Verona Pharma

Breathtaking science

Developing respiratory drugs to improve health and quality of life

Wedbush PacGrow Healthcare Conference
New York City, August 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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3rd leading cause of death in US

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

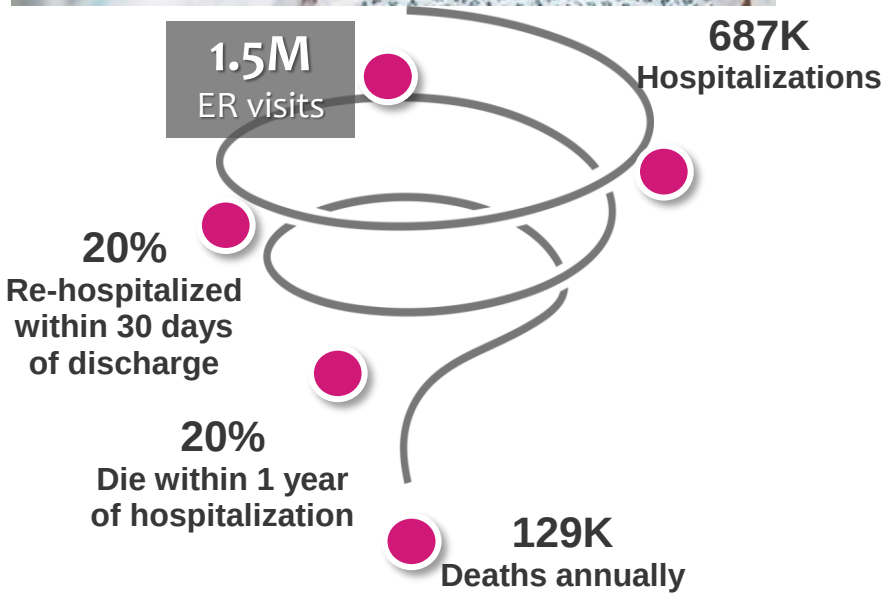
24 million US patients with COPD

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

Unmet Need For New Treatments:

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

Consequences of severe exacerbations



~$32 billion US annual direct healthcare costs[1]

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

Compelling Need For Therapy with New Mode of Action for COPD

… but few such drugs in development for COPD

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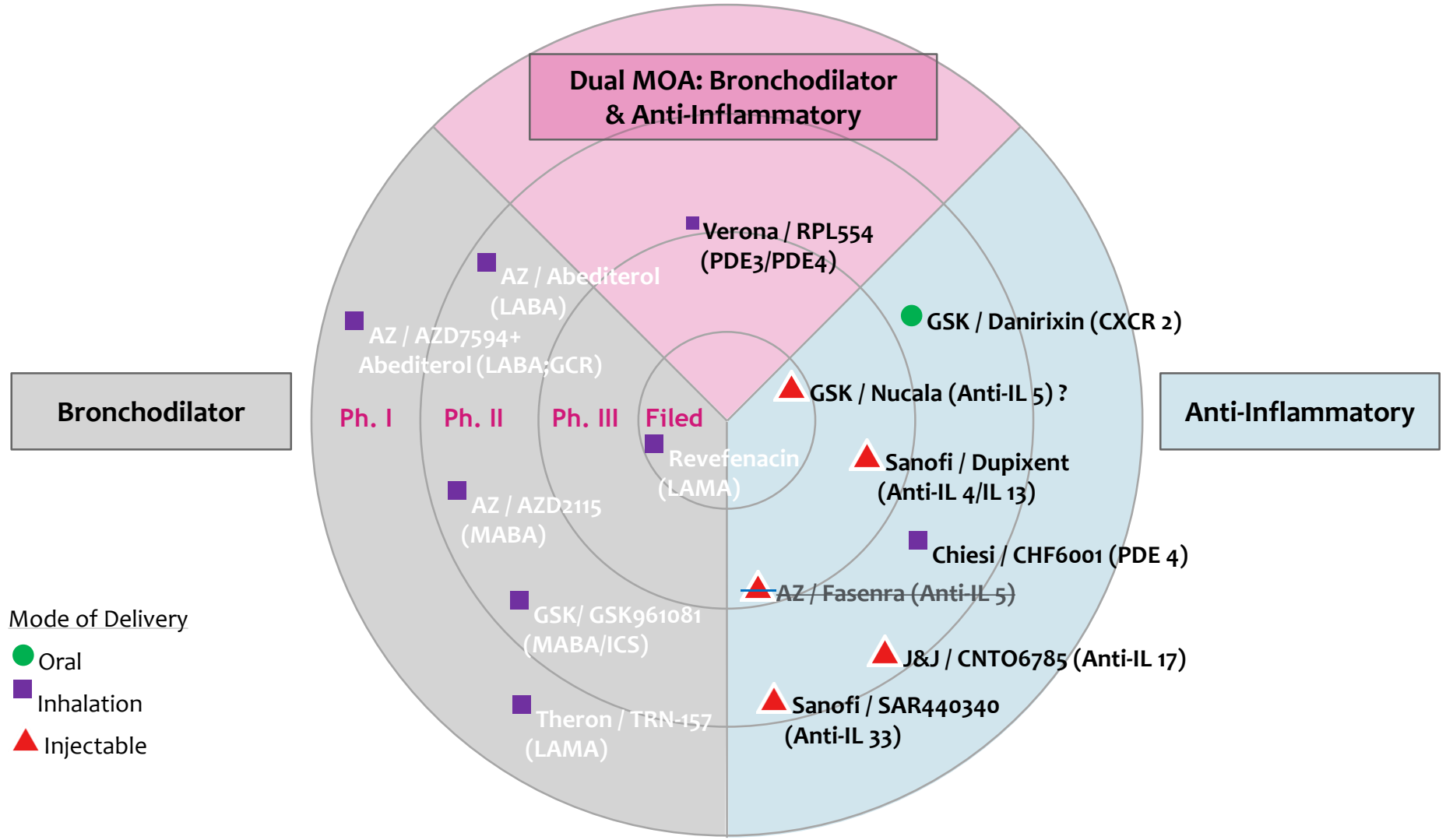

