Verona Pharma

Breathtaking science

Developing respiratory drugs for better quality of life

Wedbush PacGrow Healthcare Conf
New York August 2019

Nasdaq: VRNA
AIM: VRP
www.veronapharma.com

Forward-looking statements

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Ensifentrine is a first-in-class candidate for respiratory disease

Plan to enter global Phase 3 studies in 2020

Inhaled PDE3 and PDE4 inhibitor

Bronchodilator and anti-inflammatory agent
in a single compound
Rich patent estate (until mid-2030s)

A very significant commercial opportunity

Large US COPD market

COPD: The silent epidemic

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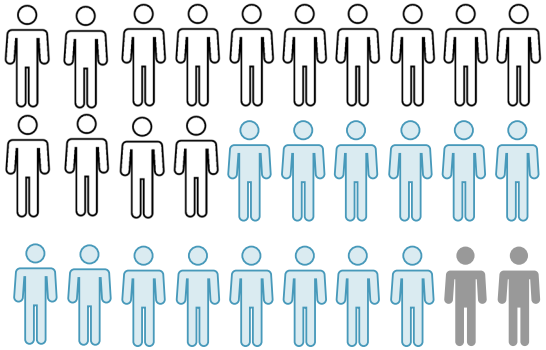
~30 million patients in US alone



~16M
Diagnosed

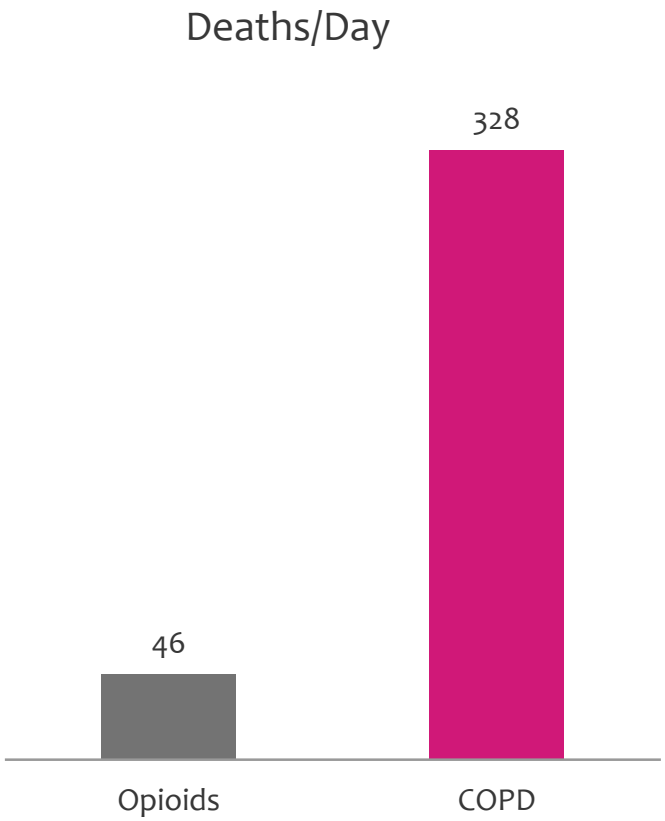
~2M
Severe/
very severe

Cost

~$50 billion/year by 2020

Indirect & direct

3rd leading medical cause of death by disease in US



Sources: COPD Foundation. Sullivan J, et al. Chronic Obstr Pulm Dis. 2018; 5(4): 324-333.

China: Large and Fast Growing COPD Market

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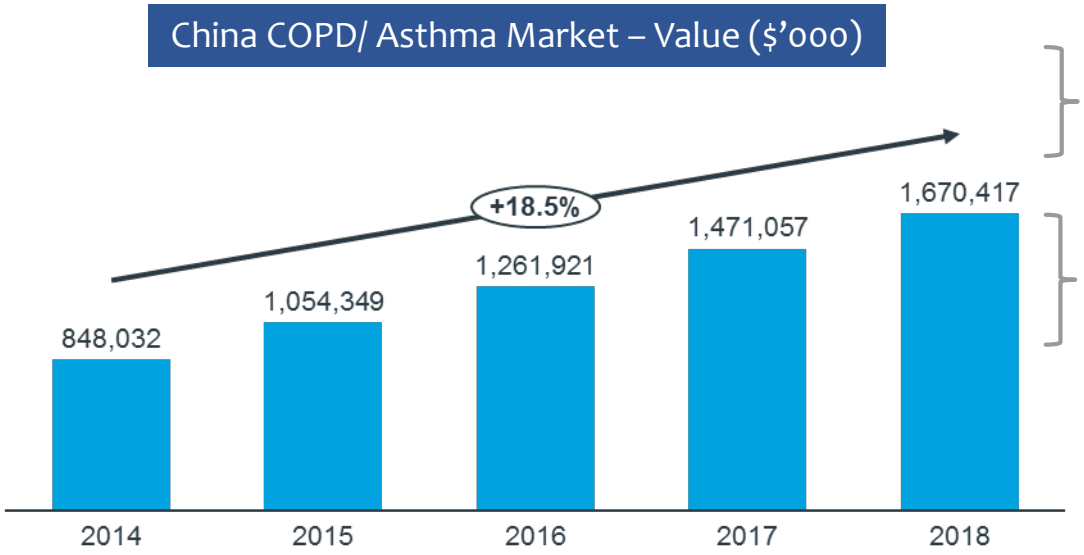

- Treated COPD population: ~8 million (vs US 6M) cigarette smoking and air pollution leading causes
- Hospital driven market ~90% of sales in terms of value (vs. US ~80% in retail channel)
- ~15,000 hospital 'nebulizer rooms' supporting annual ~$500M Pulmicort nebulized market

*Calculated based on sum of forecasted sales of R3 sub-classes from Therapy Prognosis report. MAT: Moving Annual Total. Traditional Chinese Medicines (TCMs) excluded

Sources: MIDAS (see appendix for details); IQVIA Therapy Prognosis report; IQVIA Disease Insights report

