



Verona Pharma



Breathtaking science

Developing respiratory drugs to
improve health and quality of life



H.C.Wainwright & Co 20th Annual Global Conference
New York City, September 2018

NASDAQ VRNA
www.veronapharma.com



Forward-Looking Statements

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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

Bronchodilator + anti-
inflammatory agent in single
compound

- Developing nebulized RPL554 for COPD
 - Demonstrated efficacy as **add-on to single bronchodilator**
 - Phase 2 underway to assess efficacy as **add-on to dual bronchodilators (w/wo ICS)**
 - On track to commence **Phase 3** in 2019
- Advancing DPI and MDI formulations for COPD into clinic
- Opportunities in other respiratory indications: Cystic Fibrosis , Asthma

COPD: Significant Unmet Medical



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Consequences of severe exacerbations

Third leading cause of death in US

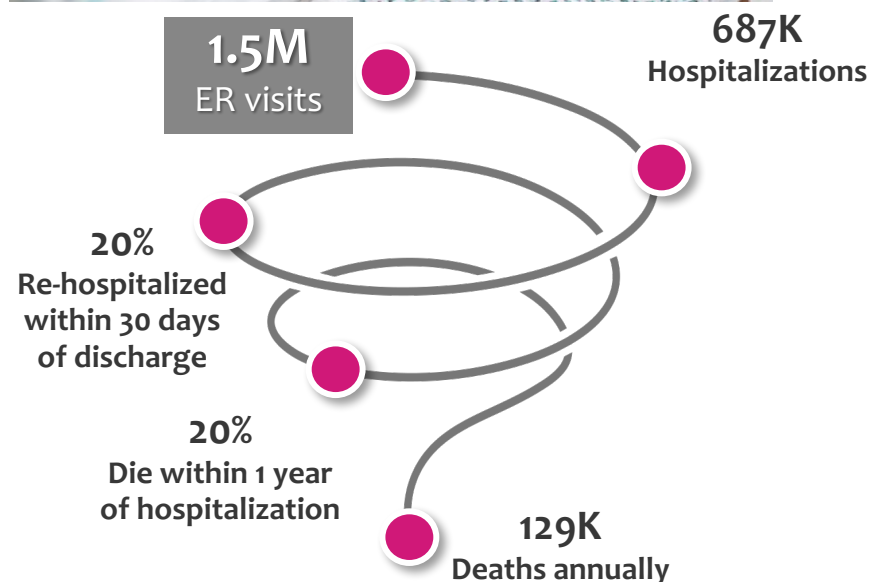
- 2-year mortality rate for severe COPD: ~50%¹

24 million US patients with COPD

- ~6 million on maintenance treatment
 - Many continue with daily COPD symptoms

Unmet Need For New Treatments: Bronchodilator + Anti-inflammatory

- Add on to current therapies
- Improve lung function
- Reduce breathlessness
- Improve symptoms
- Prevent exacerbations



~\$32 billion US annual direct healthcare costs¹

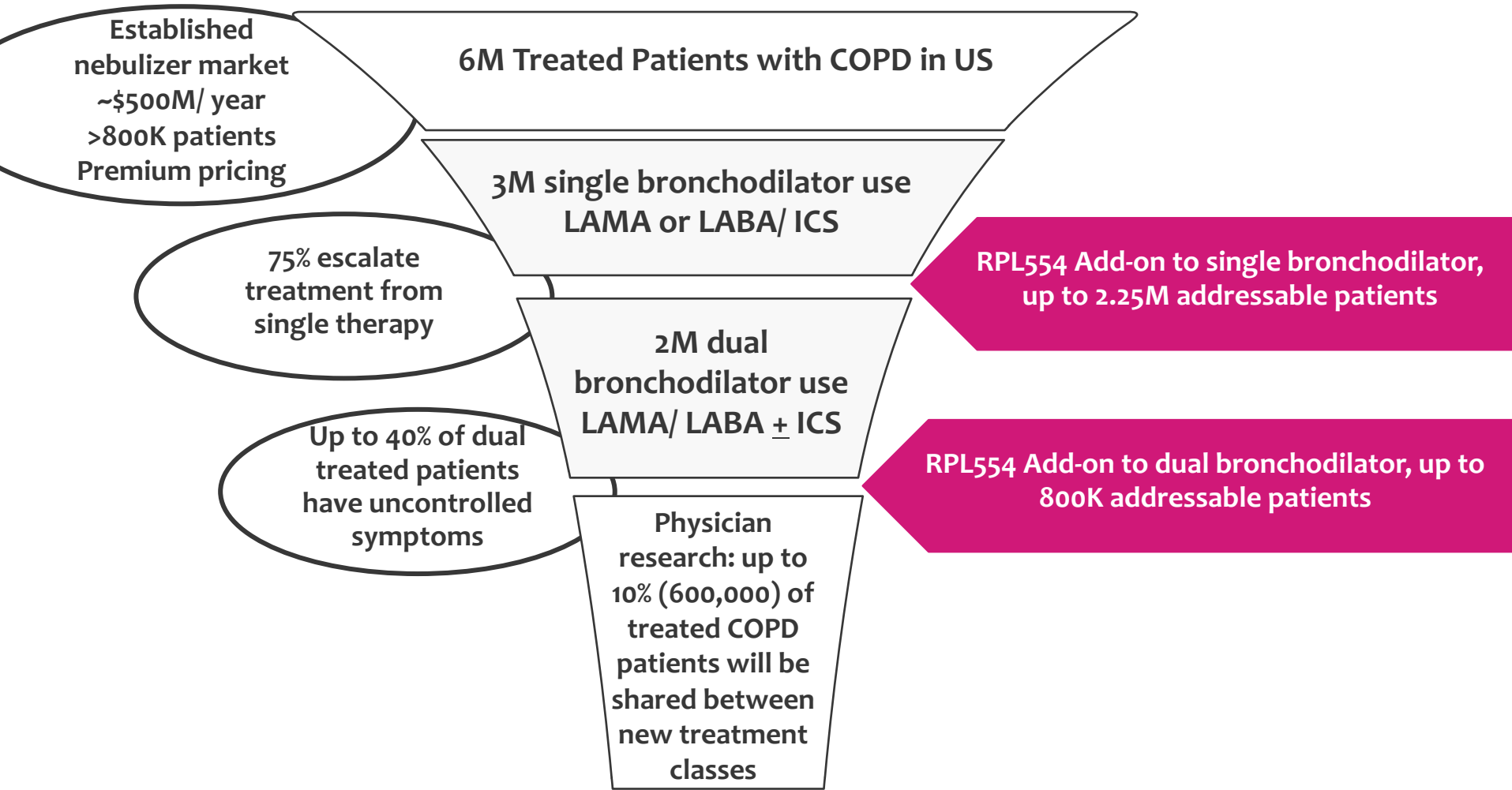
¹ Goodridge, D, RN, PhD, "COPD as Life-Limiting Illness," Topics in Advanced Nursing eJournal, 2006;6(4)
Note: U.S. only data