Includes NMEs in development for maintenance COPD only (excludes generic forms and reformulations / device combinations of existing compounds
Source: EvaluatePharma and STRH market intelligence

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

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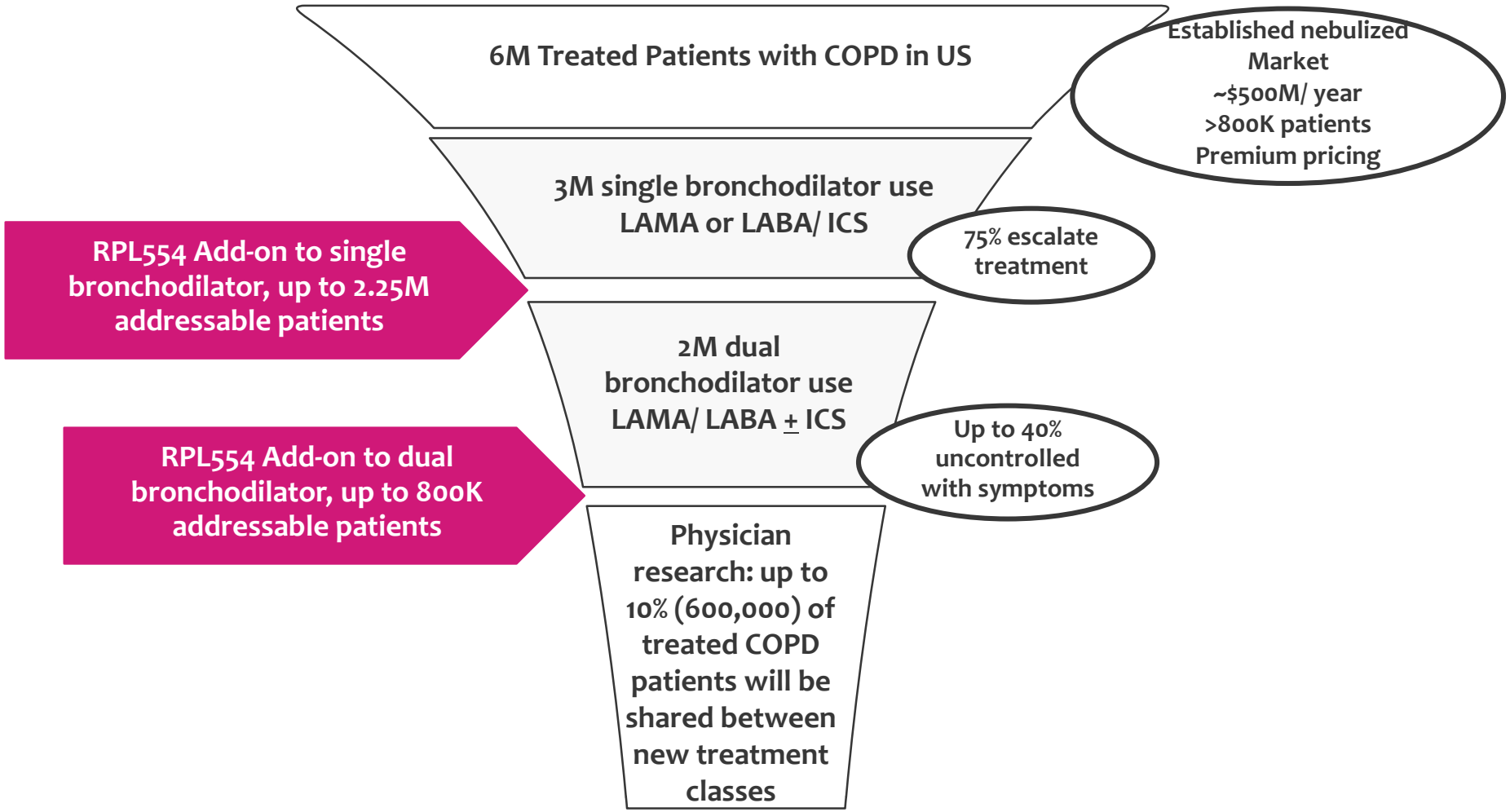
Large numbers of uncontrolled and symptomatic COPD patients