COPD: a significant unmet need

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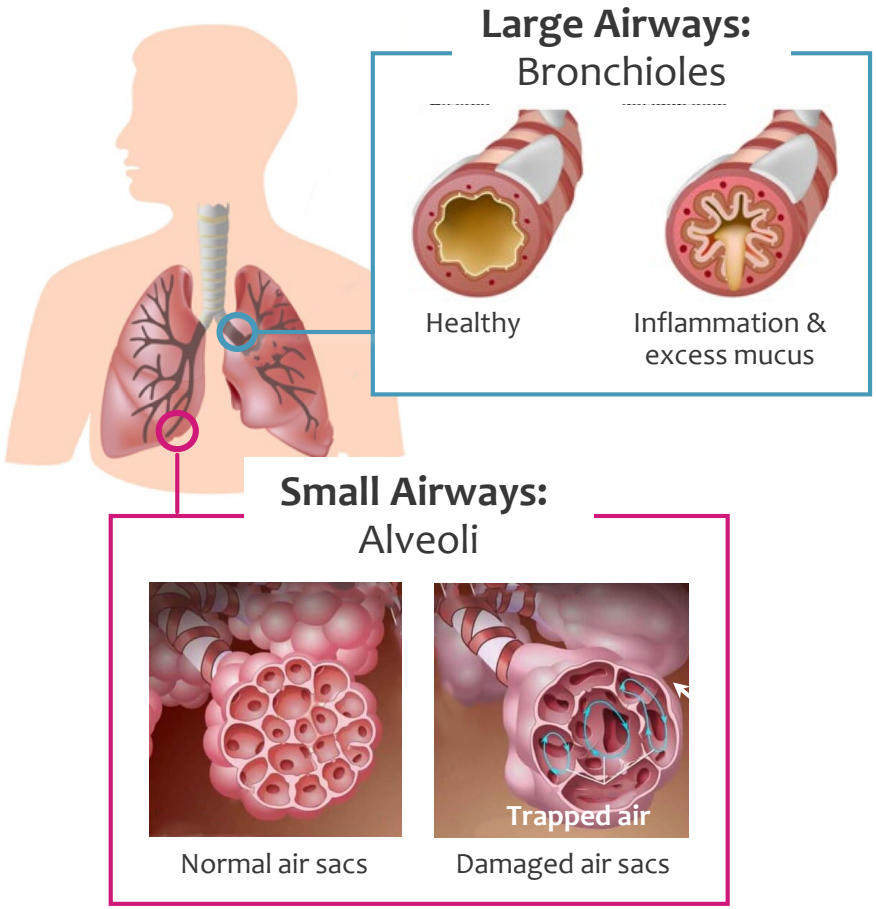

Consequences and symptoms

- Debilitating breathlessness
- Coughing, sputum
- Poor lung function
- Fatigue / struggle with daily tasks
- Exacerbations / flare-ups

Ensifentrine first-in-class candidate: Bronchodilator and anti-inflammatory in a single compound

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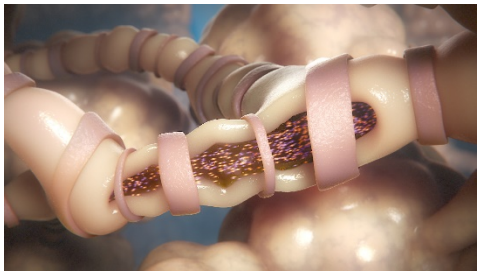
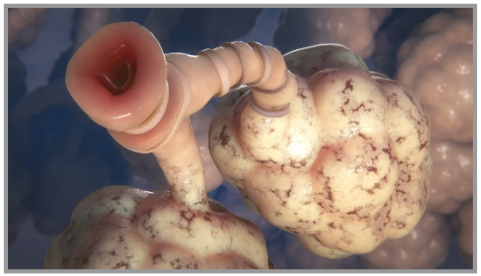
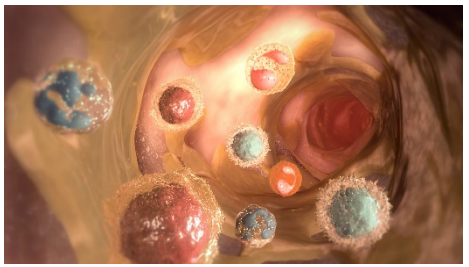
Ensifentrine (RPL554)
Dual **PDE3** and **PDE4** enzyme inhibitor

Impacts 3 Key Mechanisms in Respiratory Disease:







Airway Smooth Muscle[1-4]

PDE3, PDE4

Relaxation

↓

Increased bronchodilation

Inflammatory Cells[5,6]

Neutrophils
PDE4

Epithelial cells
PDE3, **PDE4**

Macrophages
PDE3, **PDE4**

Eosinophils
PDE4

Lymphocytes
PDE3, **PDE4**

Fibroblasts
PDE4

↓

Increased anti-inflammatory effects

Epithelial Cells[7,8]

PDE3, **PDE4**

CFTR Activation

↓

Increased mucociliary clearance

1. Calzetta L, et al. J Pharmacol Exp Ther 2013;346:414-23; 2. Calzetta L, et al. Pulm Pharmacol Ther 2015;32:15-23; 3. Matera MG, et al. Am J Respir Crit Care Med 2013;187:A1495; 4. Venkatasamy R, et al. Br J Pharmacol 2016;173:2335-51; 5. Boswell-Amith V, et al. J Pharmacol Exp Ther 2006;318:840-8; 6. Franciosi LG, et al. Lancet Respir Med 2013;1:714-27; 7. Schmidt D, et al. Br J Pharmacol 2000;131:1607-18; 8. Turner MJ, et al Am J Physiol Lung Cell Mol Physiol 2016;310:L59-70.

Nebulized ensifentrine in COPD: Very large market opportunity in US

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6M treated

2M on dual/triple therapy

800,000 symptomatic patients on dual bronchodilator/triple therapy need additional treatment

Current market data	Potential patient population
About 1/3 of moderate to severe patients use nebulizer	>250,000
Avg. Annual WAC Price of existing nebulized COPD drugs	$12,000

Attractive Medicare Part B Reimbursement

Top-prescribing physicians can be reached with targeted specialist salesforce

Source: DRG research Q4:2018. WAC; Wholesale Acquisition Cost.

Phase 2b as stand-alone treatment: Rapid, Significant and Clinically Meaningful Bronchodilator Response Maintained over Four Weeks

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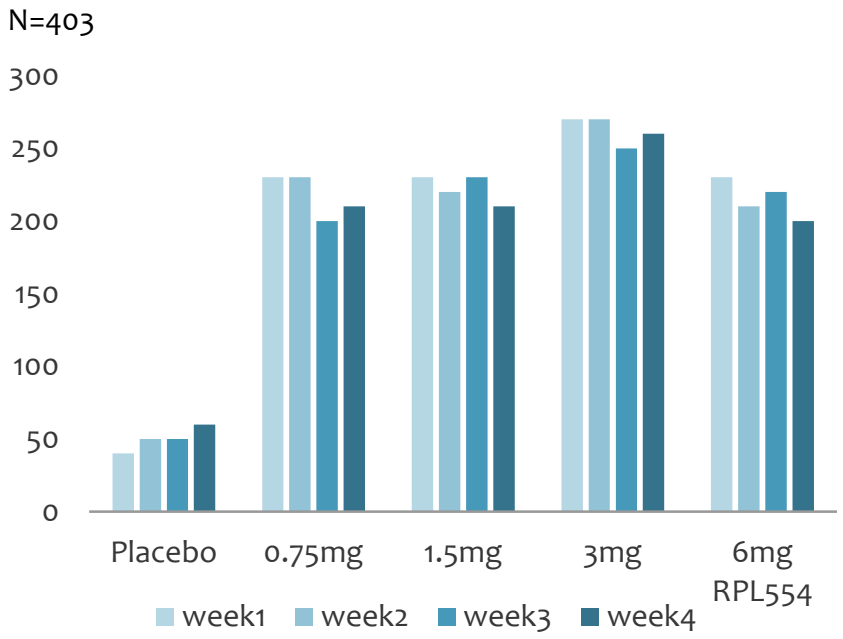
Lung function

