

Statistical Analyses for Absolute Change From Baseline in ppFEV1 Through Week 8

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| Statistical analysis title | Statistical Analyses for Absolute Change From Baseline in ppFEV1 Through Week 8 | | Analysis Type | Superiority | | | | |
| Statistical analysis description | Estimates were obtained from MMRM with dependent variable absolute change from baseline, with treatment, sequence, period, and visit within period (Weeks 2, 4, 8) as fixed effects, subject nested within sequence as a random effect, and with adjustment for the continuous baseline values of age and ppFEV1, using a compound symmetry covariance matrix. | | | | | | | |
| Comparison groups or subject analysis sets | Part 1: Ivacaftor versus Part 1: Placebo | | | | | | | |
| Number of subjects in this analysis | 38 | | | | | | | |
| Analysis specification | Pre-Specified | | | | | | | |
| Statistical hypothesis test | | | | | | | | |
| P-value | <0.0001 | | Comment | | | | | |
| Method [Required if P-value provided] | Mixed Effects Model for Repeated Measures (MMRM) | | | | | | | |
| Parameter Estimate | | | | | | | | |
| Parameter type | Least squares (LS) mean difference (Standard Error) | | | | | | | |
| Point estimate | 10.6780 (1.7100) | | | | | | | |
| Confidence interval | Level | 95% | Sides | 2-sided | Lower limit | 7.2559 | Upper limit | 14.1000 |
| Variability estimate (if applicable) | NA | | Dispersion Value | NA | | | | |