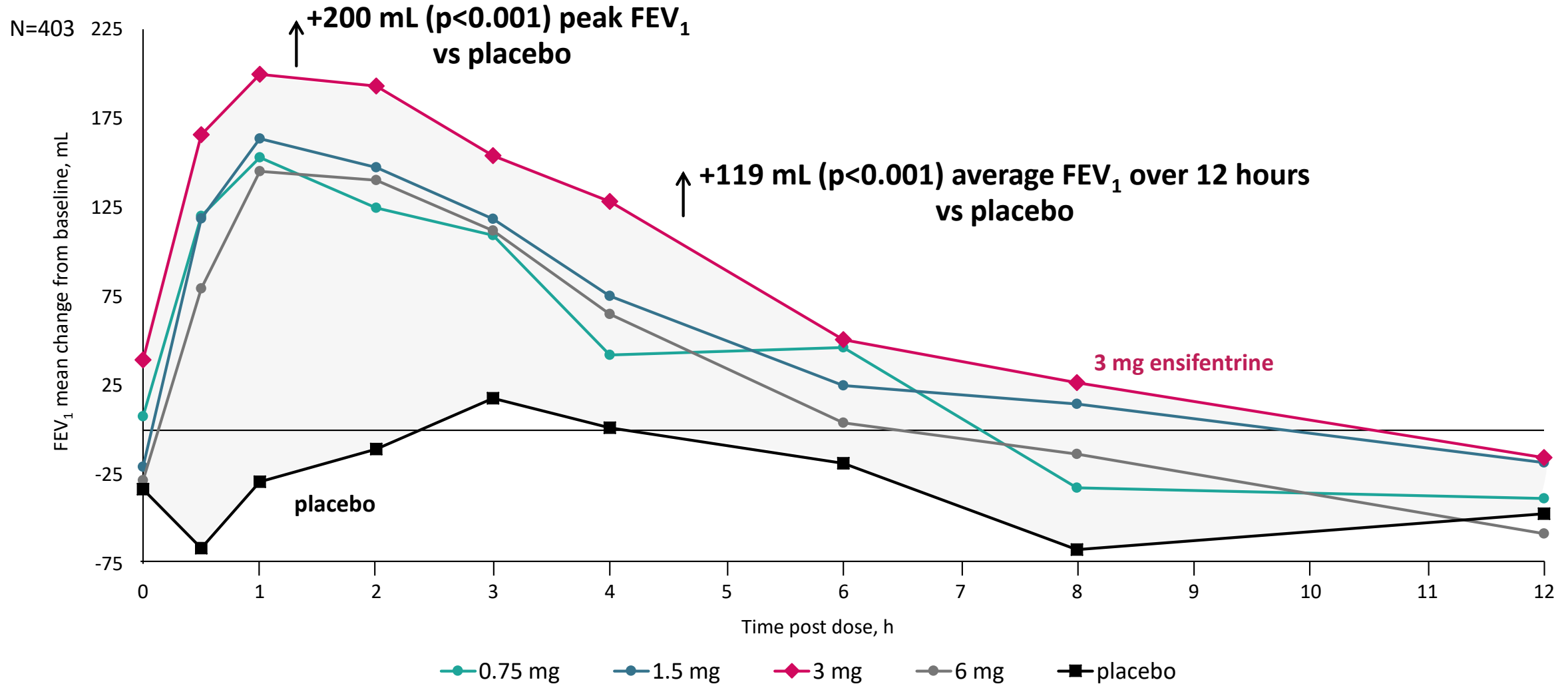
\*\* Placebo corrected

\*\*\* Minimal clinically important difference

**Bronchodilator + anti-inflammatory  
potential to reduce symptoms and exacerbations<sup>1</sup>**

# Ensifentrine monotherapy supports twice daily dosing

## 12-Hour Spirometry Profile at Week 4





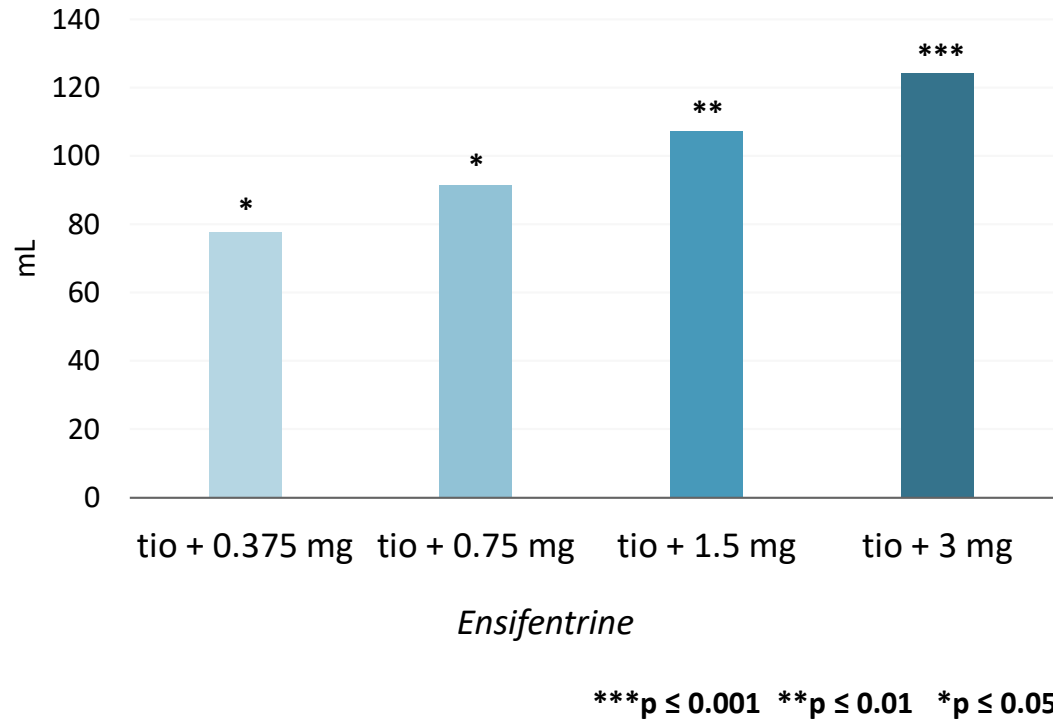
# Ensifentrine added to tiotropium rapidly improved lung function