Peak Change FEV_1 (mL), p<0.001*



*Peak Change from Day 1 in Baseline in FEV_1 (mL) on Day 28, Week 4, Primary endpoint was met

4 Week Phase 2b: Progressive symptom relief as single treatment

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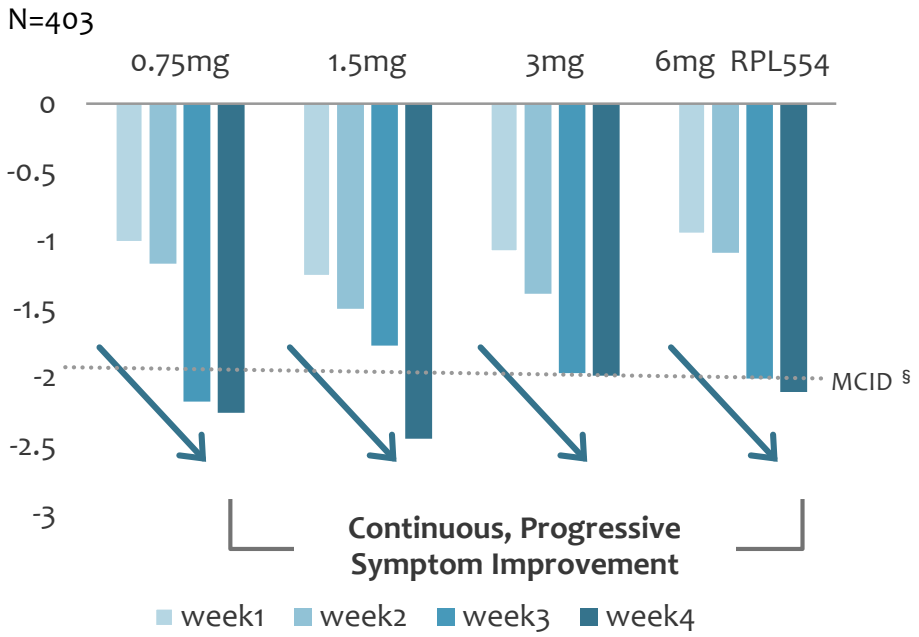
Symptom relief

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



** Placebo corrected
§ Minimal clinically important difference

Symptom improvement believed to be due to anti-inflammatory effect

- Symptoms are a precursor to exacerbations;

Müllerová H, et al. PLoS One 2014;9:e85540

Effective symptom improvement in both reversible and non-reversible patients with COPD

Symptom improvement appears unrelated to magnitude of bronchodilation

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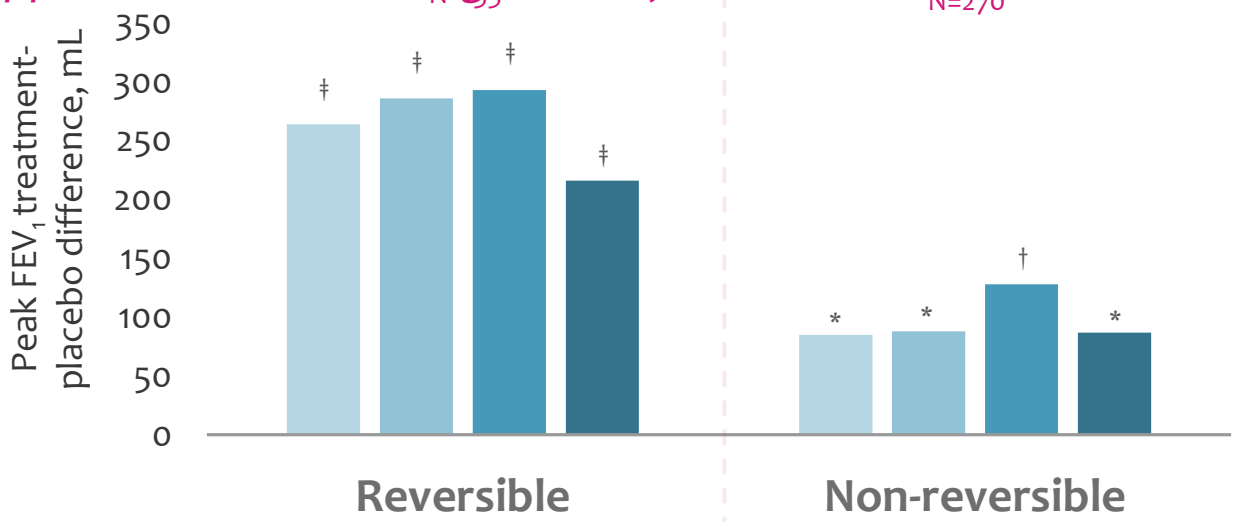
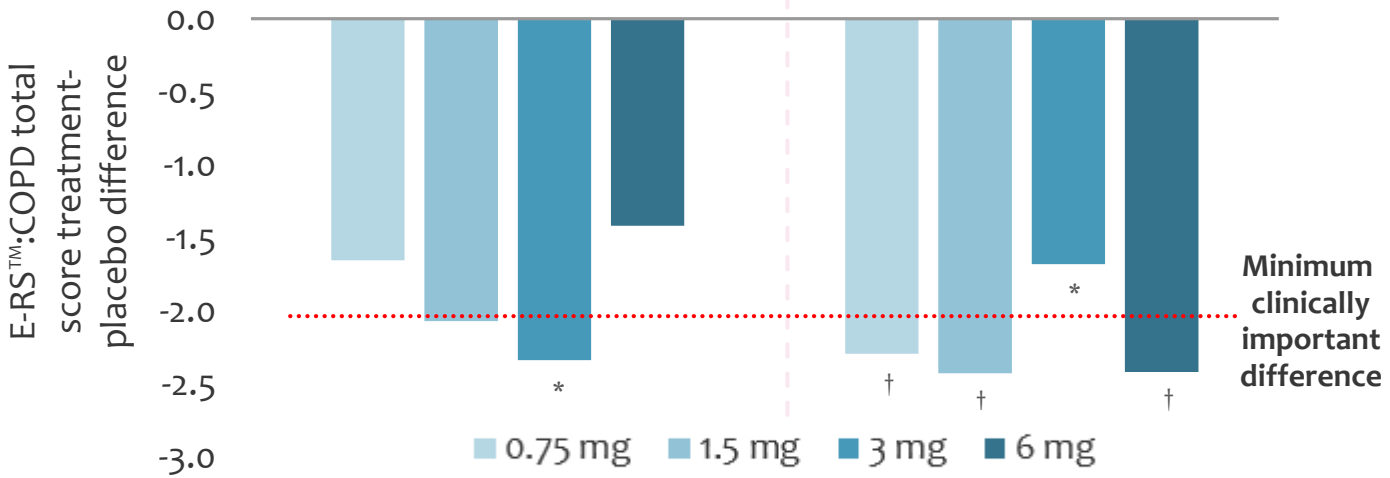
Lung function
Peak FEV_1 at Week 4



Reversibility defined as pre- to post-salbutamol change in FEV1 at screening of ≥200 mL and ≥12%.