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 (TRINTY); a double-blind, parallel group, randomised controlled trial, The Lancet, Vol 389, p. 1919-1929; May 13, 2017

Nebulized RPL554: Significant Commercial Opportunity in Maintenance Treatment of COPD

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	Add-on to single bronchodilator (LAMA or LABA +ICS)	Add-on to dual bronchodilator (LAMA + LABA or LAMA+LABA+ICS)
Est. total US patient population	3,000,000	2,000,000
Est. patient population with uncontrolled symptoms	Up to 75% escalate to next line therapy within 12 mo	Up to 40% continue to have symptoms Up to 800,000 patients
RPL554 market opportunity	Physician research anticipate up to 10% of treated patients will be shared between new treatment classes	
Reference WAC price per month	Reference to nebulized LABA; ~$970/mo	Reference to nebulized LABA ~$970/mo

Sales ($) of common bronchodilators in US	Avg monthly $ WAC price (Oct 17-Jan 18)	US $ sales May16-Apr17 ($ Million)
Advair Diskus (LABA+ICS)	398	1,094
Spiriva Respimat (LAMA)	398	1,779
Anoro Ellipta (LAMA+LABA)	398	277
Brovana (Neb. LABA)	971	339
Perforomist (Neb. LABA)	972	155
Trelegy Ellipta (Triple)*	530	-

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

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

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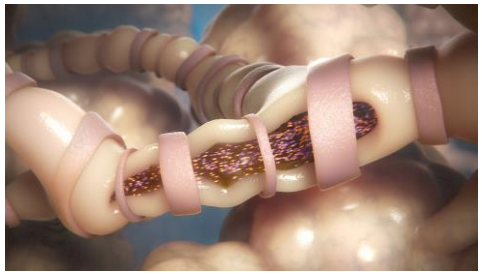
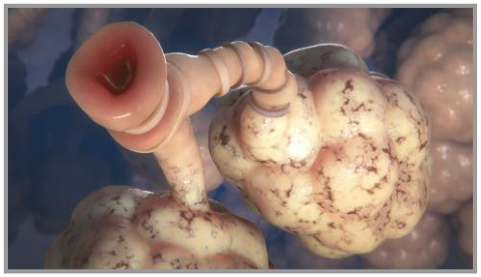
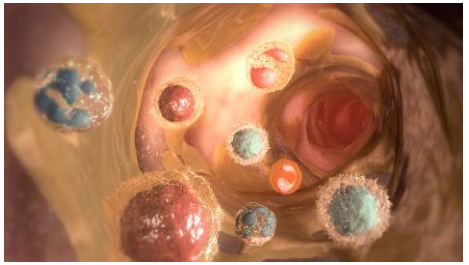
RPL554
Dual **PDE3** and **PDE4** enzyme inhibitor

Impacts 3 Key Mechanisms in Respiratory Disease:







Airway Smooth Muscle

PDE3, PDE4

Relaxation

↓

Increased bronchodilation

Inflammatory Cells

Neutrophils
PDE4

Epithelial cells
PDE3, **PDE4**

Macrophages
PDE3, **PDE4**

Eosinophils
PDE4

Lymphocytes
PDE3, **PDE4**

Fibroblasts
PDE4

↓

Increased anti-inflammatory effects

Epithelial Cells

PDE3, **PDE4**

CFTR Activation

↓

Increased mucociliary clearance

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

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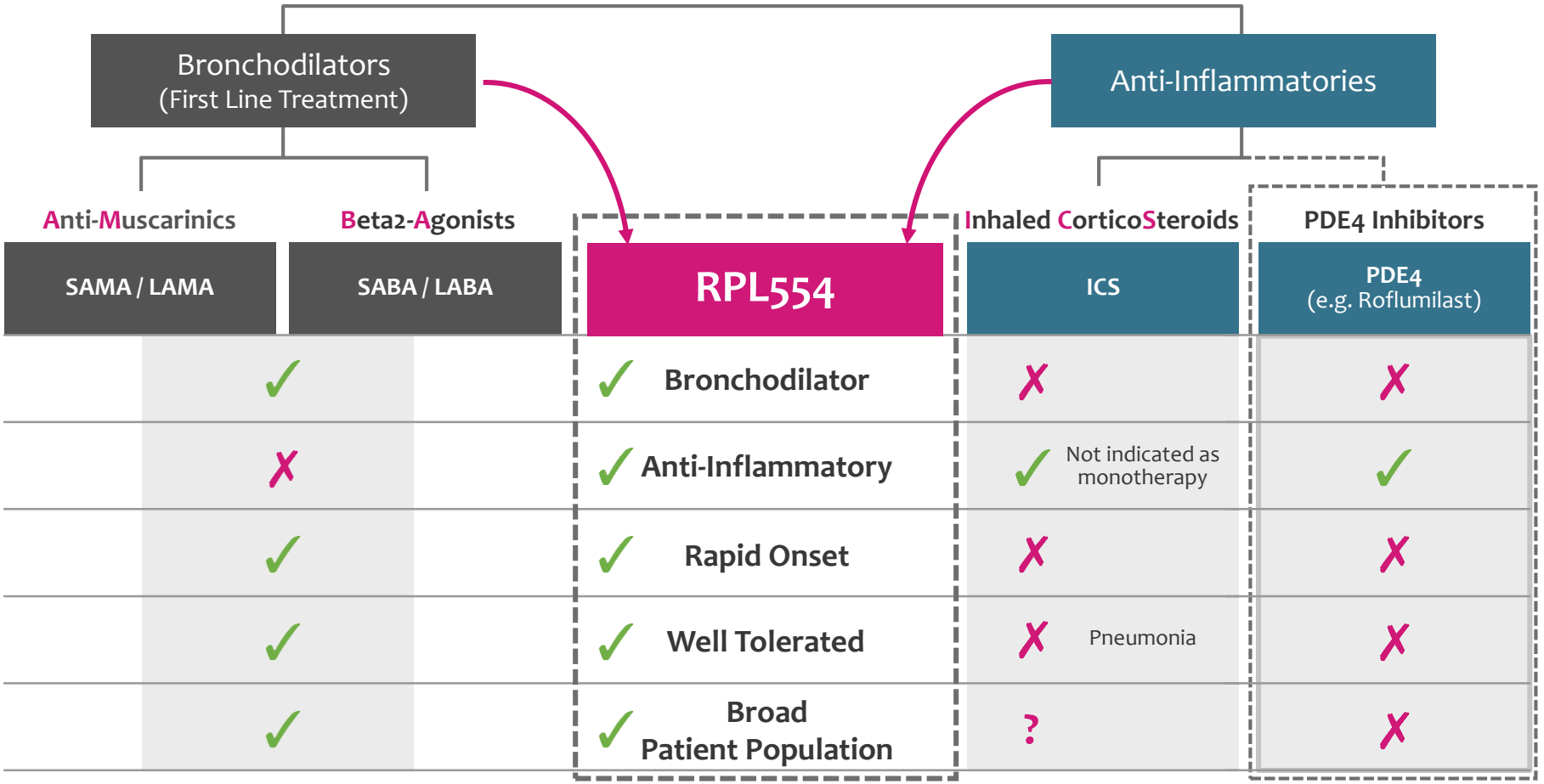

New therapies required for patients with progressive disease and symptoms despite current treatment options

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

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Trial	Program	# of Subjects	Duration	Status
Completed 5 studies in 105 subjects with RPL554 initial proof-of-concept solution formulation				
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 (tiotropium/olodaterol; Stiolto Respimat)	~75	Dose twice daily for three days	Started July 2018

RPL554: Four Week Phase 2b Study in Moderate to Severe COPD

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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