RPL554: Uniquely Placed as Bronchodilator and Anti-inflammatory with Novel Mode of Action



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Large numbers of uncontrolled and symptomatic COPD patients



Sources: Q2 2017 US COPD patient database & physician survey research, IQVIA MIDAS Sales, Mullerova H., et al., Characterization of COPD Patients Treated With Inhaled Triple Therapy Containing Inhaled Corticosteroid [ICS], Long-Acting Beta2-Agonists [LABA], and Long-Acting Muscarinic Antagonists [LAMA] in the UK, American Journal of Respiratory and Critical Care Medicine 2017;195:A4986

6 Vestbo J, et al., Single inhaler extrafine triple therapy versus long-acting muscarinic antagonist therapy for chronic obstructive pulmonary disease (TRINITY); a double-blind, parallel group, randomised controlled trial, The Lancet, Vol 389, p. 1919-1929; May 13, 2017

RPL554: Novel Dual Mechanism and Well Tolerated Profile Potential to Address Limitations of Current Therapies



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Bronchodilators (First Line Treatment)		Anti-Inflammatories		
Anti-Muscarinics	Beta2-Agonists	RPL554	Inhaled Corticosteroids	PDE4 Inhibitors
SAMA / LAMA	SABA / LABA		ICS	PDE4 (e.g. Roflumilast)
✓			✗	✗
✗			✓ Not indicated as monotherapy	✓
✓			✗	✗
✓			✗ Pneumonia	✗
✓		✓ Broad Patient Population	?	✗

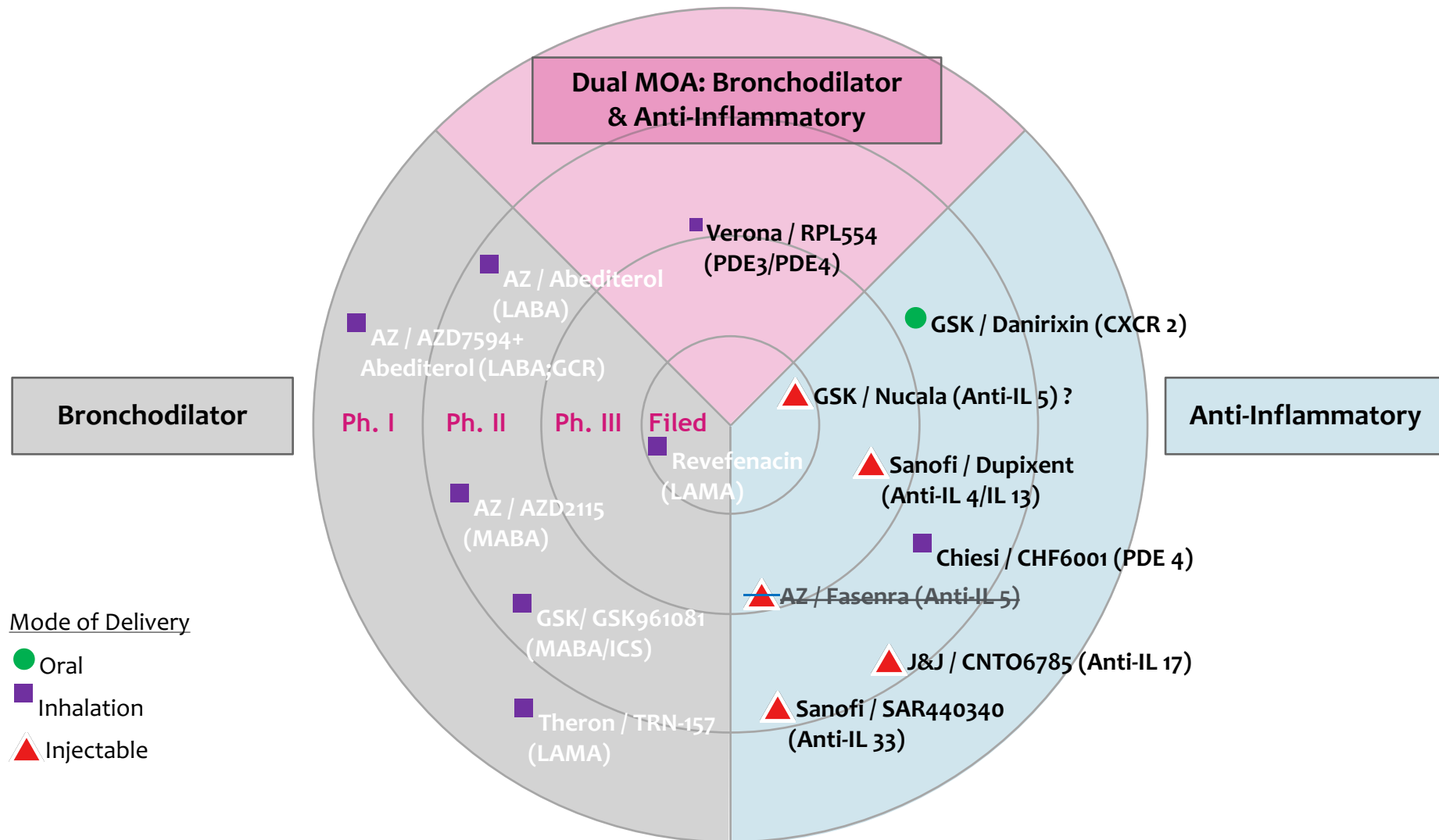
New therapies needed. COPD is a progressive disease, symptoms tend to become uncontrolled, despite current treatment options

