Verona Pharma

RPL554: Top-Line Data from Four Week Phase 2b Clinical Trial in COPD Patients

March 26, 2018

RPL554 – Four Week Phase 2b Study in 400 Patients with Moderate to Severe COPD

Verona Pharma

Trial Overview

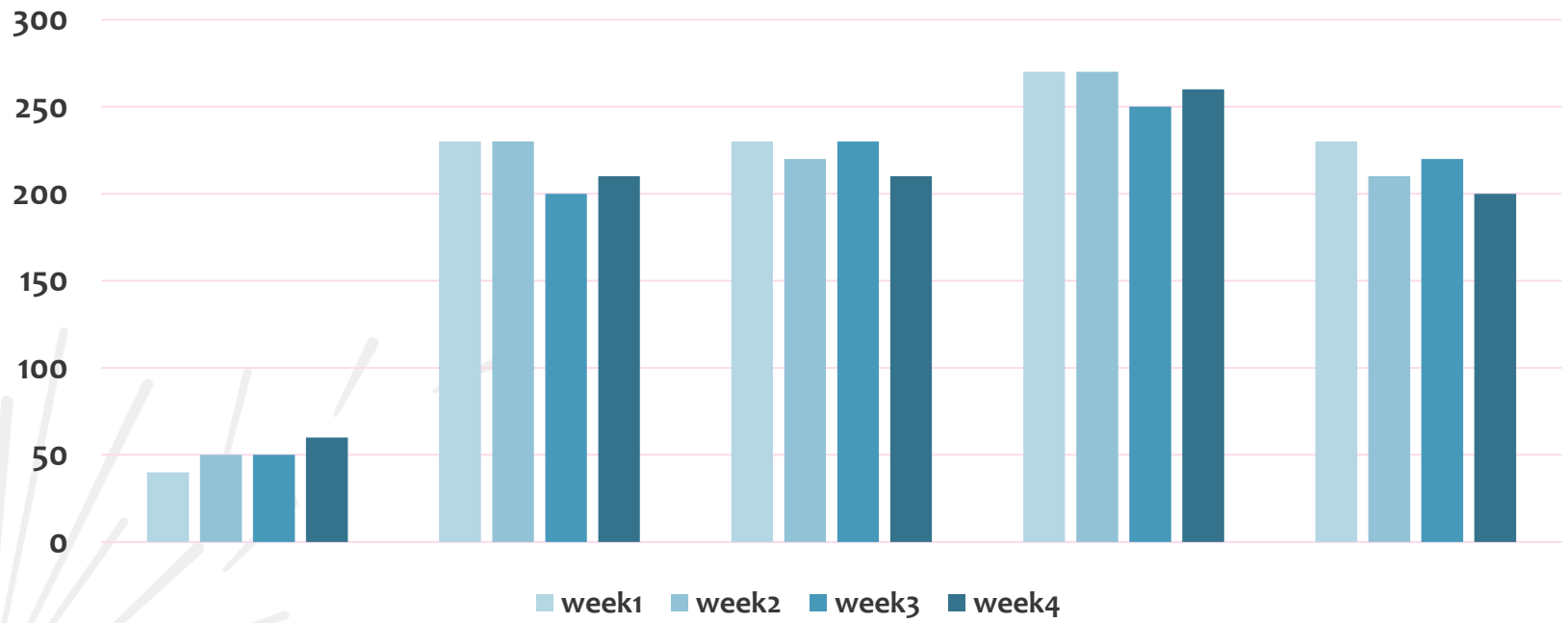
- Trial Description: Phase 2b randomized, double blind, placebo controlled, dose ranging study to assess the effect of nebulized RPL554 in patients with moderate to severe COPD in the outpatient setting
- Patient Population: 403 moderate-to-severe COPD patients diagnosed >12 months previously, males and females, age 40-75
- Location: approx. 45 centres in Western & Eastern Europe
- RPL554 Dosage: Four week, five arm parallel design twice daily dosing with RPL554 at 0.75 mg, 1.5 mg, 3 mg, 6 mg or placebo treatment

RPL554 Provides Significant, Clinically Meaningful Bronchodilator Response that is Maintained over Four Weeks

