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BIOS 718

Homework 1

Cystic fibrosis (CF) is a serious genetic disorder that affects about 30,000 people in the United States. Recurrent pulmonary infection caused by the pseudomonas aruginosa bacterial strain is a common complication of CF. A study was conducted to determine if individuals with CF could be effectively treated for infection with aerosolized antibiotic therapy. The study was a double-blind controlled trial where 520 CF patients aged 10 to 60 years were randomized to receive the active treatment or placebo. The pulmonary function test forced expiratory volume in one second (FEV1) was the primary endpoint. Measurements were collected before patients were randomized and again at the conclusion of the 24-week study. The change in FEV1 score from the beginning of the trial to the end of the trial was calculated for each patient. The initial FEV1 score, follow-up FEV1 score, and change in FEV1 score variables are all approximately normally distributed. An ANOVA test was done to determine if there is a difference in mean change in FEV1 score between the active treatment and placebo group. The ANOVA test determined that there is a statistically significant difference in change in FEV1 score between the active treatment and placebo groups (p-value < 0.001). An ANCOVA model was run to determine if initial FEV1 score affected change in FEV1 score when accounting for treatment type. Treatment type was found to be significant (p-value < 0.001) in the ANCOVA model, while initial FEV1 score was found to be borderline significant (p-value 0.05). A linear regression model was run with treatment type and initial FEV1 score as predictors of change in FEV1 score. With placebo as the reference, the slope estimate of active treatment in the regression model was 0.122 (p-value < 0.001). The slope estimate of initial FEV1 was –0.041 (p-value 0.05). The adjusted R2 value of the model was 0.047. These results indicate that patients in the active treatment group had a greater increase in FEV1 score than patients in the placebo group. A second ANCOVA model was run to determine if initial FEV1 score affected follow-up FEV1 score when accounting for treatment type. Both treatment type and initial FEV1 score were found to be statistically significant in the ANCOVA model (p-values < 0.001). A linear regression model was run with treatment type and initial FEV1 score as predictors and follow-up FEV1 score as the outcome. With placebo as the reference, the slope estimate of active treatment in the regression model was 0.122 (p-value < 0.001). The slope estimate of initial FEV1 was 0.959 (p-value < 0.001). The adjusted R2 of the model was 0.806. In this trial, aerosolized antibiotic therapy was found to be effective in treating infection in individuals with CF. More research can be done, however, to determine how baseline FEV1 score affects follow-up FEV1 score when using this treatment.

Table 1

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