Compelling Need For Therapy with New Mode of Action for COPD

... but few such drugs in development for COPD



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Maintenance Treatment of COPD: Substantial Market with Premium Pricing in Nebulized



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US Sales of common bronchodilators	Administration	Class	Avg monthly \$ WAC price ¹	US only sales \$M ²
Brovana (Sunovion)	Nebulizer - open	LABA	971	339
Perforomist (Mylan)	Nebulizer - open	LABA	972	155
Lonhala (Sunovion) ³	Nebulizer - closed	LAMA	1,190 ⁴	-
Revefenacin (Mylan/Theravance)	Nebulizer - open	LAMA	Nov '18 PDUFA	
Advair (GSK)	Inhaler	LABA / ICS	398	1,094
Spiriva (Boehringer)	Inhaler	LAMA	398	1,779
Anoro (GSK)	Inhaler	LAMA / LABA	398	277
Trelegy (GSK) ³	Inhaler	LAMA / LABA / ICS	530	-

1. Oct 17 – Jan 18
2. May 2016 – April 2017
3. Launched April 2018
4. Retail price, www.drug.com

Sources: Q2 2017 US COPD patient database & physician survey research, IQVIA MIDAS Sales. Mullerova H., et al., Characterization of COPD Patients Treated With Inhaled Triple Therapy Containing Inhaled Corticosteroid [ICS], Long-Acting Beta2-Agonists [LABA], and Long-Acting Muscarinic Antagonists [LAMA] in the UK, American Journal of Respiratory and Critical Care Medicine 2017;195: A4986. Vestbo J, et al., Single inhaler extrafine triple therapy versus long-acting muscarinic antagonist therapy for chronic obstructive pulmonary disease (TRINTY); a double-blind, parallel group, randomised controlled trial, The Lancet, Vol 389, p. 1919-1929; May 13, 2017

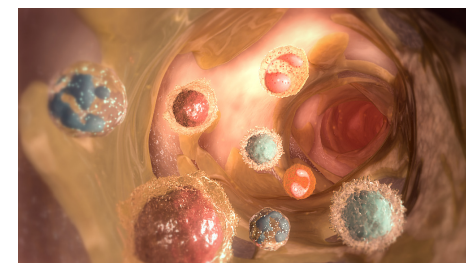
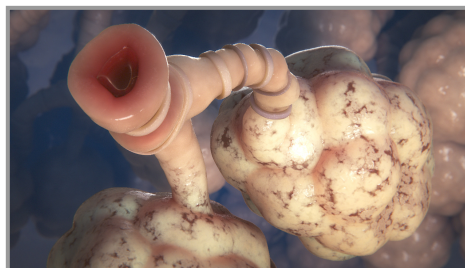
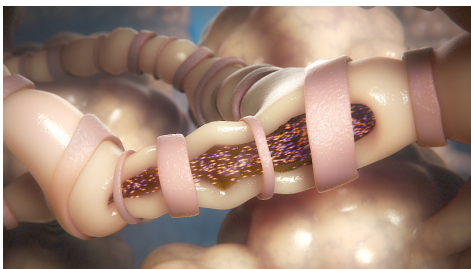
RPL554 First-in-Class Candidate: Bronchodilator and Anti-inflammatory in a Single Compound



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RPL554
Dual **PDE3** and **PDE4** enzyme inhibitor

Impacts 3 Key Mechanisms in Respiratory Disease:



Airway Smooth Muscle



PDE3, **PDE4**

Relaxation



Increased bronchodilation

Inflammatory Cells



Neutrophils
PDE4



Eosinophils
PDE4



Epithelial cells
PDE3, **PDE4**



Lymphocytes
PDE3, **PDE4**



Macrophages
PDE3, **PDE4**



Fibroblasts
PDE4



Increased anti-inflammatory effects

Epithelial Cells



PDE3, **PDE4**

CFTR Activation



Increased mucociliary clearance

Nebulized RPL554: Effective and Well Tolerated in 12 Clinical Trials with >730 Subjects



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Recent trials:

Trial	Program	# of Subjects	Duration	Status
Phase 1/2	SAD MAD study with new suspension formulation	112	Single dose and twice daily for 5 days	Completed Sept 2015
Phase 2a	Dose ranging in asthma	29	Single dose	Completed March 2016
Phase 2a	Add-on to each of albuterol or ipratropium	30	Single dose	Completed May 2016
Phase 2a	Add-on to tiotropium (Spiriva®)	30	Dosed twice-daily for three days	Completed Sept 2017
Phase 1	Pharmacokinetic trial, US FDA new IND	12	Single dose	Completed Sept 2017
Phase 2b	Maintenance treatment	403	Dosed twice daily for four weeks	Completed March 2018
Phase 2	Add-on to dual bronchodilator therapy (LAMA/LABA: Stiolto)	~75	Dose twice daily for three days	Started July 2018

Four Week Phase 2b Study Moderate to Severe COPD

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 centres in Western & Eastern Europe