## Progressive improvement in quality of life in COPD

### Lung function

Peak Change FEV<sub>1</sub> (mL) at Week 4

N=413

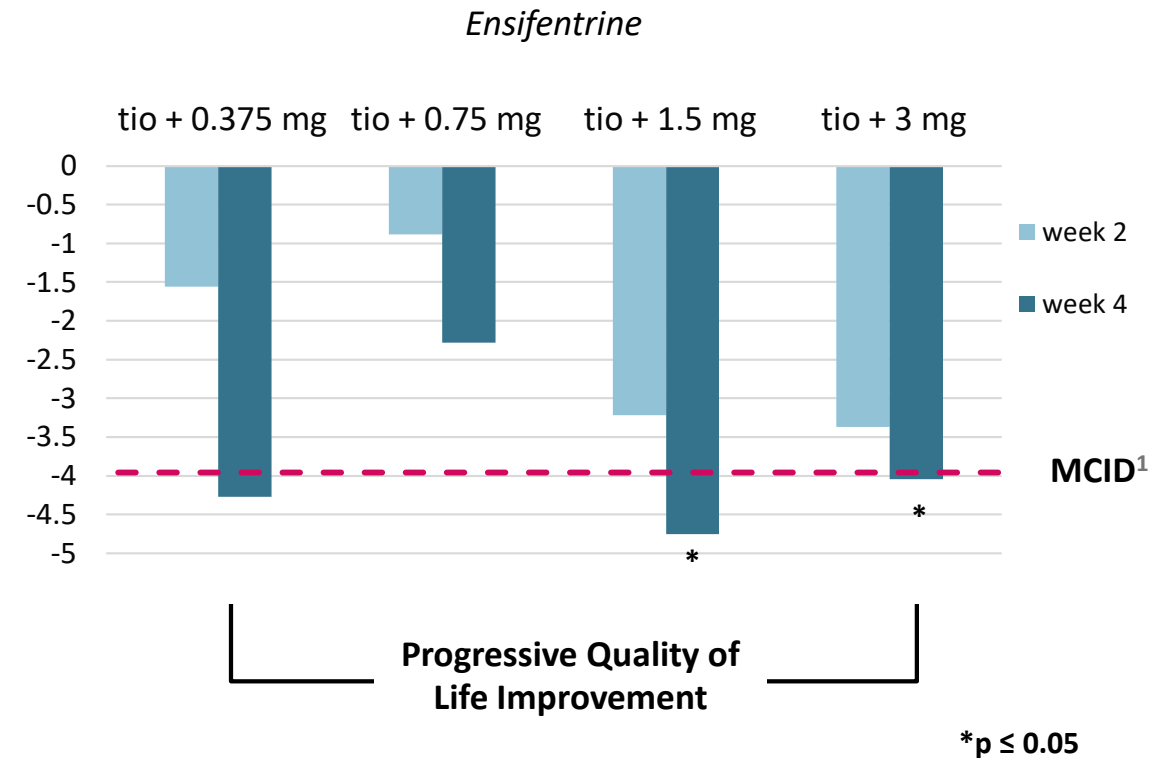


Primary endpoint met; placebo corrected

### Symptom relief

Total Score SGRQ-C: COPD by Week