Verona Pharma

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

N=403

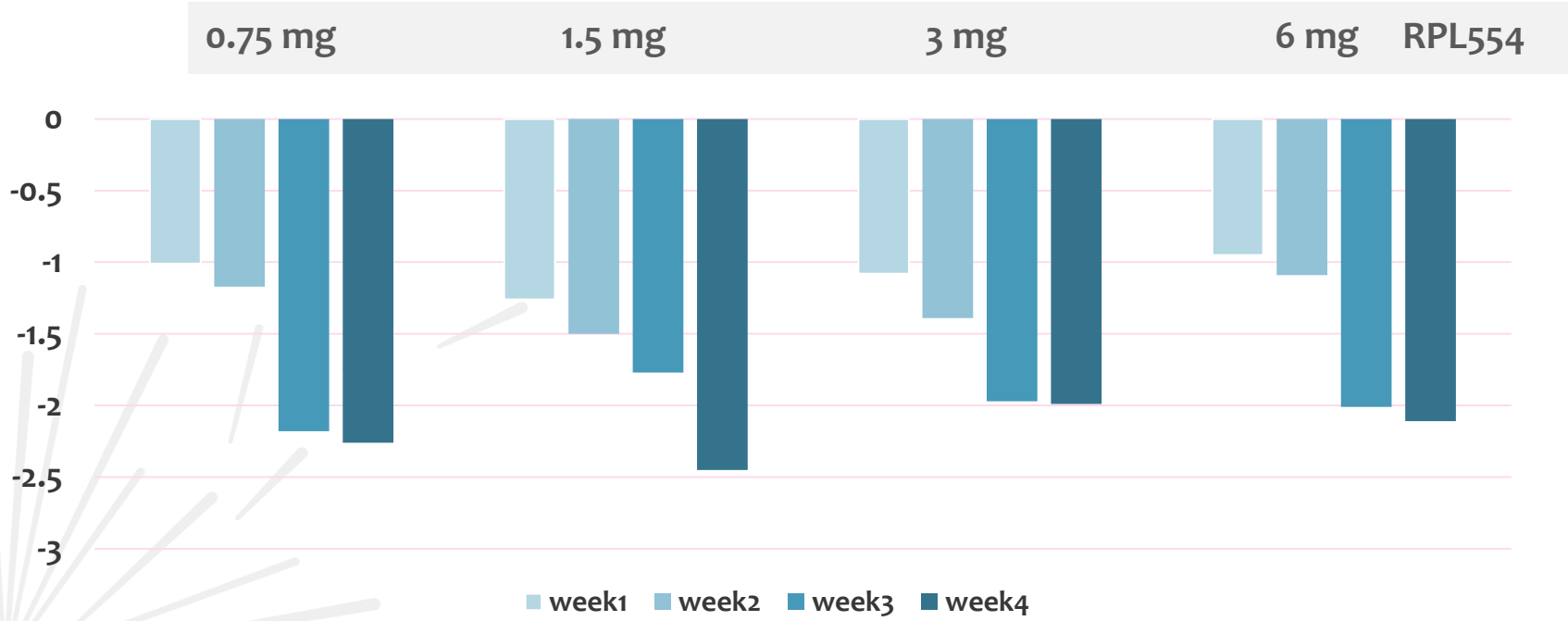


RPL554 Produced Progressive Improvement of COPD Symptoms with all Doses from Weeks 1 to 4

Verona Pharma

Total score (0-40) E-RS: COPD by week (placebo corrected, p<0.02)

N=403



RPL554 Shown to be Effective and Well Tolerated over Four Weeks when Treating COPD Patients in Outpatient Setting

Verona Pharma

Primary endpoint:

- RPL554 met the primary endpoint, peak FEV1; all doses showed a statistically significant difference vs. placebo ($p<0.001$)
- Peak bronchodilator effect observed at first dose, sustained over four weeks ($p<0.001$)

Secondary endpoints include:

- Statistically significant improvement in average FEV1 over 12 hours was observed at all dose levels at the first dose, and the effect was sustained over the four weeks of dosing
- This study did not demonstrate consistent improvements in trough FEV1
- Statistically significant and progressive improvements in daily COPD symptoms, using E-RS ($p<0.02$ improvements in all sub domains of EXACT-PRO)
- RPL554 was well tolerated at all doses with an adverse event profile similar to placebo

Next Steps – Focused Development in Attractive Market Segments

Verona Pharma

Key activities leading up to Phase 3 in maintenance treatment in COPD with nebulized RPL554:

- Positioning study: RPL554 in addition to established combination treatments
- Market research and evaluation of optimal positioning
- “End of Phase 2” meeting to discuss regulatory path
- Pivotal clinical trials in the COPD maintenance setting expected to start in 2019

Development of pMDI and DPI formulations of RPL554:

- Expected start of pre-clinical studies 2H 2018

Development as anti-inflammatory treatment in Cystic Fibrosis:

- Following positive Phase 2a results, KOL and regulatory discussion ahead of clinical proof-of-concept study