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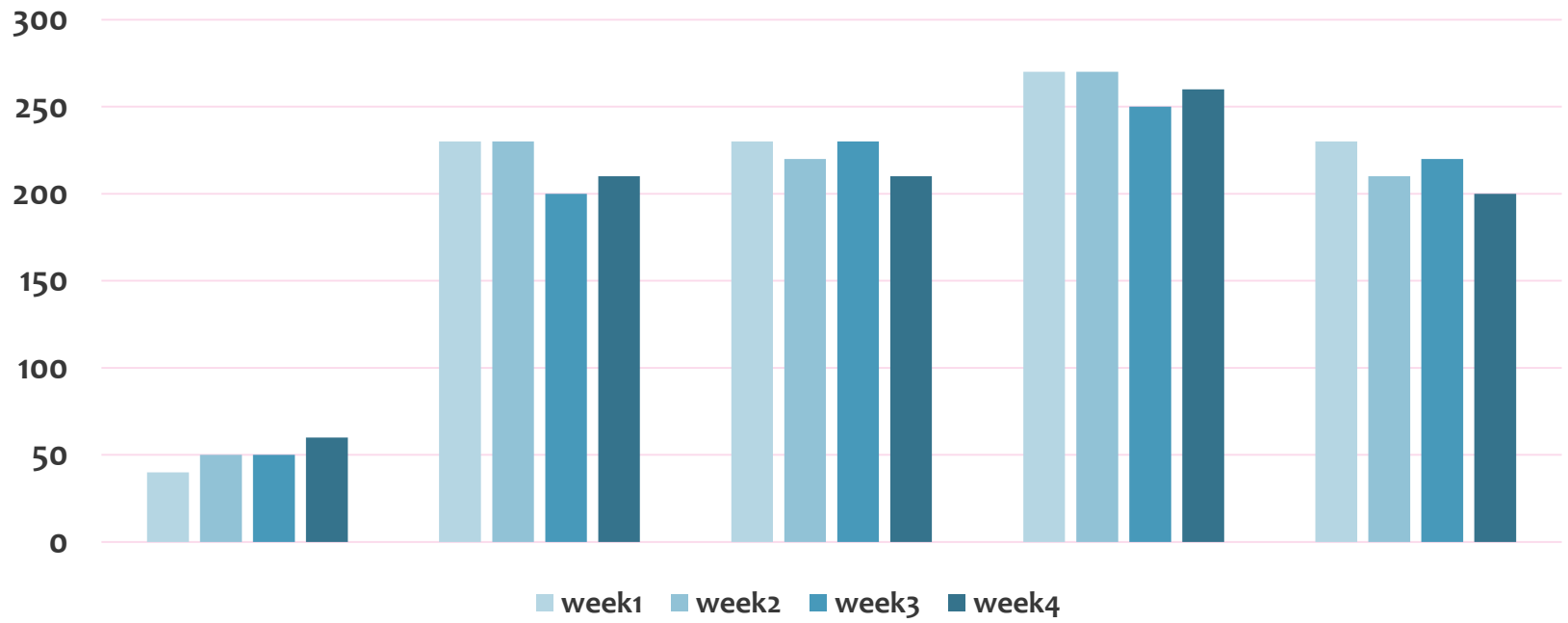
Peak Change from Day 1 in Baseline in FEV_1 (mL) on week 4 ($p<0.001$)

N=403



RPL554: Rapid and Progressive Improvement of COPD Symptoms with All Doses from Weeks 1 to 4

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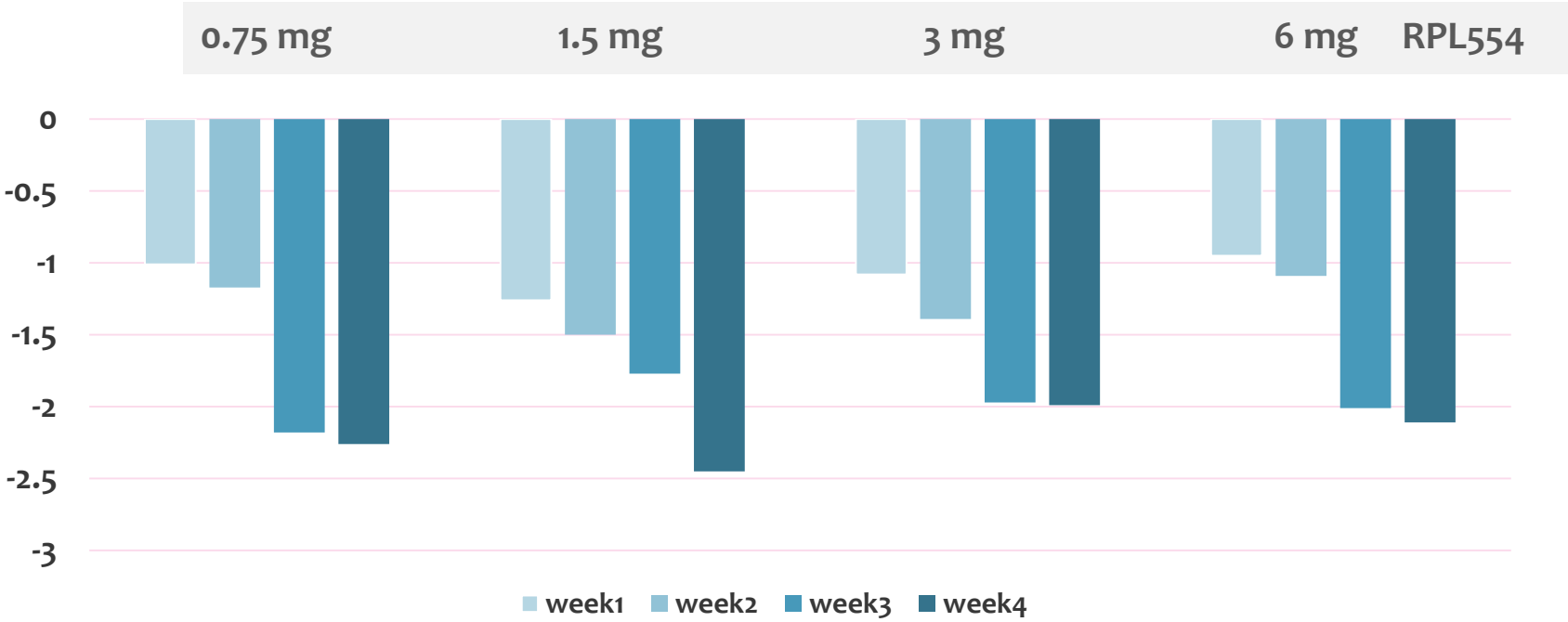
Total score E-RS*: COPD by week (placebo corrected, p<0.02)