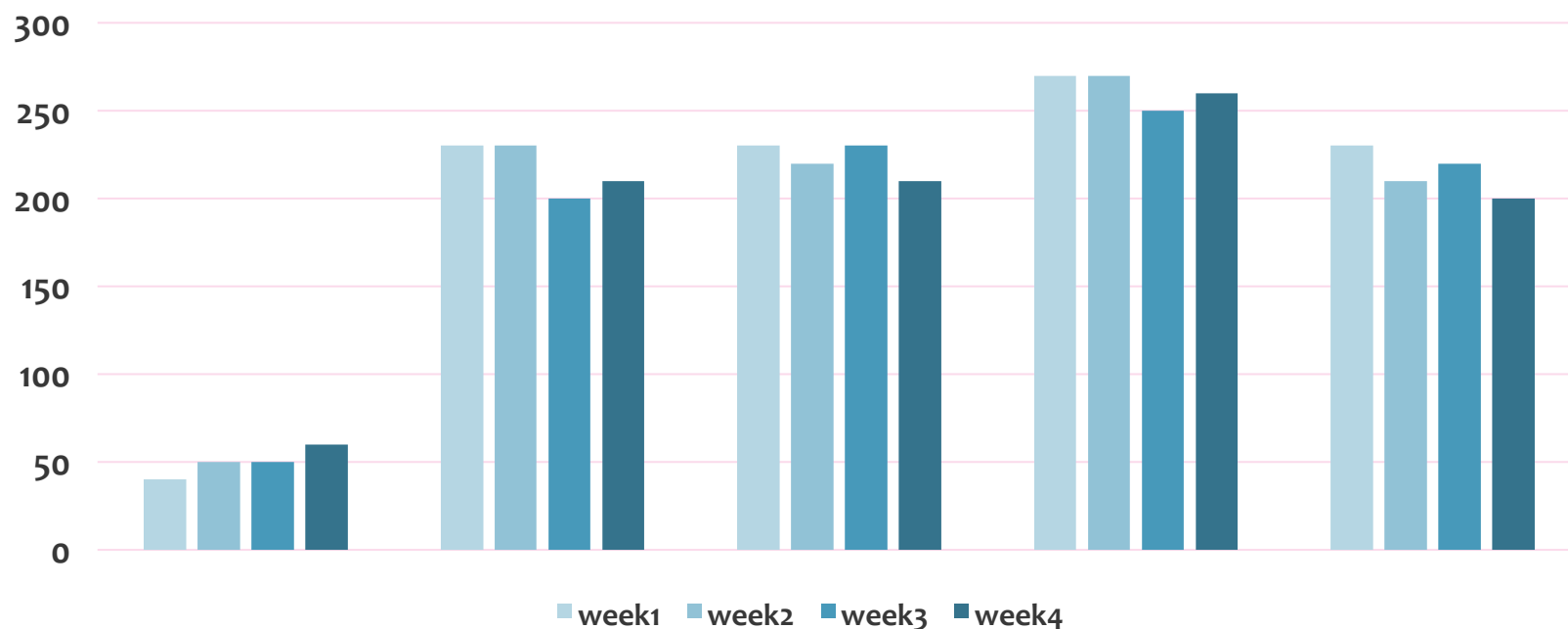
RPL554 Dosage:

- 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 FEV₁ (mL) on week 4 (p<0.001)

N=403



placebo

0.75 mg

1.5 mg

3 mg

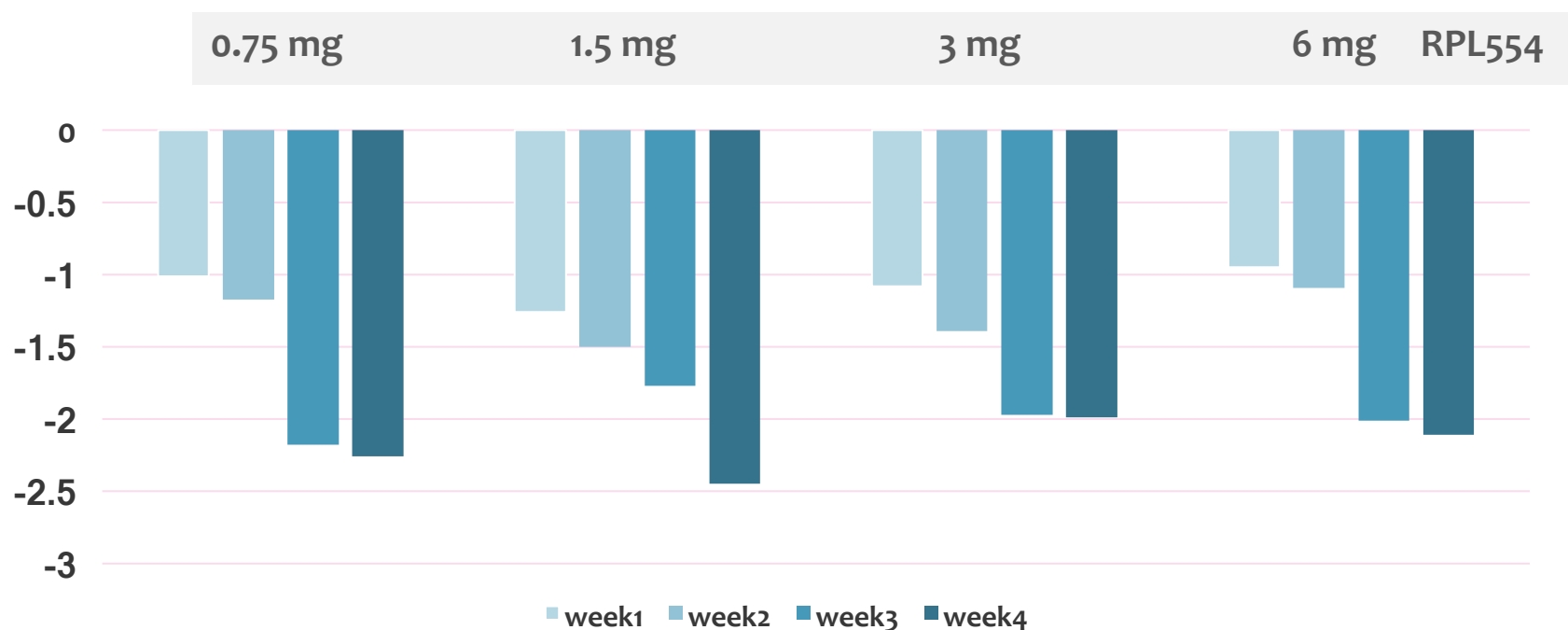
6 mg

RPL554

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)