Symptom relief
(E-RS™:COPD total score at Week 4)



4-week Phase 2b study in 403 moderate-severe COPD patients, no background bronchodilator therapy.

*p<0.05; †p<0.01; ‡p<0.001. Data are least squares mean ensifentrine–placebo differences.

Phase 2 as add-on to tiotropium (Spiriva): Significant Additional Improvements in lung function

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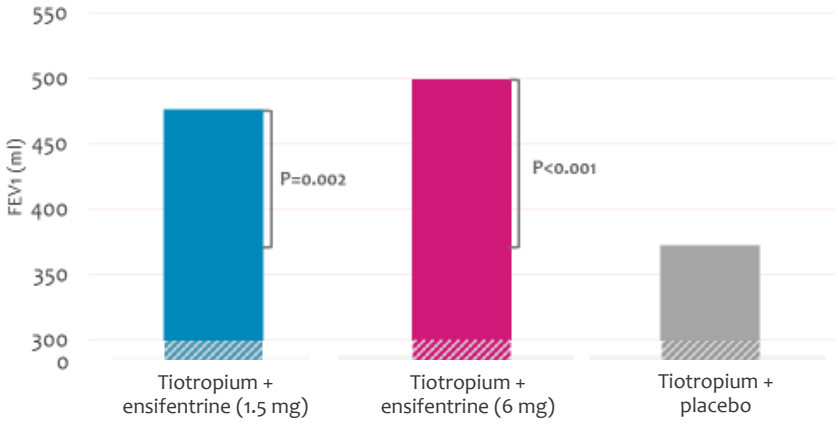
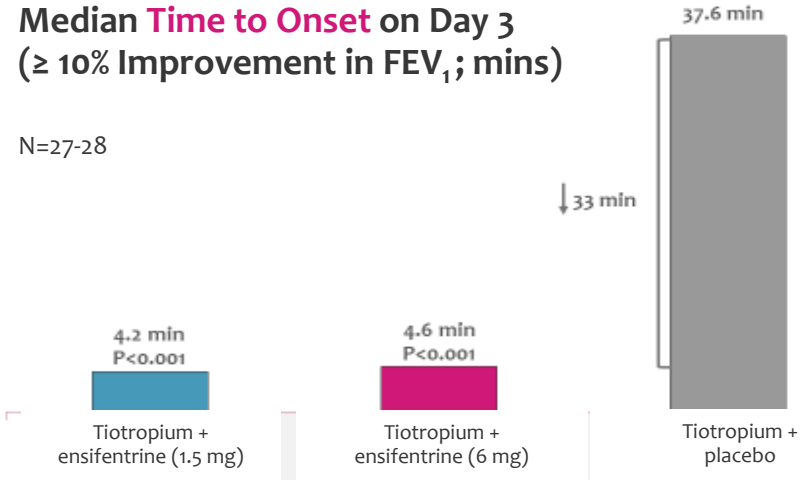
Change in Morning Peak FEV_1 from Baseline (mL), Day 3

N=27-28



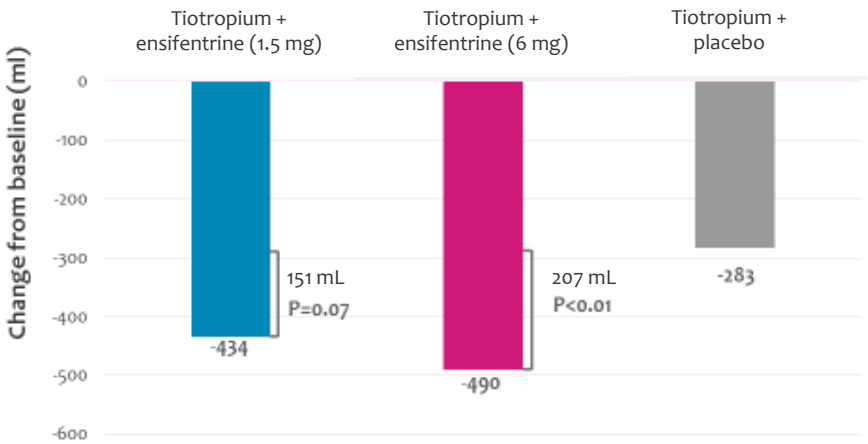
Median Time to Onset on Day 3 (≥ 10% Improvement in FEV_1; mins)

N=27-28



Reduction in Hyperinflation (mL) on Day 2 (Morning)

N=27-28



- Additional improvement in peak FEV1
- Reduction of hyperinflation - typically correlated with improvement in symptoms
- Rapid onset of action
- Well tolerated

Phase 2 as add-on to dual and triple COPD therapy: additional lung function improvement over 24 hours