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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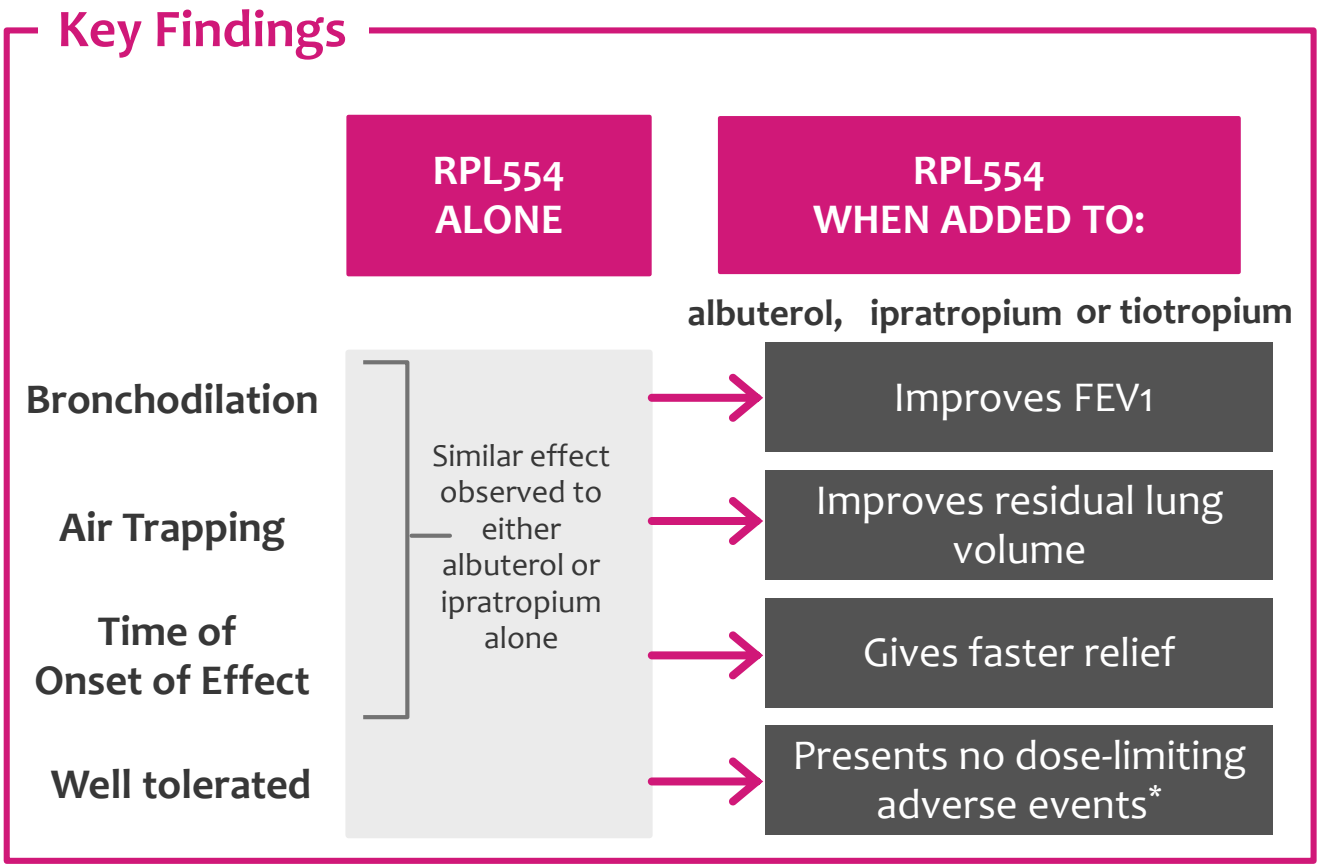
Key Findings