N=413



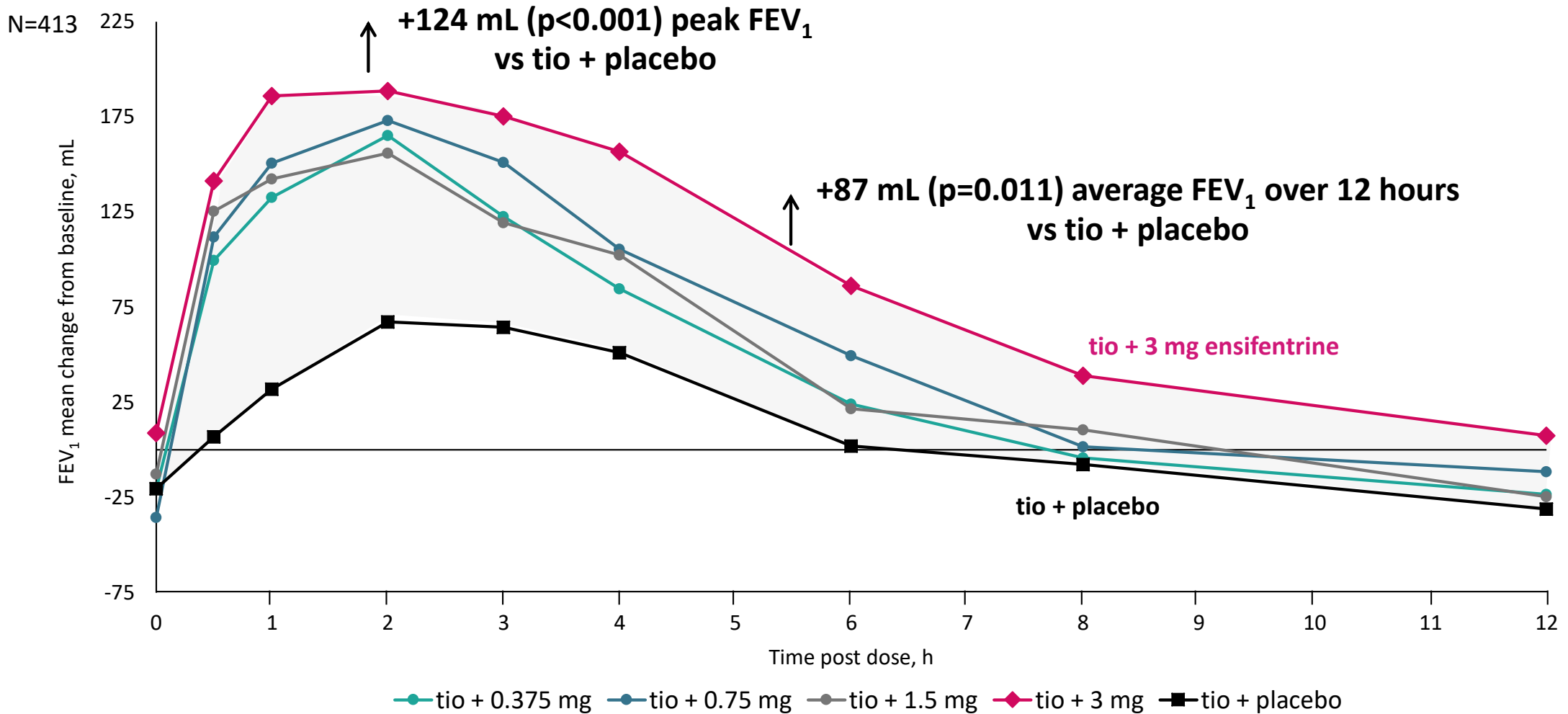
Placebo corrected

<sup>1</sup>Minimal clinically important difference (-4 units)



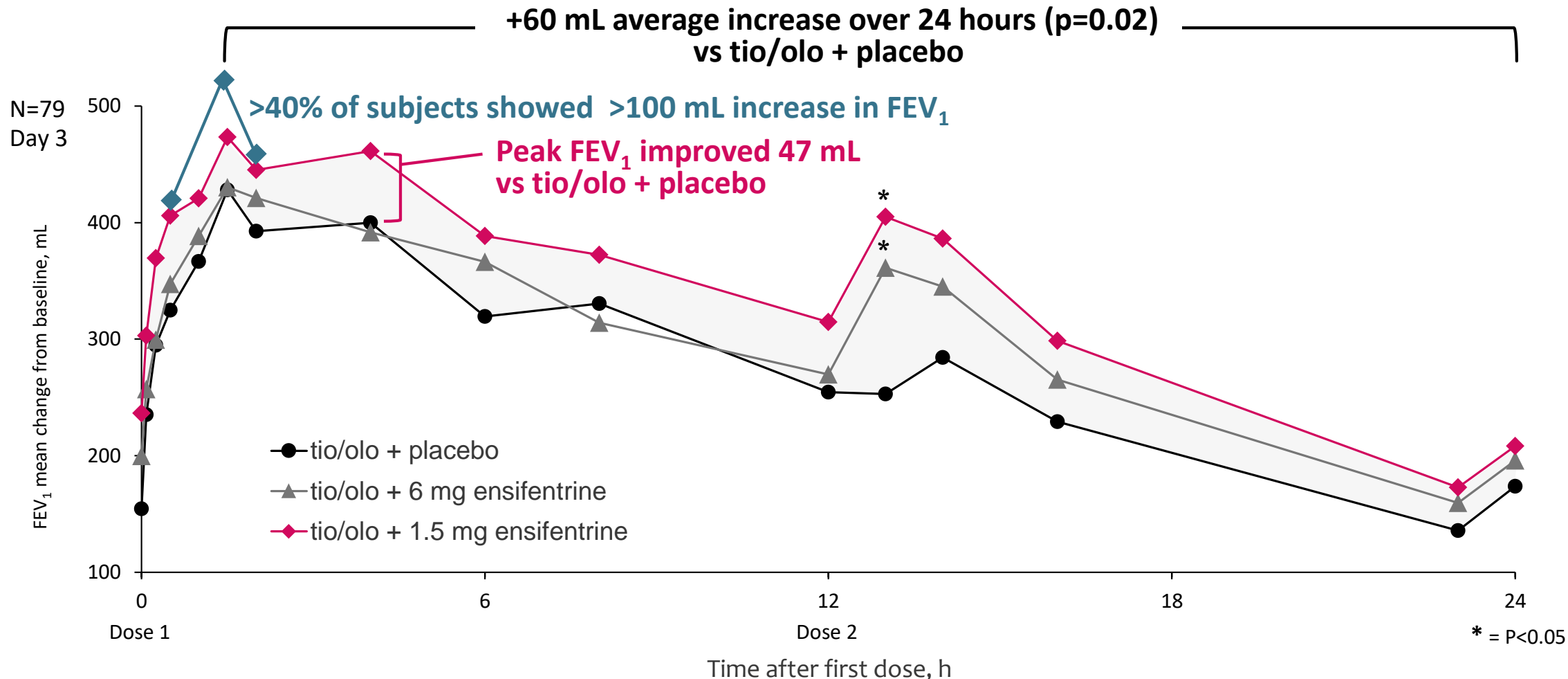
# Ensifentrine added to tiotropium supports twice daily dosing