Further reduction in hyperinflation 140-260 ml

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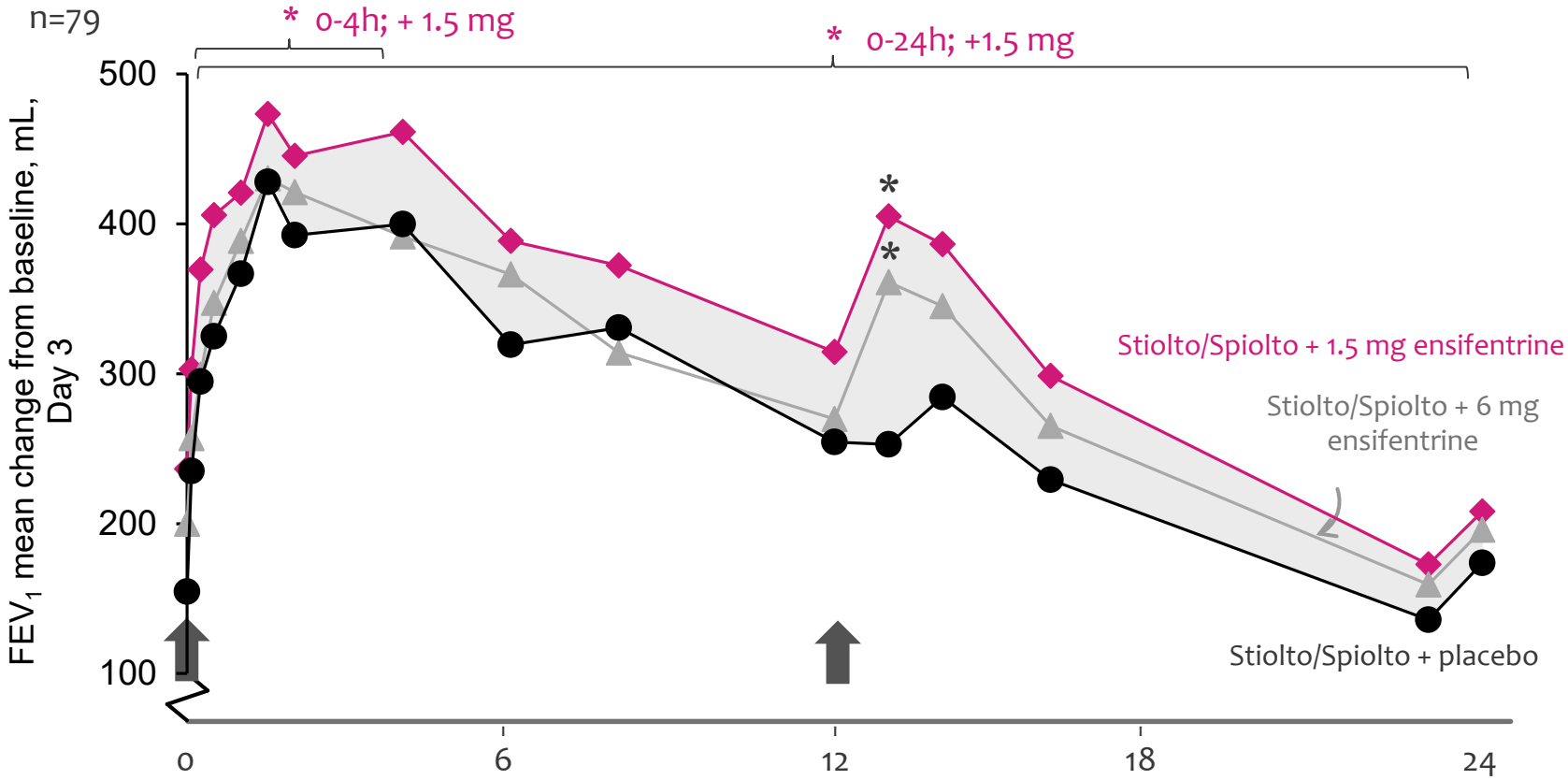

Day 3 morning Peak FEV1, primary endpoint (not statistically significant)

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

Potential to improve FEV1 and symptoms in patients with no further maintenance treatment options

Data on file, Verona Pharma.

Learnings from 3 day study informs Ph3 positioning study in COPD

Results from post hoc analysis

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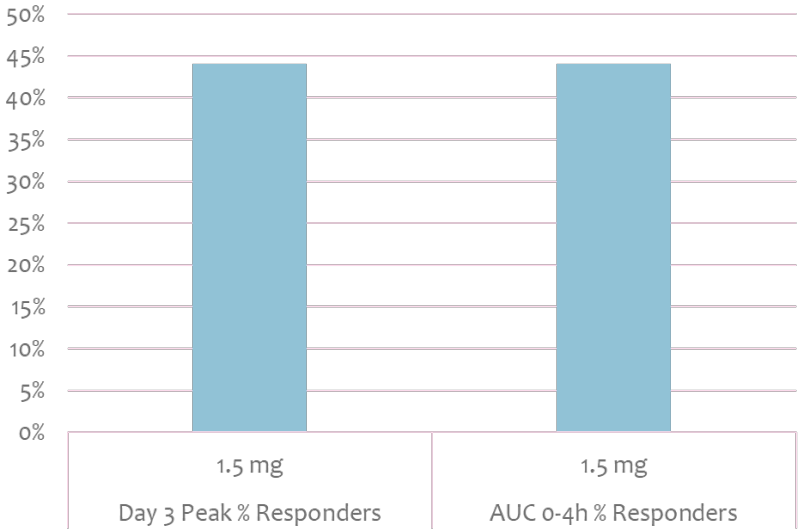
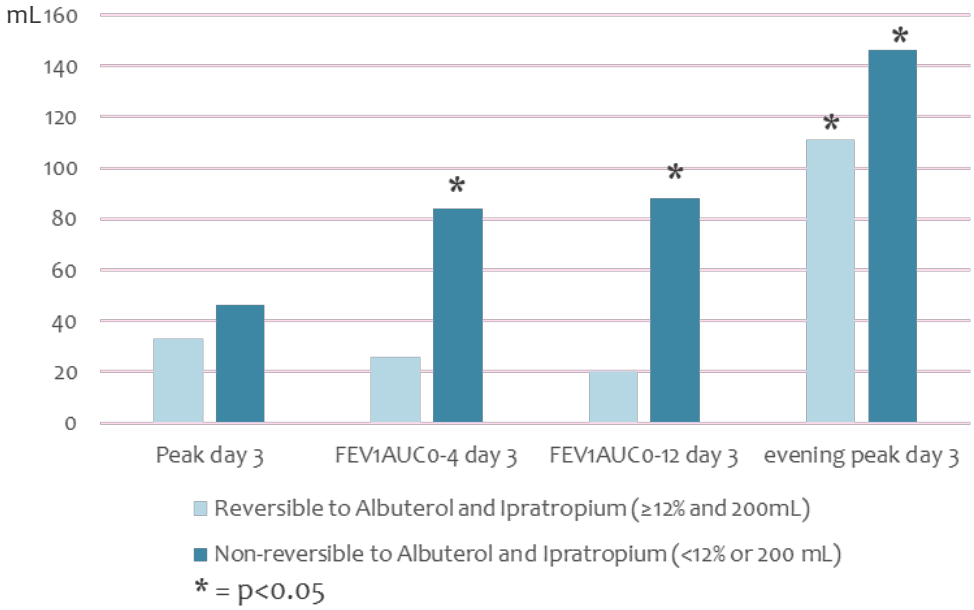
>40% of patients had ≥ 100 mL increase in peak FEV1 vs placebo



1.5 mg ensifentrine: additional response in non-reversible patients vs. those reversible to beta2 agonist and muscarinic antagonist



1) Enrich Ph3 study, as add-on to dual/triple therapy, with symptomatic patients that are also poorly reversible to standard bronchodilators, 2) explore most effective endpoints and 3) drop 6 mg top dose

Phase 2b, 4 week study as add-on to tiotropium to inform EoP2, Ph3 and commercial positioning

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Study design

- **Purpose:** Investigate dose response of ensifentrine in moderate to severe COPD patients who are symptomatic despite treatment with tiotropium
  - **Facilitate dose selection for Phase 3** (0.375, 0.75, 1.5 and 3 mg vs placebo)

- **Population:** Moderate to severe COPD
  - **Patients will be required to be symptomatic** at randomization; mMRC ≥2
  - **Stable tiotropium as required background therapy** (2-week run-in on tiotropium Respimat)