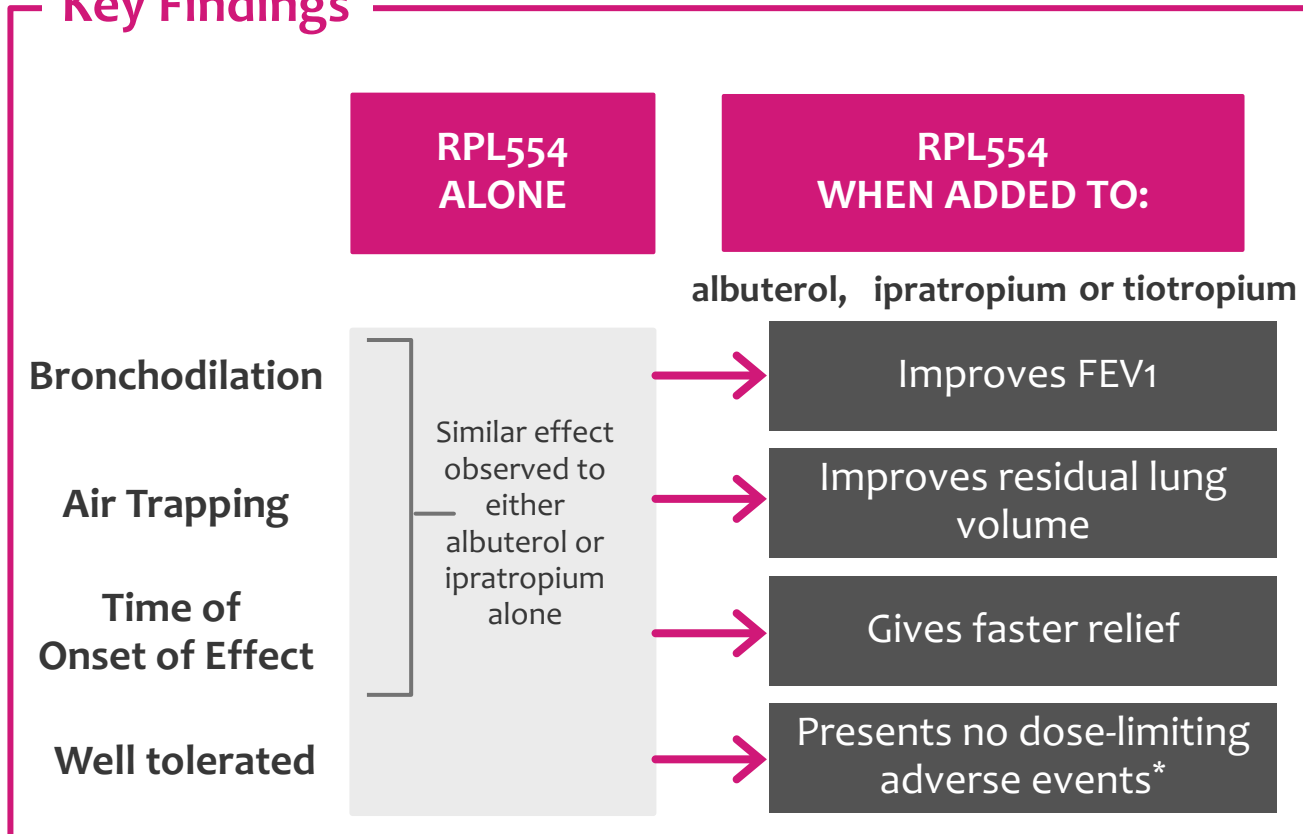
Improvement in lung function and reduction of COPD symptoms could potentially reduce COPD exacerbations

RPL554: Add-on Effect to Single Bronchodilator Reproduced in two Independent Studies



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Key Findings



*in completed clinical trials

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

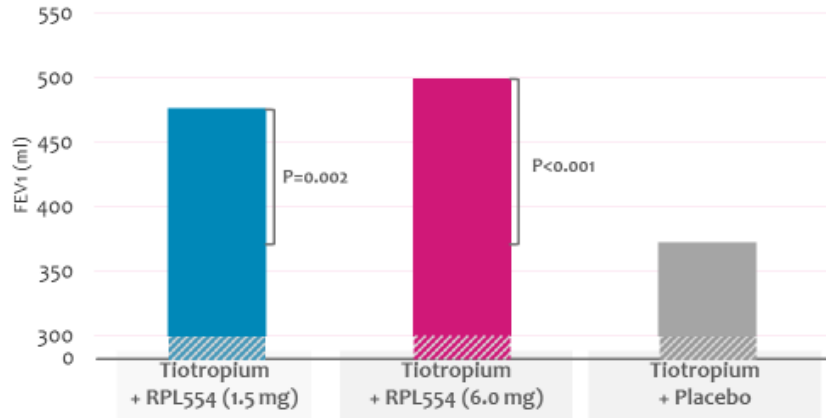
RPL554: Significant Additional Bronchodilator Response when Inhaled on Top of Tiotropium (Spiriva)