*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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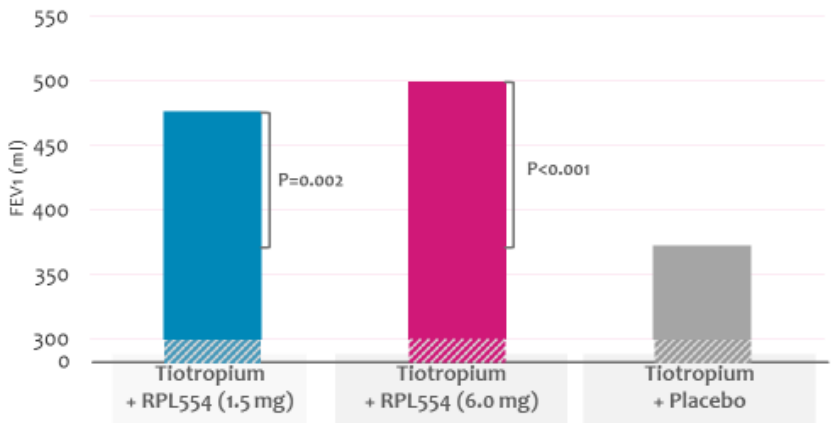
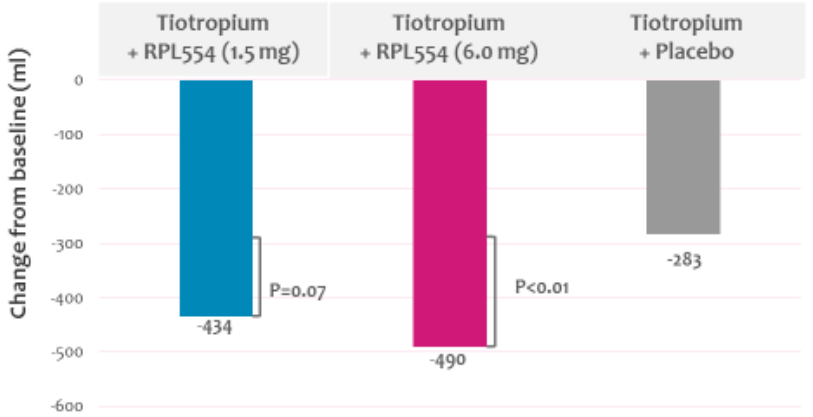
Peak Change from Baseline in FEV_1 (ml) on Day 3

N=27-28



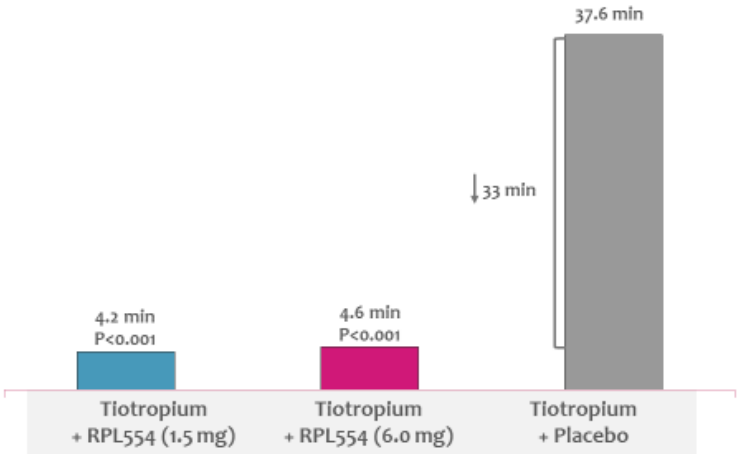
Reduction in Hyperinflation (ml) on Day 2

N=27-28



Median Time to Onset (≥10% improvement in FEV_1; 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

Ongoing clinical study

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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

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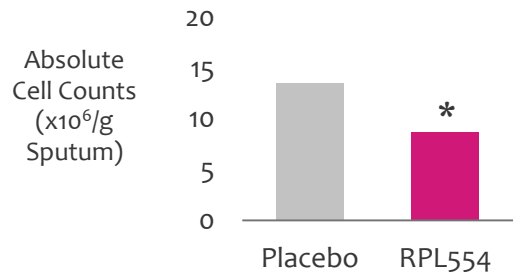
Reduction in Inflammatory Cells

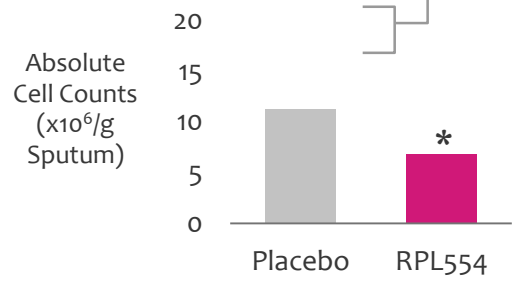
- RPL554 (n=21)
- Placebo (n=21)