## 12-Hour Spirometry Profile at Week 4



# Ensifentrine in addition to dual therapy

## Additional lung function improvement



The primary endpoint of morning peak FEV<sub>1</sub> was not statistically significant in this study; 29% of patients received triple therapy  
RPL554-CO-204 Full Phase 2a Analysis Set, data on file; Dual therapy = tiotropium/olodaterol (Stiolto®/Spiolto®)



Verona Pharma

# COPD:

A global silent epidemic

**3rd leading cause of death by 2030 with 384 million patients worldwide<sup>1,2</sup>**

## Breathless

Millions of patients remain symptomatic despite maximum treatment<sup>3-6</sup>

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## Progressive deterioration

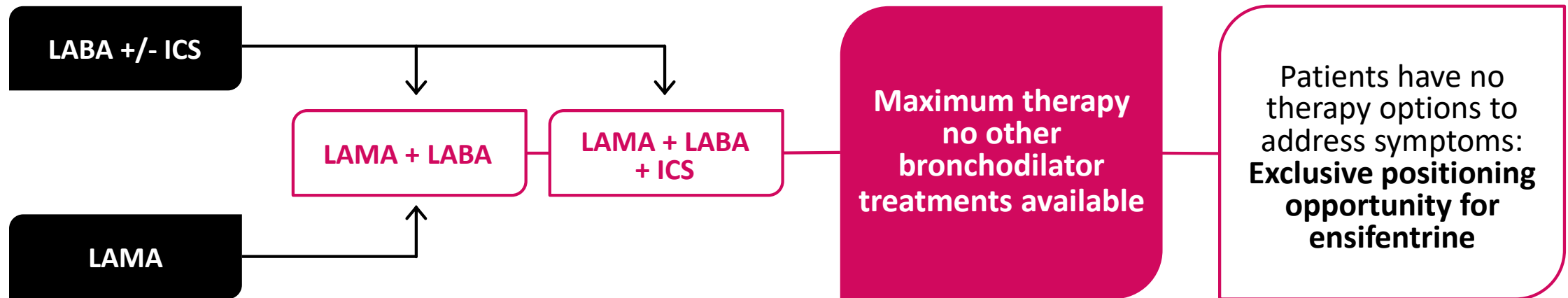
Loss of lung function, leading to hospitalizations and death

*“When I bend over, I can’t breathe. I can’t unload the dishwasher, or make a bed... I wake up but I can’t move. I am so short of breath.”*

John Linnell, Living with COPD

# Nebulized ensifentrine expected to be the only bronchodilator option as add-on to dual / triple therapy

COPD treatment pathway\*



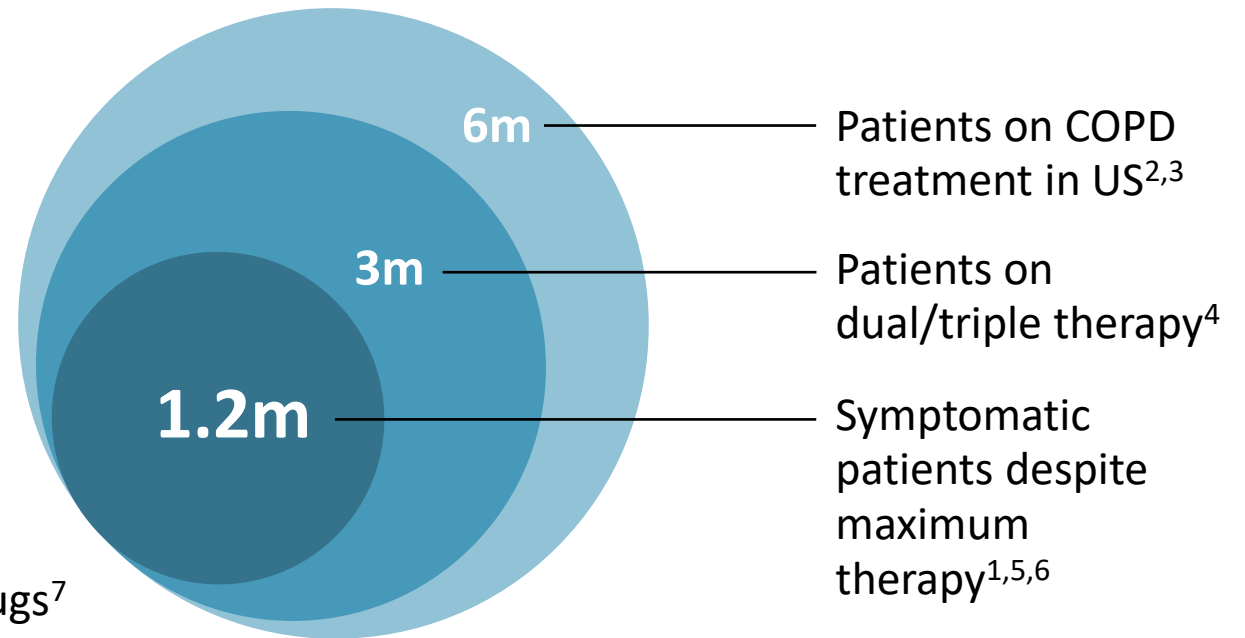
# Nebulized ensifentrine in COPD:

Large US market opportunity

About 40% of COPD patients on dual/triple therapy are uncontrolled, continuing to experience debilitating breathlessness and exacerbations<sup>1</sup>

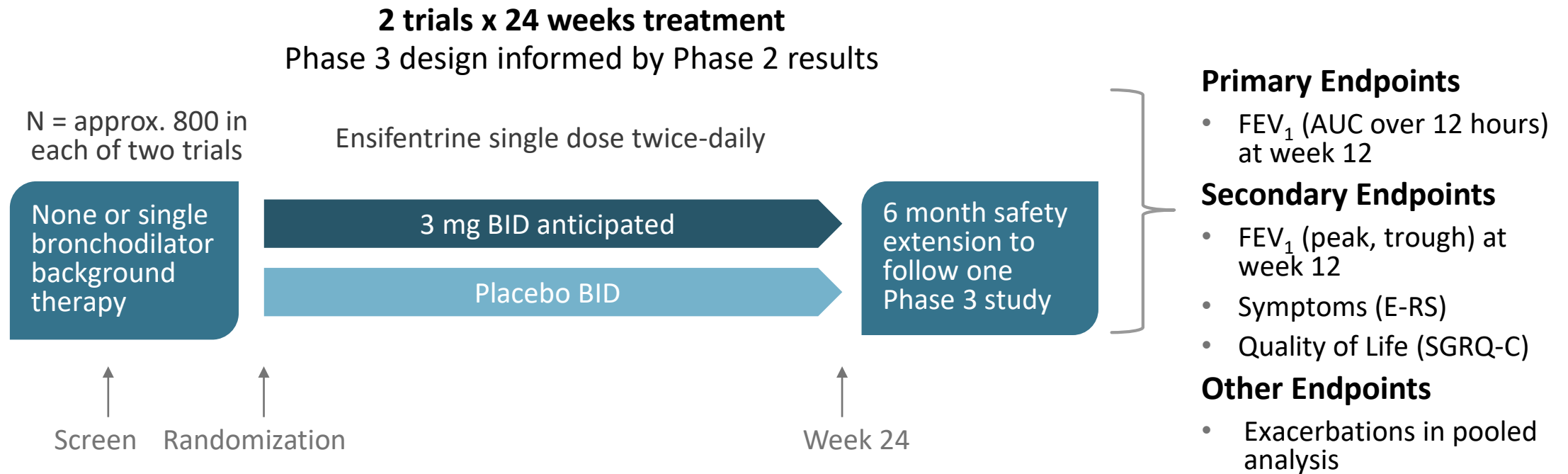
**\$12,000**

Avg. Annual WAC Price of existing nebulized COPD drugs<sup>7</sup>  
Medicare Part B Reimbursement



# Potential Phase 3 Design and Timing

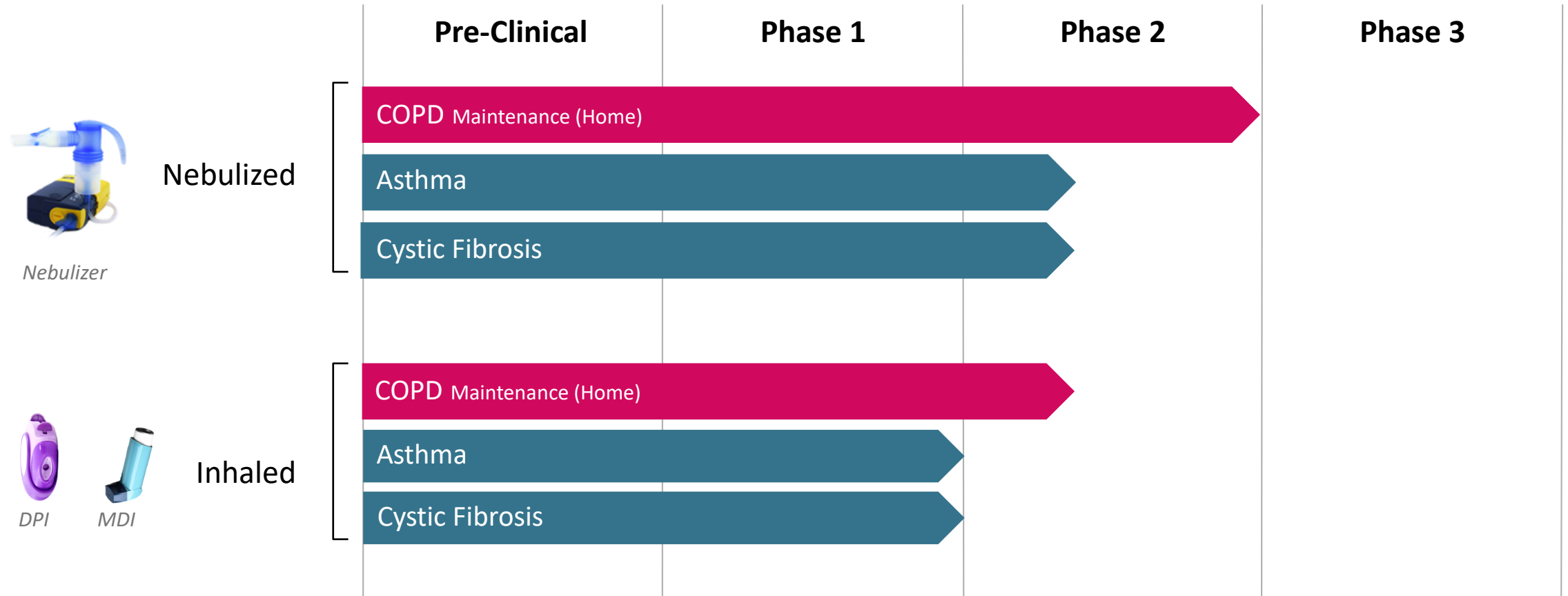
Confirm efficacy and safety of ensifentrine in moderate to severe COPD