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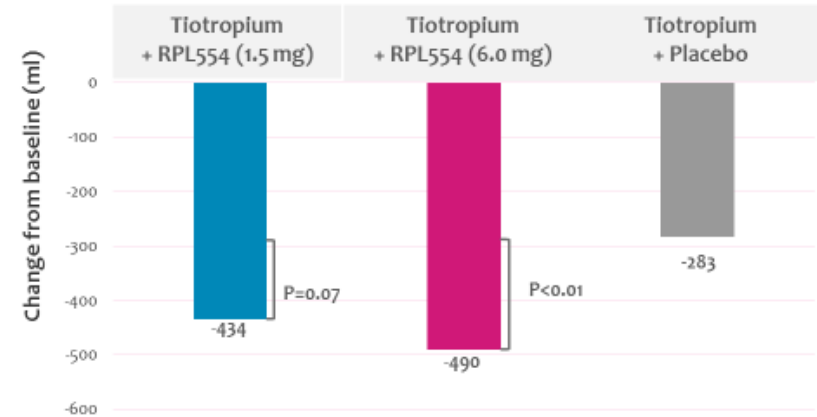
Peak Change from Baseline in FEV₁ (ml) on Day 3

N=27-28



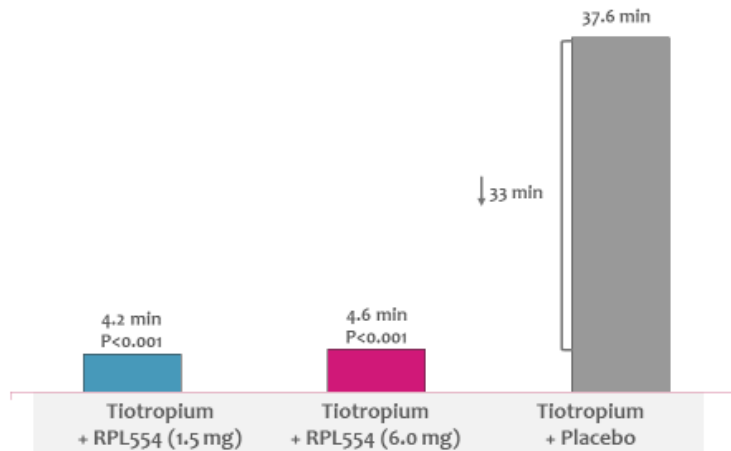
Reduction in Hyperinflation (ml) on Day 2

N=27-28



Median Time to Onset ($\geq 10\%$ improvement in FEV₁; mins) on Day 3

N=27-28



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

Evaluating RPL554 as Add-on to Dual Bronchodilator Treatment in COPD Patients



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Ongoing clinical study, data expected 1Q19

Trial Description:

- Phase 2 randomized, double blind, placebo controlled, cross-over study
- Three day treatment with baseline to peak FEV1 on Day 3 as primary endpoint
- Assess nebulized RPL554 as add-on to LAMA/LABA treatment; some patients will maintain stable dose of ICS providing a triple background

Patient Population:

- About 75 moderate-to-severe COPD patients
- Males and females, age 40-75

Location:

- Centres in US and UK

RPL554 Dosage:

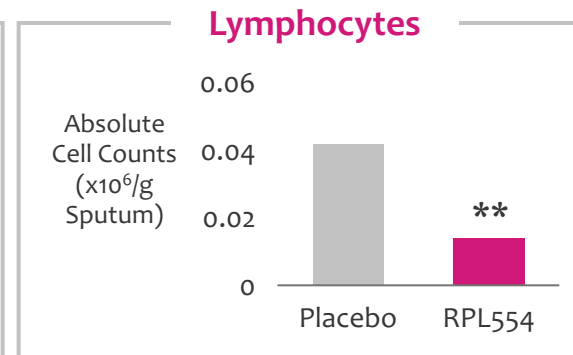
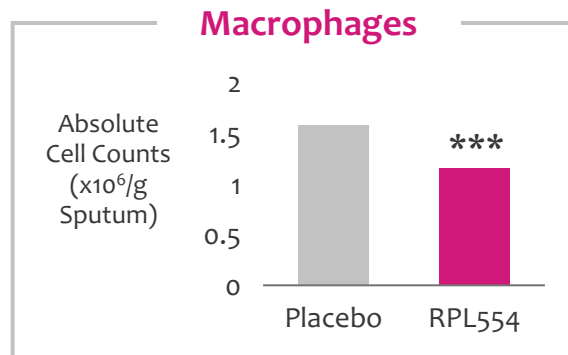
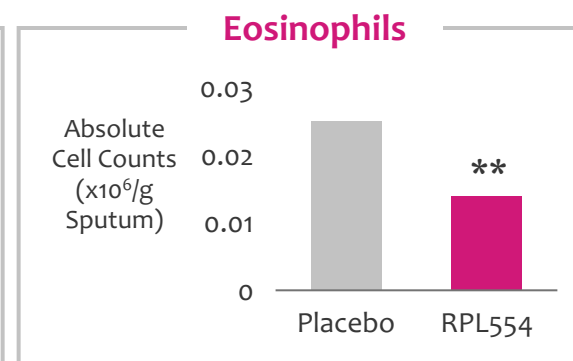
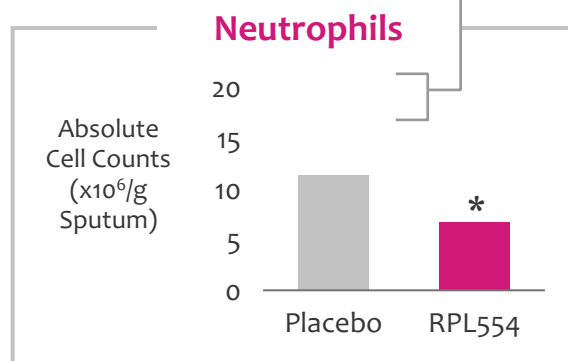
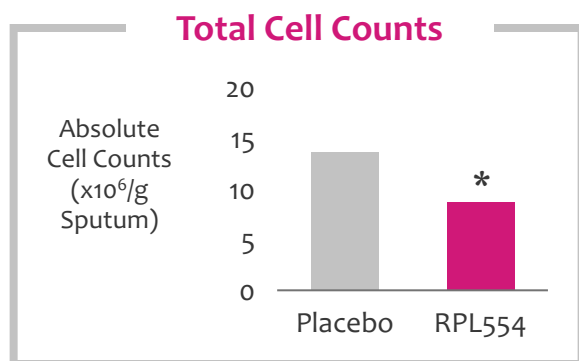
- Three arms, twice daily dosing with RPL554 at 1.5 mg and 6 mg or placebo