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

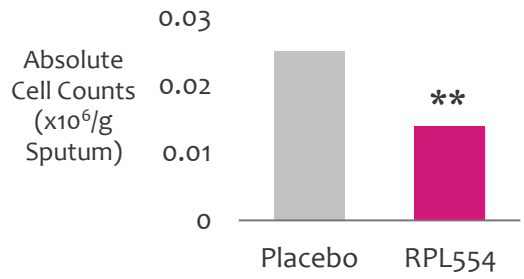
Total Cell Counts



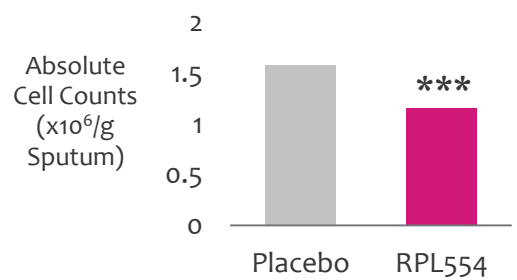
Neutrophils



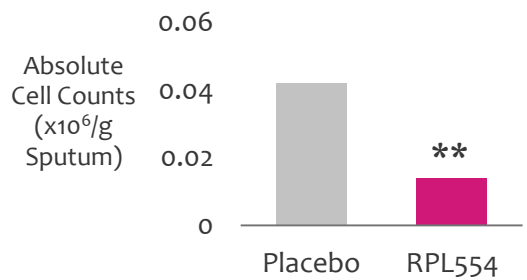
Eosinophils



Macrophages



Lymphocytes



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

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- 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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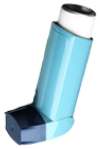
Indication	Delivery	Pre-Clinical	Phase 1	Phase 2	Phase 3
COPD Maintenance (Home)	 Nebulizer				
COPD Acute (Hospital)					
Cystic Fibrosis					
COPD Maintenance (Home)	MDI 				
COPD Maintenance (Home)	DPI 				

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

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- 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

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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
  - 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 the NACF meeting in October

Well Financed with Major Healthcare Investors

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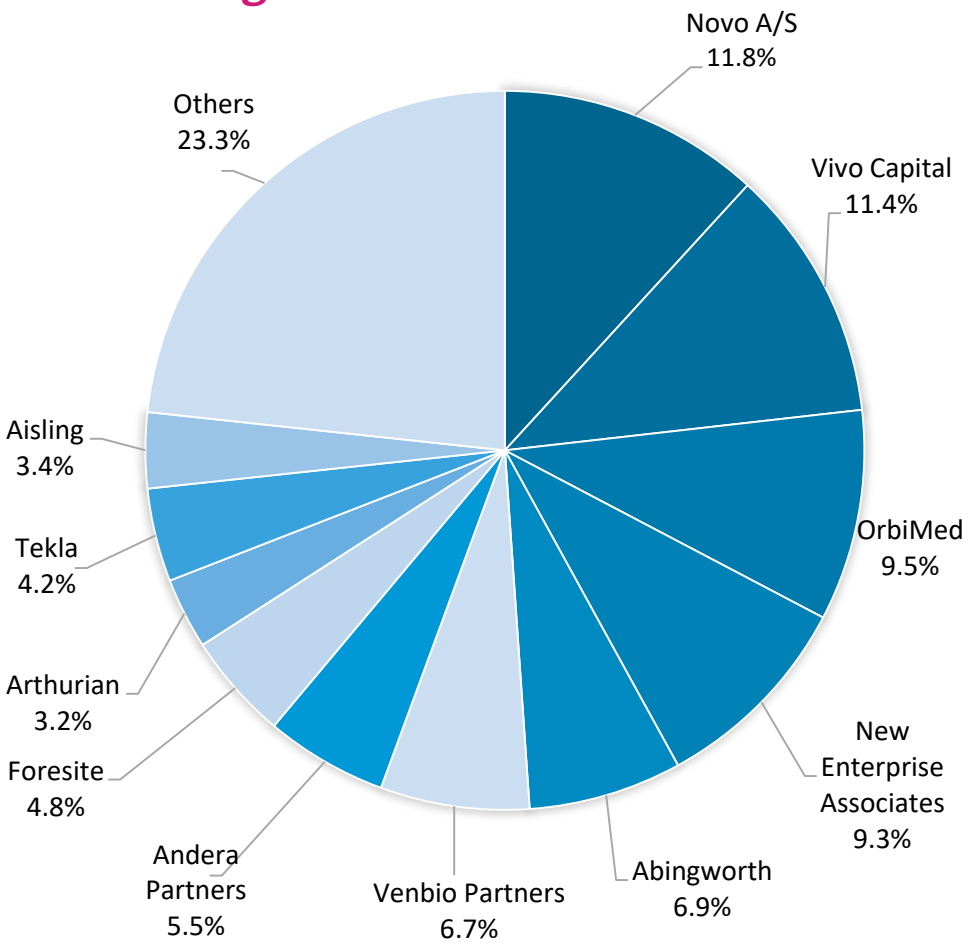
Financial Overview June 30, 2018

Cash and Cash Equivalents	$90.9M[1]
Operating Expenses 2Q18	$7.5M[1]
Market cap	$205M[2]

[1]Exchange rate used (US dollars per pound sterling): June 30, 2018 $1.3197
[2]Fully diluted 125m shares or 15.6m ADSs, share price 123p August 10, 2018

Shareholdings



Multiple Upcoming Inflection Points

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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