- **Key Endpoints:** FEV1 (peak, AUC, trough), E-RS symptoms

Recruitment initiated in May – data expected around year end

Nebulized ensifentrine: Advancing towards Phase 3 with differentiated profile

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Phase 2: Establish activity + profile → **Phase 3:**

1 Monotherapy
(Dose Ranging)
400 pts
Bronchodilator + anti-inflammatory
Completed 2018

Add-on to Single Therapy
(2 Ind. P2 Studies)
Bronchodilator
Completed 2017

2 Add-on to Single Therapy
(Dose Ranging)*
Bronchodilator + anti-inflammatory*

Add-on to Double/Triple Therapy
Bronchodilator
Completed Jan 2019

* *Results expected in 4Q 2019/1Q2020*

End of Phase 2 Meeting with FDA, target H1 2020

A. Pivotal studies:
Ph3 design and endpoints as in Ph2 studies

2 trials of 6 month duration, one with 6 month safety extension
-
None or single bronchodilator Background 1 + 2
-
FEV1 and symptom improvement, explore exacerbations in pooled data

B. Positioning study:
Inform physicians and payors

Add-on treatment to dual bronchodilators / triple therapy

Ensifentrine lifecycle: Expanding the pipeline

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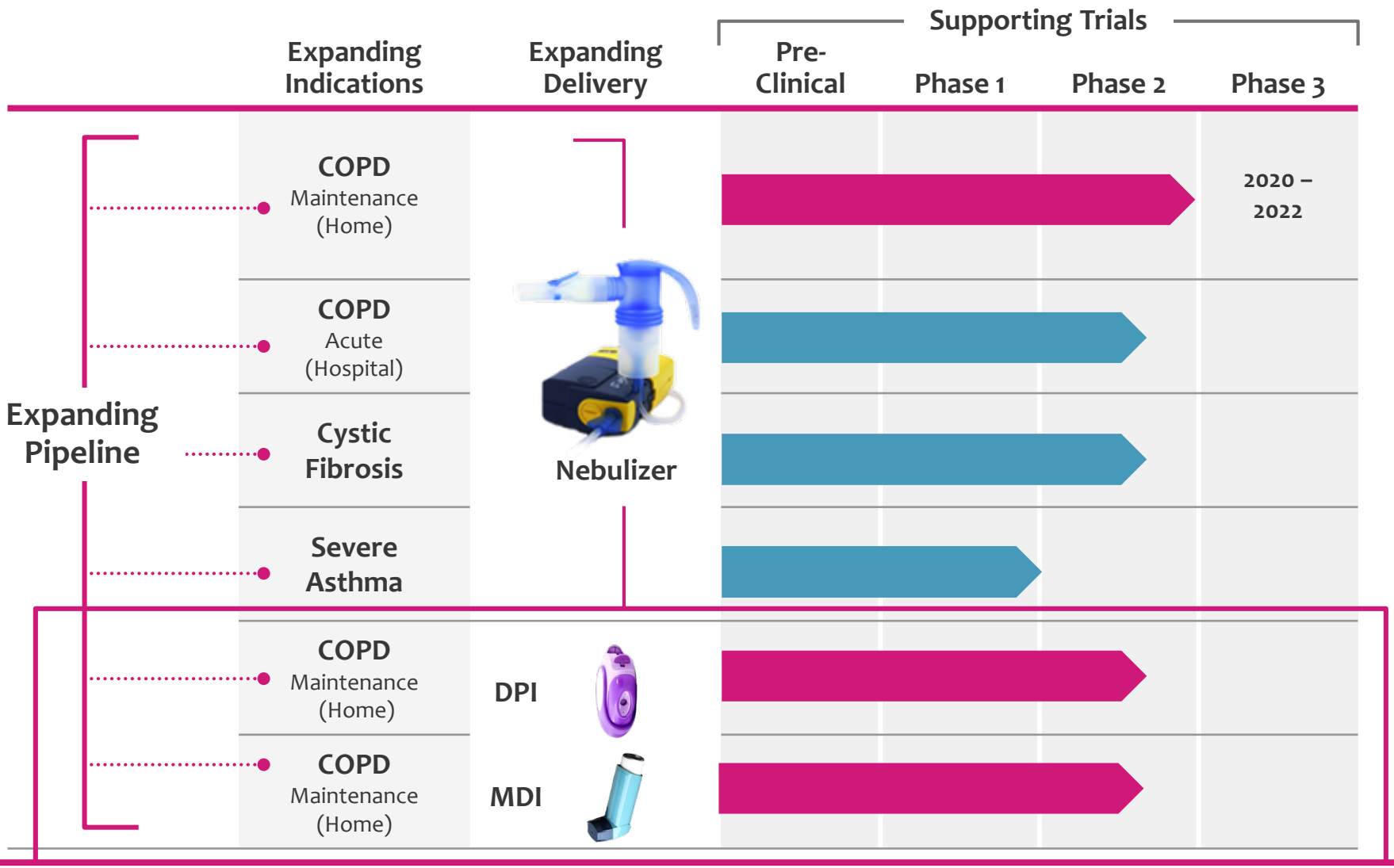
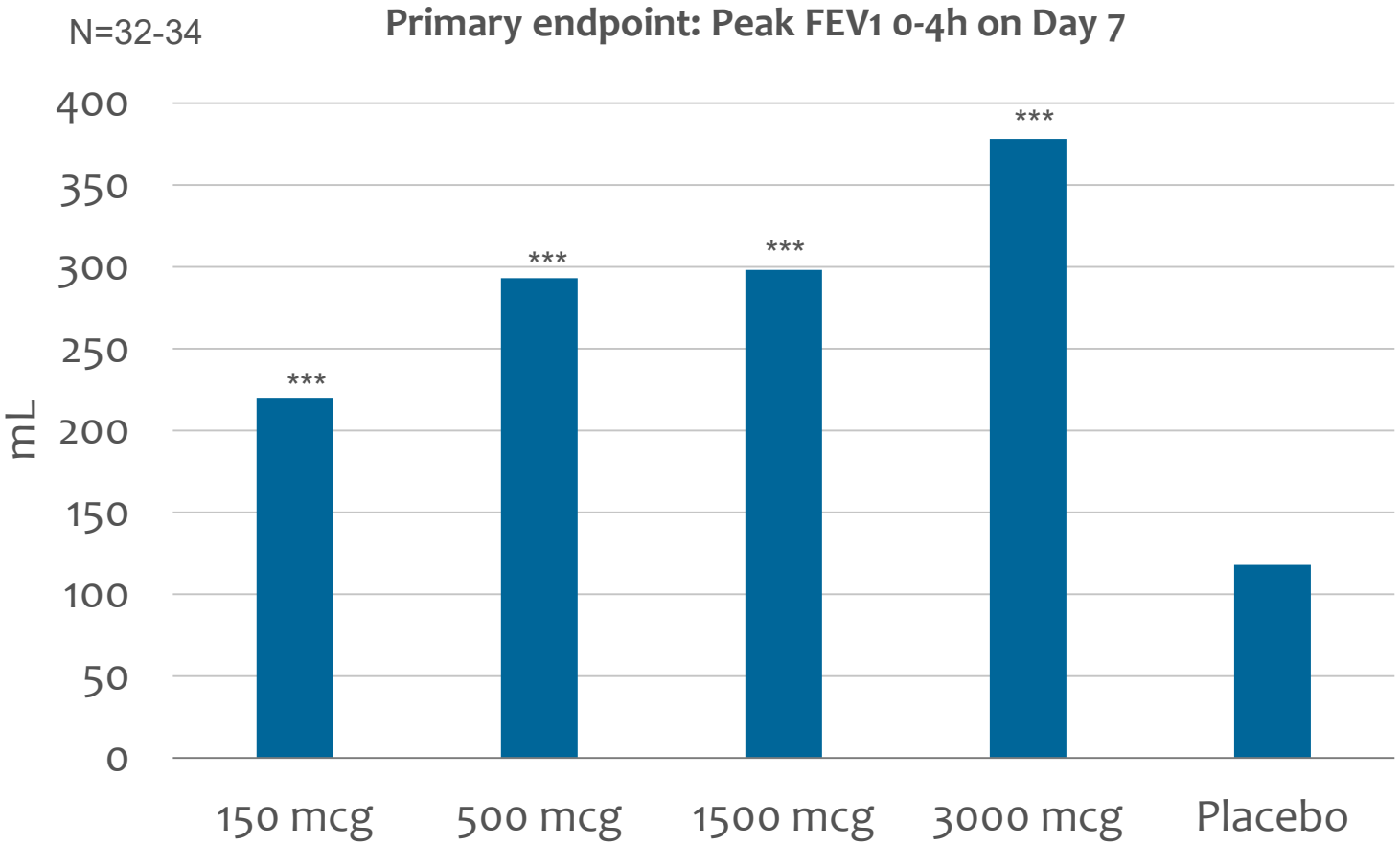