RPL554: Broad Anti-Inflammatory Activity

Reduction in Inflammatory Cells

■ RPL554 (n=21)
■ Placebo (n=21)

- Significantly lower absolute number of neutrophils in sputum
 - A critical inflammatory cell in COPD
- Inhaled corticosteroids have no effect on neutrophils



Source: Study VRP 120120, P1 clinical trial; n = 21 healthy subjects; May 2013

* p=0.002

** p=0.001

*** p=0.044



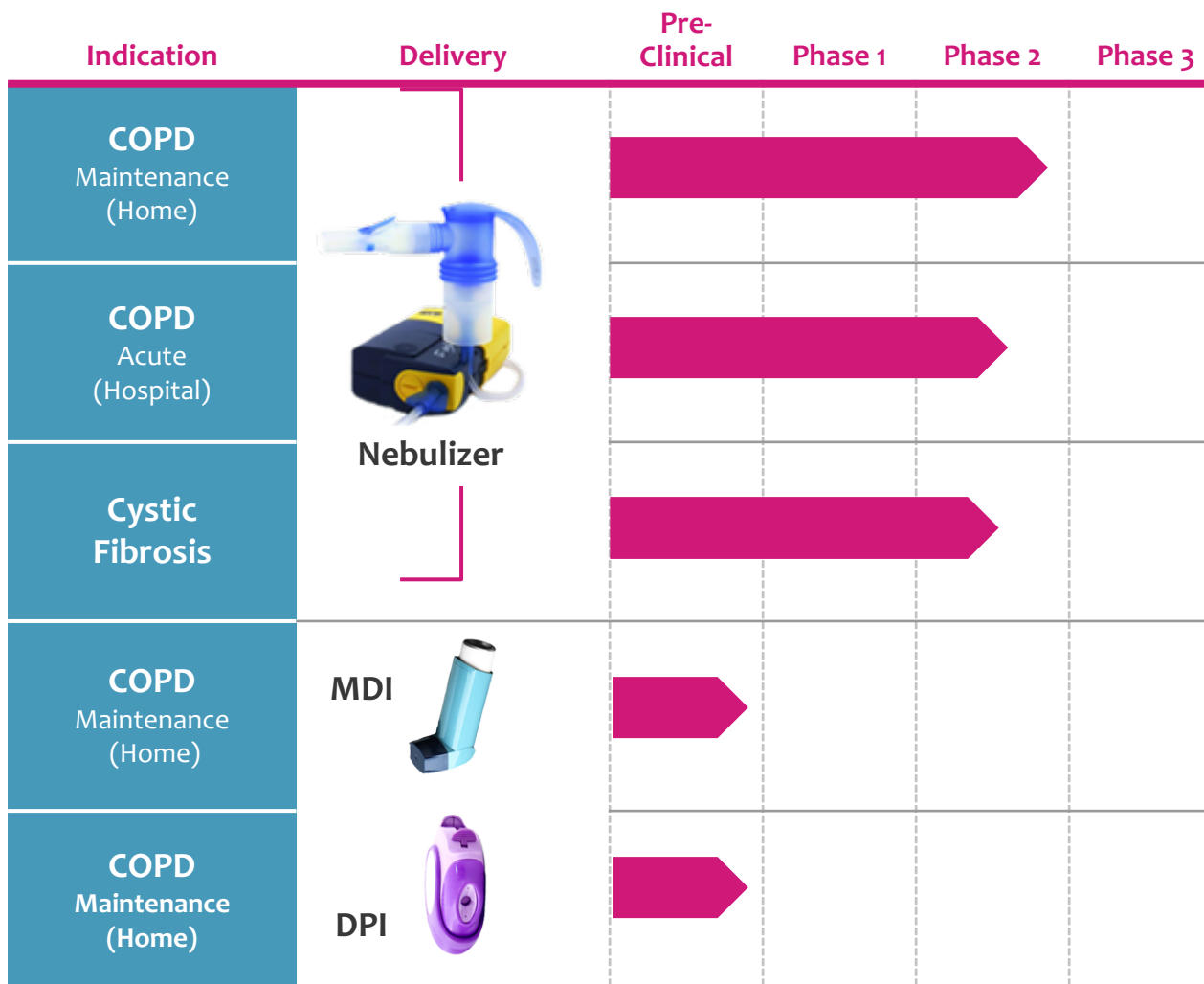
Nebulized RPL554: Heading into Phase 3

- Pivotal trials in moderate to severe/very severe COPD 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. FEV1, residual volume) and/or symptoms
- Focus on speed and cost in pivotal trials with nebulizer treatment
 - Two trials 3 to 6 months duration; collecting 12 months safety data
 - Pivotal trials planned to commence in 2019
 - NDA filing planned for 2021/22

RPL554: Robust Product Pipeline



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Compelling data in COPD and CF

Additional opportunities in novel inhaler formulations and potentially in asthma

DPI and MDI Formulation of RPL554 - Potential to Expand Commercial Opportunity in COPD

- Inhaler usage for maintenance therapy (U.S. estimates)
 - ~90% of 3.7 million mild/moderate COPD patients
 - ~80% of 2.7 million severe/very severe COPD patients
- Next steps in DPI and MDI formulation development
 - DPI clinical trials planned to start 4Q 2018
 - MDI clinical trials planned to start 1H 2019
- Potential to broaden use in other indications, such as asthma
- Available for out-licensing