**Nebulized ensifentrine as maintenance treatment for COPD**

# Ensifentrine

Multiple indications; multiple dosing forms



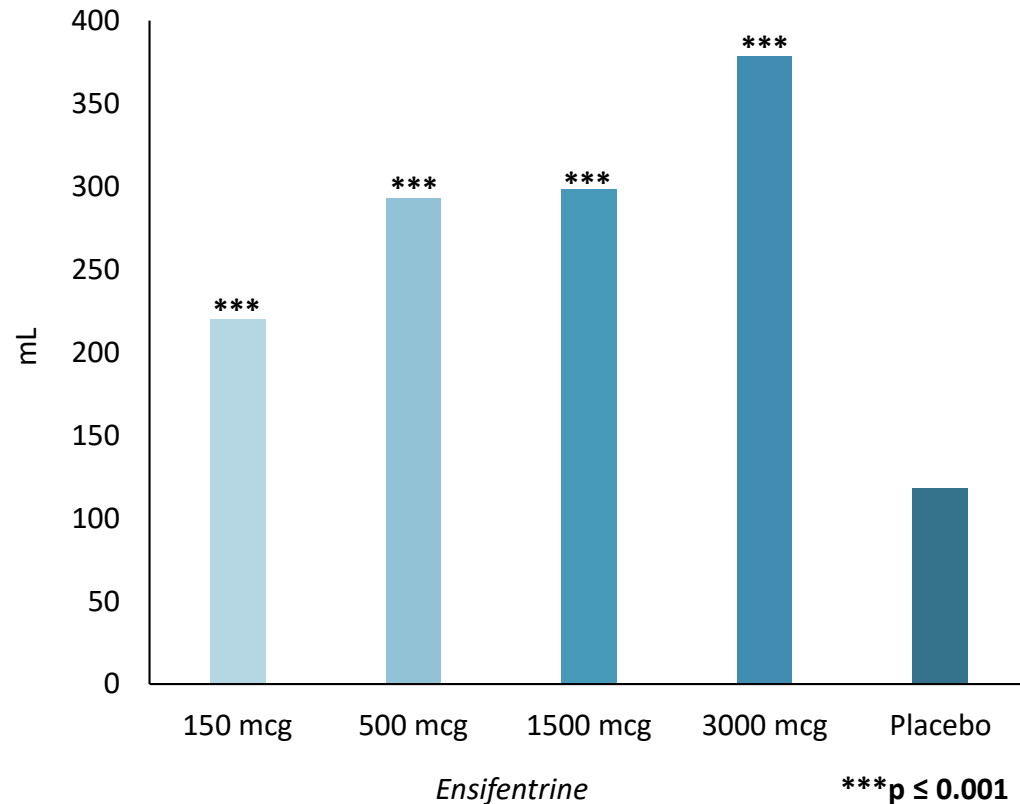


# DPI formulation of ensifentrine improved lung function

Clinically meaningful, statistically significant bronchodilation

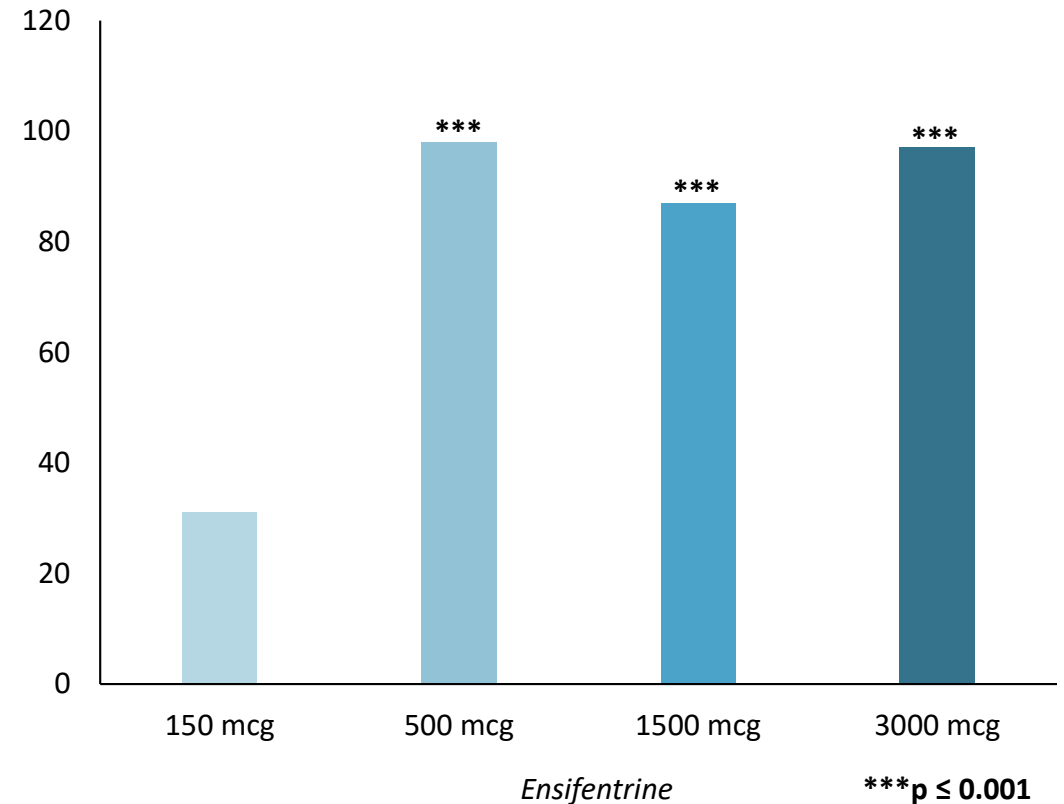
N=32-34

**Primary endpoint: Peak FEV<sub>1</sub> on Day 7**



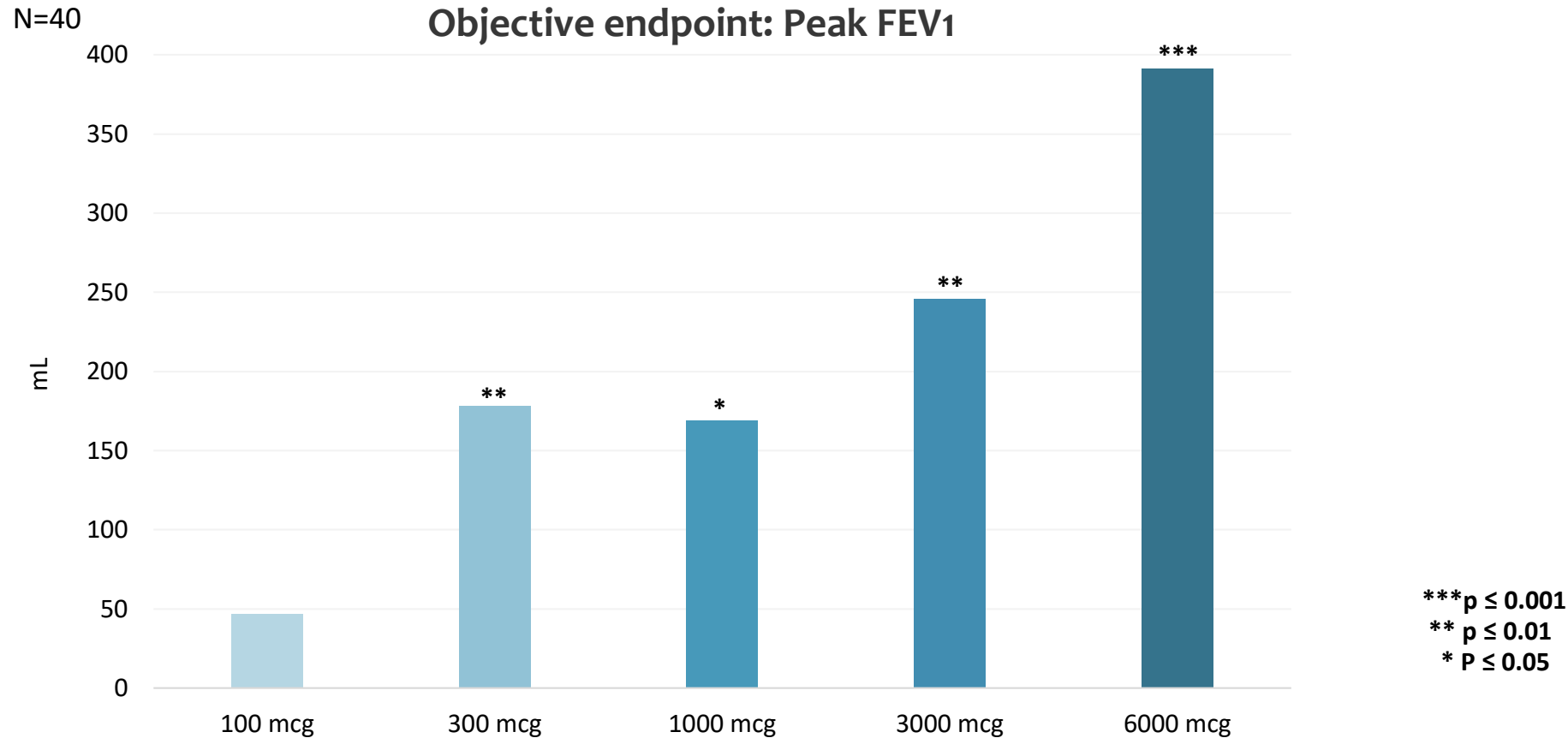
N=32-34

**Morning Trough FEV<sub>1</sub> on Day 7 vs placebo**



# Single dose of MDI formulation of ensifentrine improved lung function

Clinically meaningful, statistically significant bronchodilation

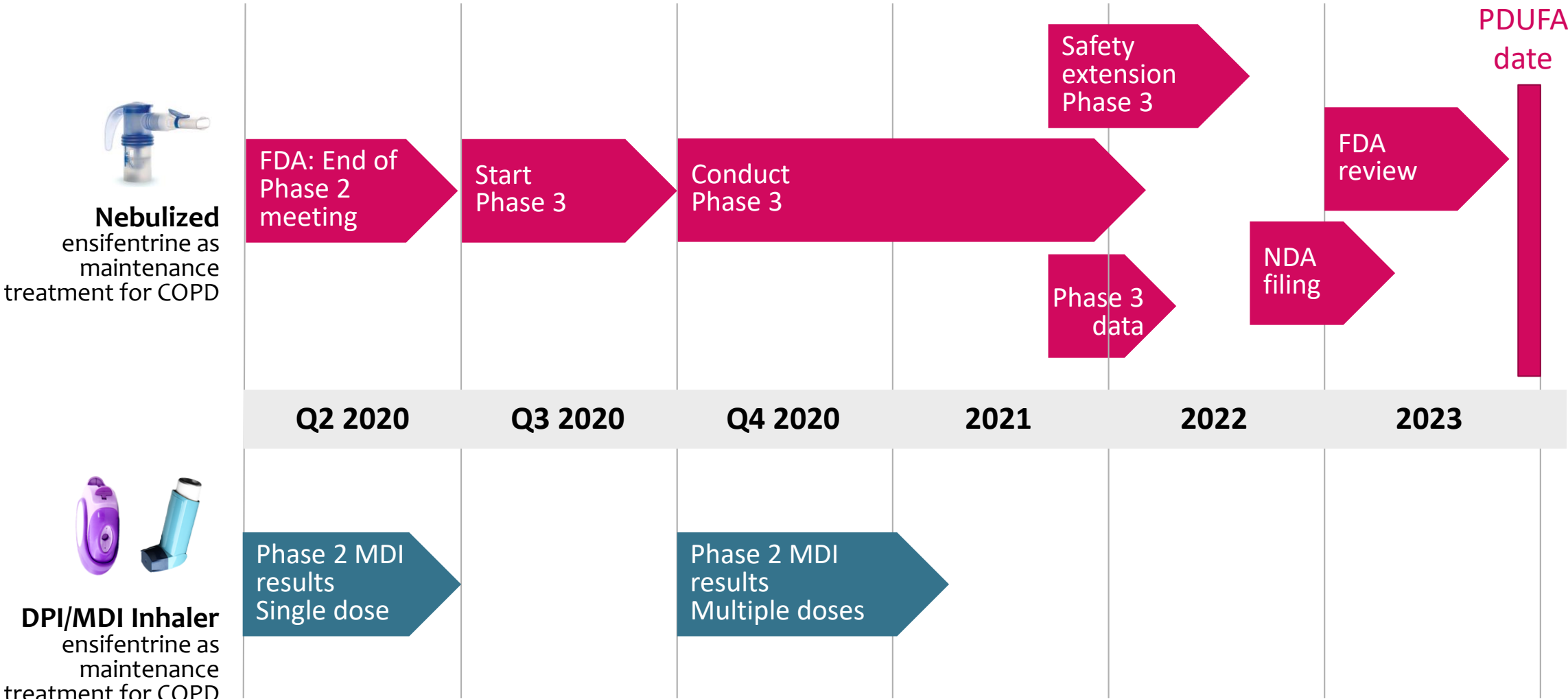


Placebo corrected

*Ensifentrine*

