

Statistical Analyses for Part 1: Absolute Change From Baseline in BMI Through Week 8

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| Statistical analysis title | Statistical Analyses for Part 1: Absolute Change From Baseline in BMI Through Week 8 | | Analysis Type | Superiority | | | | |
| Statistical analysis description | Estimated change from baseline per 56 days was obtained from a linear mixed model with dependent variable BMI, treatment, sequence, and period as fixed effects, and intercept, visit (days on study, including all visits through Week 8 in each period), and treatment by visit interaction as random effects, with adjustment for baseline ppFEV1 and age. | | | | | | | |
| 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 | P-value for the treatment effect is from the slope of BMI (kg/m ²) versus time (days). | | | | |
| Method [Required if P-value provided] | Linear Mixed Model | | | | | | | |
| Parameter Estimate | | | | | | | | |
| Parameter type | LS mean difference (Standard Error) | | | | | | | |
| Point estimate | 0.6624 (0.1653) | | | | | | | |
| Confidence interval | Level | 95% | Sides | 2-sided | Lower limit | 0.3366 | Upper limit | 0.9881 |
| Variability estimate (if applicable) | NA | | Dispersion Value | NA | | | | |