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



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- 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
Data to be presented at North American Cystic Fibrosis Conference in October



RPL554 IP Summary

Patent Portfolio:

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

Verona Pharma has global rights



Well Financed with Major Healthcare Investors

Financial Overview June 30, 2018

Cash and Cash Equivalents	\$90.9M ¹
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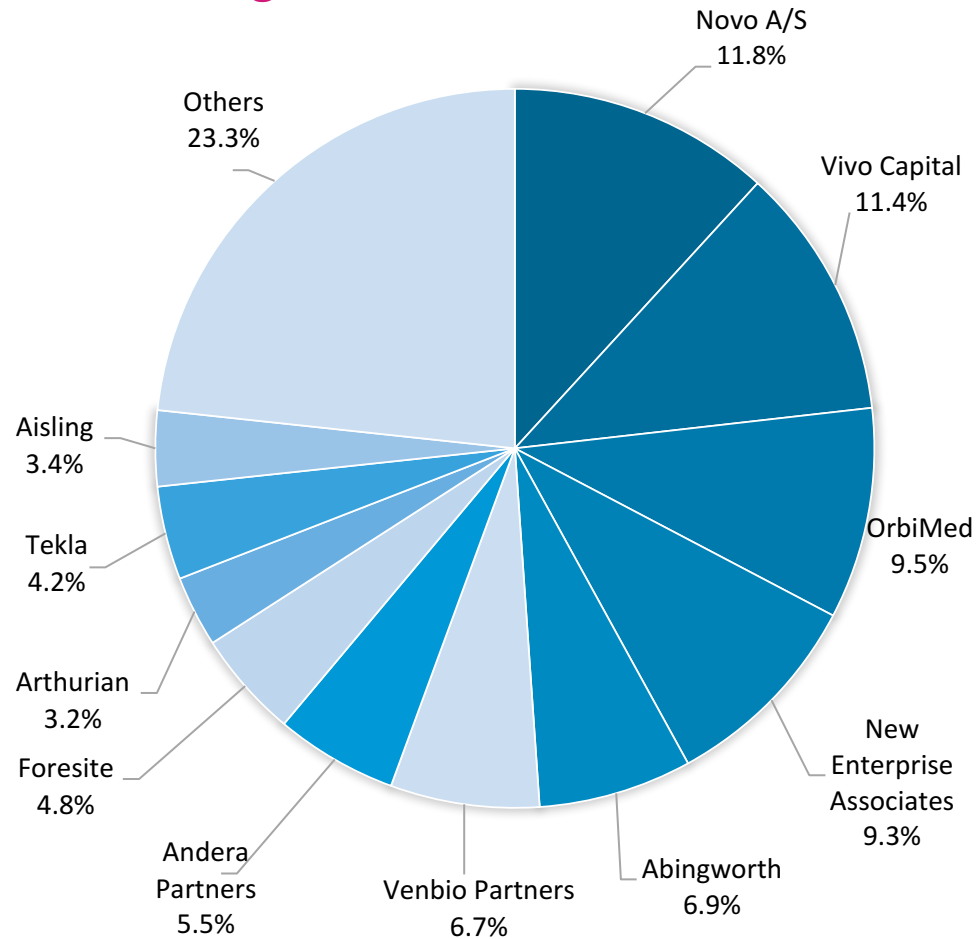
Operating Expenses 2Q18	\$7.5M ¹
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Market cap	\$190M ²
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¹Exchange rate used (US dollars per pound sterling): June 30, 2018 \$1.3197

²Fully diluted 125m shares or 15.6m ADSs, share price 117p August 31, 2018

Shareholdings





Multiple Upcoming Inflection Points

Clinical Development	Timing
Nebulized RPL554 as maintenance treatment of COPD	
Additional data from Phase 2b study to be presented at ERS	Sept 2018
Top-line data from Phase 2 RPL554 as add-on to LAMA/LABA w/wo ICS	1Q 2019
Regulatory clarity on Phase 3 studies	Mid 2019
Nebulized RPL554, start Phase 3 pivotal studies	2H 2019
RPL554 DPI and MDI	
DPI start of clinical Phase 2 trials	4Q 2018
Top-line data from Phase 2 DPI studies	1H 2019
MDI start of clinical Phase 2 trials	1H 2019
Estimated top-line data MDI Phase 2 trials	2H 2019
RPL554 anti-inflammatory treatment in Cystic Fibrosis	
Clinical anti-inflammatory / Proof-of-concept study	TBD

RPL554 – unique PDE3/4 inhibitor with bronchodilator and anti-inflammatory effects
Advancing nebulized RPL554 into pivotal Phase 3 trials in COPD in 2019
Significant commercial opportunities

Back up



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Experienced Management Team and Board

Management

Jan-Anders Karlsson, PhD
Chief Executive Officer

Piers Morgan, MA, ACA
Chief Financial Officer

Richard Hennings, BSc
Commercial Director

Peter Spargo, PhD
SVP CMC

Claire Poll, LLB
Legal Counsel

Desiree Luthman, DDS
VP Regulatory Affairs



Board

David Ebsworth, PhD
• Ex CEO Vifor Pharma; CEO Galenica

Jan-Anders Karlsson, PhD
• CEO Verona Pharma

Ken Cunningham, MD
• Chair Abzena plc
• Ex Chair Prosonix; CEO SkyePharma

Rishi Gupta, JD
• Private Equity Partner, OrbiMed

Mahendra G. Shah, PhD
• Managing Director, Vivo Capital
• Ex Chair CEO, NextWave Pharmaceuticals, First Horizon Pharma

Andrew Sinclair, PhD
• Partner and Portfolio Manager, Abingworth

Vikas Sinha, CPA
• Ex EVP, CFO, Alexion

Anders Ullman, PhD, MD
• Ex Head R&D, Baxter Biosciences; EVP R&D, Nycomed Pharma

In team's prior lives ...

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

including