RPL554-MD-201 Ph 2 study, data on file

# Anticipated milestones as ensifentrine advances



# Ensifentrine patent estate

Global rights through 2030s

Invention	Granted/Pending Application	Estimated Patent Expiry
Composition of matter	Granted US, Europe, Asia, other	March 2020
Suspension formulations	Granted US, Europe, Asia, other	2035
Polymorph	Granted US, Europe, Asia, other	2031
MDI formulation	Pending	2039
DPI formulation	Pending	2040
Manufacturing process	Granted Europe; Pending other	2037
Salt forms	Granted US; Pending other	2036
Treatment of cystic fibrosis	Granted Europe; Pending other	2035
Combinations with beta-agonists	Granted US, Europe, pending Canada	2034
Combinations with anti-muscarinics	Granted US, Europe, other, pending other	2034

# Blue chip shareholder base with long-term focus

## Financial highlights

Cash and equivalents (as of Dec 31, 2019)	<b>\$40.8M<sup>1</sup></b>
Operating expenses (Year ended 12/31/19)	<b>\$54.5M<sup>1</sup></b>
Market cap (Nasdaq) (as of March 31, 2020)	<b>\$54.7M<sup>2</sup></b>
Shares outstanding (as of March 31, 2020)	<b>106.2M shares</b> (equal to 13.25M ADRs)

novo  
holdings

VIVO  
CAPITAL

 **OrbiMed**  
Healthcare Fund Management

NEA

 **ABINGWORTH**  
Life Science Investing

venBio<sup>™</sup>

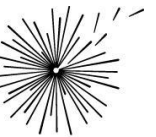
 **POLAR  
CAPITAL**

 **TEKLA**  
Capital Management LLC

**AISLING  
CAPITAL**



**Thank you**



**Verona Pharma**