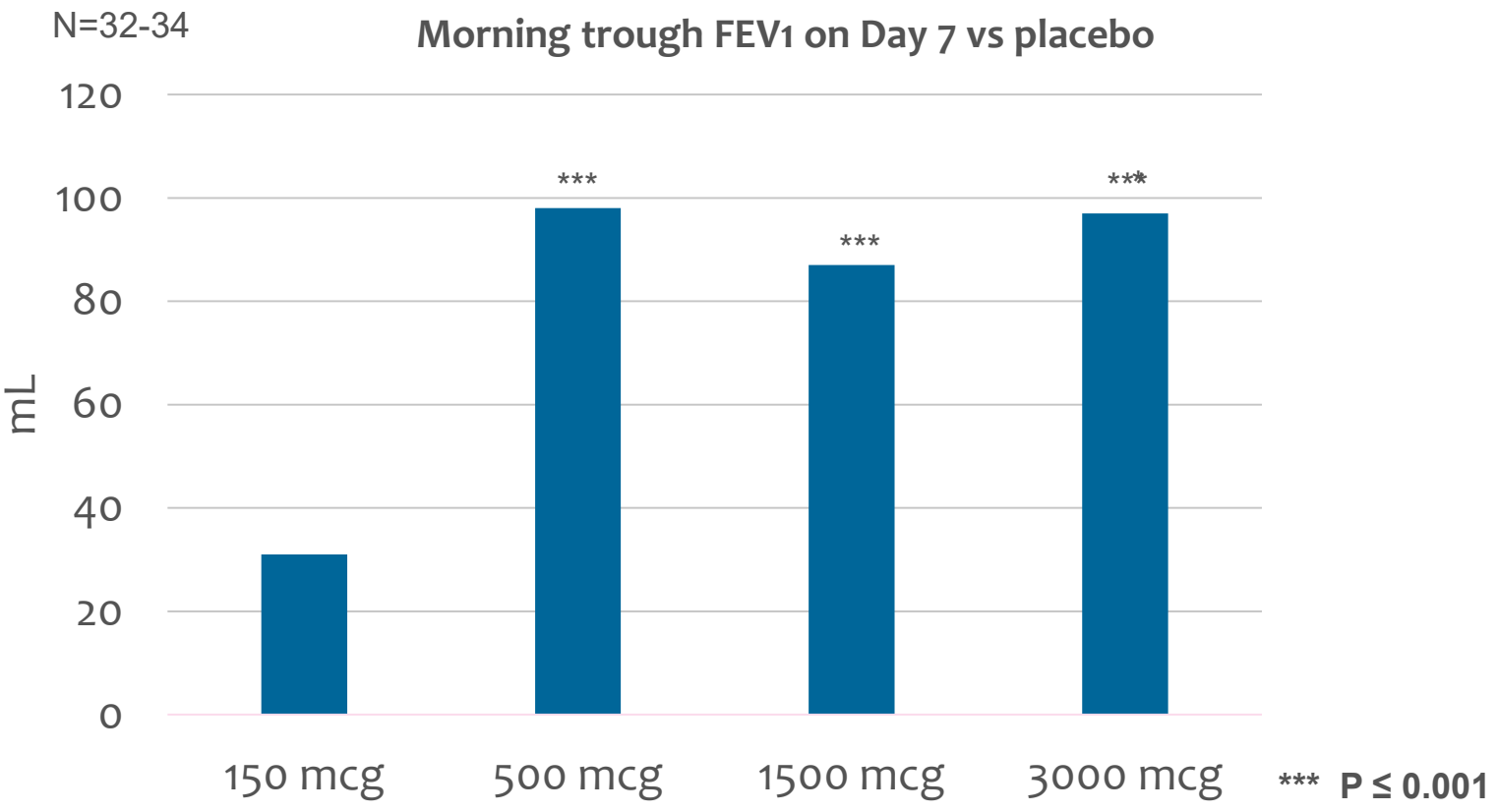
Primary endpoint met - dry powder formulation of ensifentrine produced highly significant improvement in Peak FEV_1 in COPD

Clinically meaningful, statistically significant and dose-dependent bronchodilation



Secondary end point met: Consistent Trough FEV_1 Response further support twice daily dosing

Statistically significant and meaningful improvement in trough FEV1



DPI/ MDI partnering opportunity could dramatically expand commercial potential

Backed by major healthcare investors

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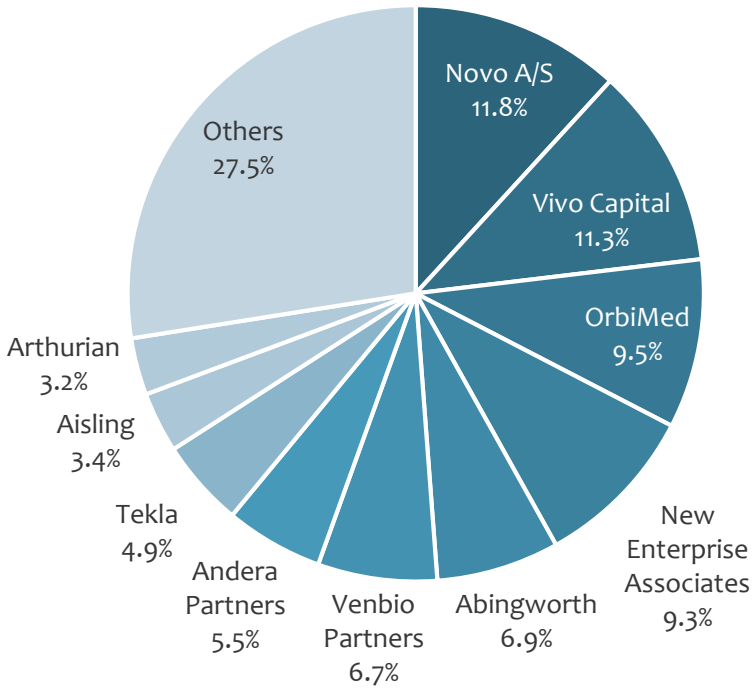
Financial overview June 30, 2019

Cash and cash equivalents	$59.1M[1]
Operating expenses 1H19	$25.2M[1]
Market cap	$58.0M[2]

Shareholdings[3]



[1]Exchange rate used (US dollars per pound sterling): June 28, 2019: $1.2704
Cash and cash equivalents comprises cash + cash deposits > 3 months maturity
Cash and equivalents at June 30, 2019 amounted to £46.5M ($59.1M)
[2]Current issued 105.3M shares or 13.2m ADSs, share price $4.41 on August 9, 2019
[3]As disclosed to the Company in accordance with AIM Rule 26, or through s80 notices and 13F and 13G filings

Ensifentrine: Multiple value creation opportunities

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In COPD

Nebulized formulation in US

- 800,000 symptomatic patients on dual bronchodilator/triple therapy need additional treatment

Nebulized formulation in China

- Prevalence ~70 million COPD patients; potential large market for nebulized drugs as about 90% of drug sales are in the hospital

DPI or MDI formulation for COPD

- Large market, >5 million patients in US; partnering opportunity

In other indications

Cystic fibrosis

- Potential first anti-inflammatory drug, independent of CF mutation status

Severe Asthma

- Bronchodilator and anti-inflammatory agent, possibly before initiating more restrictive biologics treatments

Chronic cough

- Anti-inflammatory mechanism reduces cough and improves mucociliary clearance

Nebulizer Phase 3 planned to start in US in 2020

Upside potential: China, DPI/MDI formulations and additional indications

2019: Multiple significant milestones as ensifentrine advances towards Phase 3 in 2020

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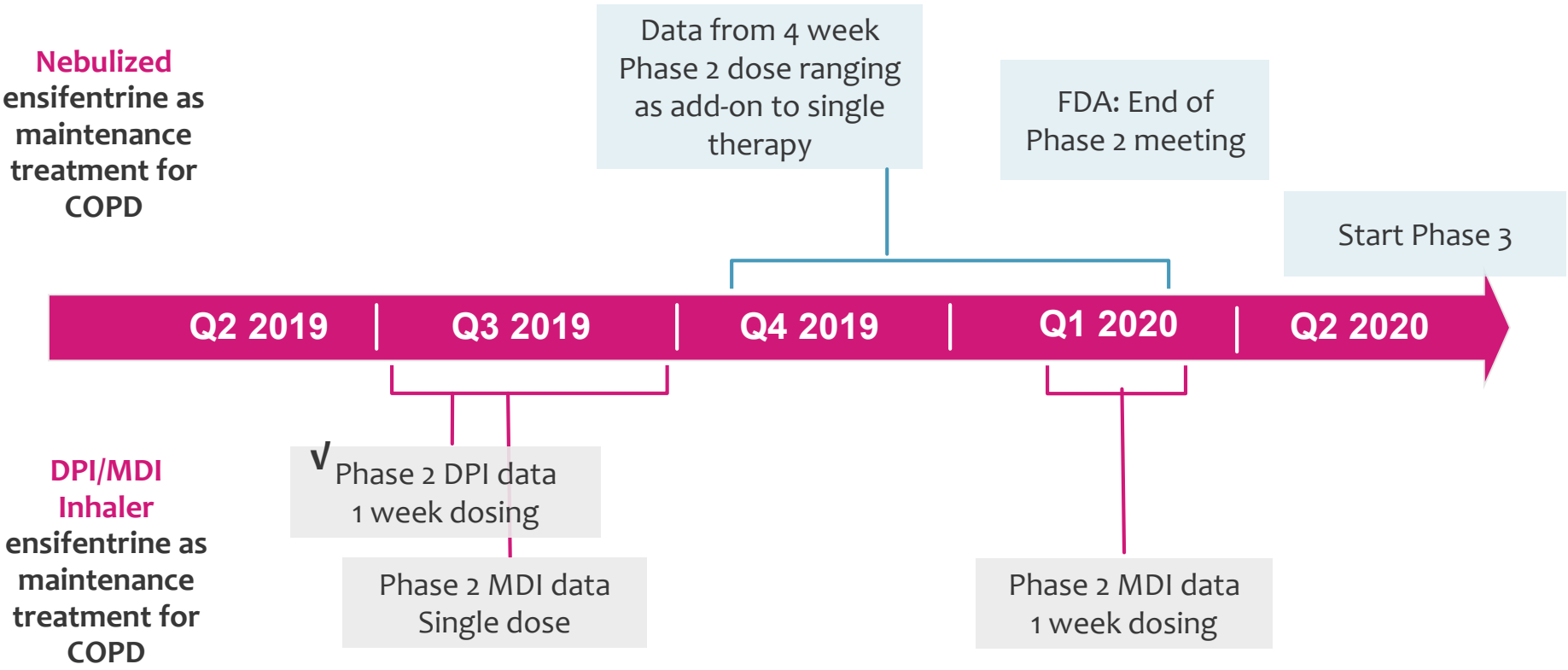

Simple Phase 3 trial design, similar to Phase 2b studies, to increase likelihood of regulatory success

Ensifentrine: Promising novel treatment for patients with COPD

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- ✓ First-in-class PDE3/4 inhibitor with bronchodilator and anti-inflammatory effects, rapid onset of action and well tolerated
- ✓ Reduces residual volume/air trapping
- ✓ Improves symptoms in moderate to severe, symptomatic COPD patients on twice daily dosing
- ✓ Novel Mode of Action improves lung function in patients poorly responsive to currently available bronchodilators
- ✓ Targeting FDA End of Phase 2 Meeting 1H 2020
- ✓ Subsequently, advancing nebulized ensifentrine into Phase 3 trials in patients symptomatic despite using standard COPD medications

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Thank you