# PBC Analysis

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## Background

The Data used was a Mayo Clinic sutdy of Primary Biliary Cirrhosis (PBC) done between 1974 and 1984. The study was a randomized trial to test the efficacy of a drug called D-penicillamine. Participants were measured at regular intervals for various symptoms associated with the disease, for example the presence of edema, and also had blood work done to provide additional clues as to the health of the individual. The study ended for each participant when they died, when they received a liver transplant, or when the study ended.

## Data Summary

Most participants remained in the study for 1-5 years, with some participants staying in for longer than the 10 year period of the trial. The majority of the participants had reached an advanced stage of the disease, stage 3 or stage 4, by the time they enrolled in the study.

Below are a few representative graphs of the data. The quantities obtained from bloodwork are shown in Figure 1. The lines mark the median and the 2nd and 3rd quartile. The plots are divided into groups that ended with different outcomes, treatments, and between men and women. A general decrease in albumin levels can be seen in all groups which indicates a decrease in liver health.

The proportion of participants with an important disease symptoms, the presence of ascites as shown in Fig. 2, increase over the course of the study, and this trend appears independent of which treatment group the participants were in.

## Statistical Tests

The difference between the last and baseline data for the blood work is calculated. And a two sample t-test was run to see if there was a difference between the placebo and treated groups. In fact, the data shows that there is no difference between the two groups. Fig. 3 illustrates the two sample t-test for the quantities measured in blood work. All 95% confidence intervals contain 0 as a possible difference between the two groups.

## Conclusion

From this analysis, the drug was ultimately ineffective at improving outcomes for most participants, failing to produce significant differences in outcomes between treatment groups. Intermediate data between the first and last visit was ignored, so there is a possibility that the drug could have short term benefits to the patient.

The study in question had very few men compared to women, and many participants were already in an advanced stage of the disease. Both of these could be sources of bias in the study.

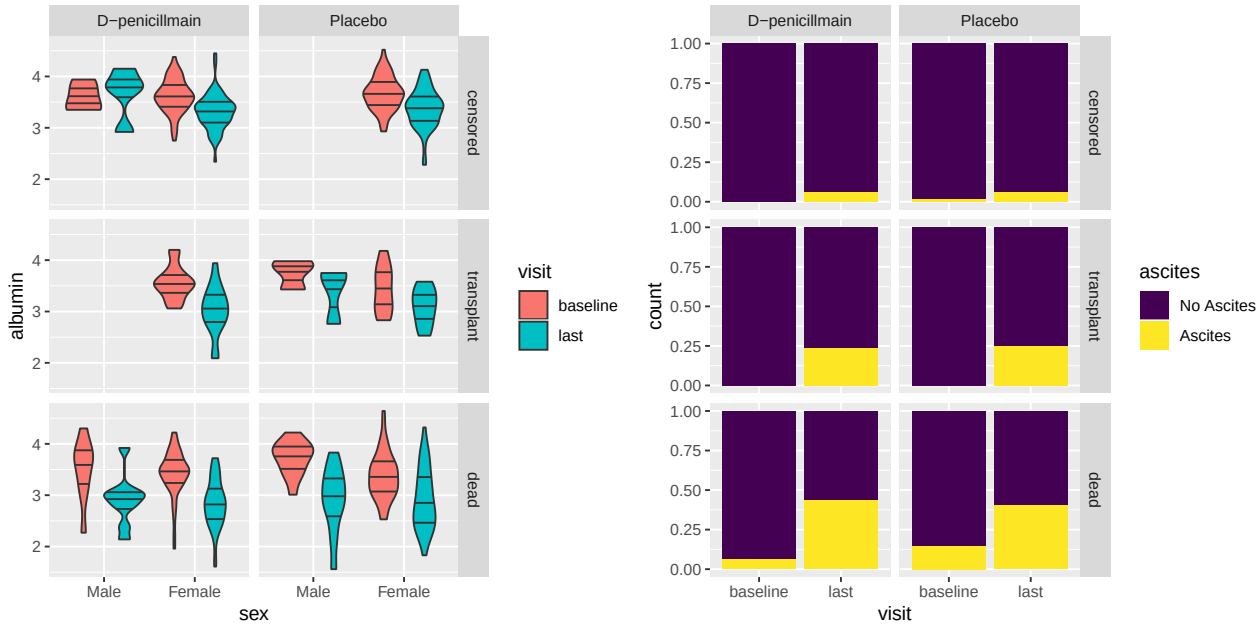


Figure 1: Summary Figures

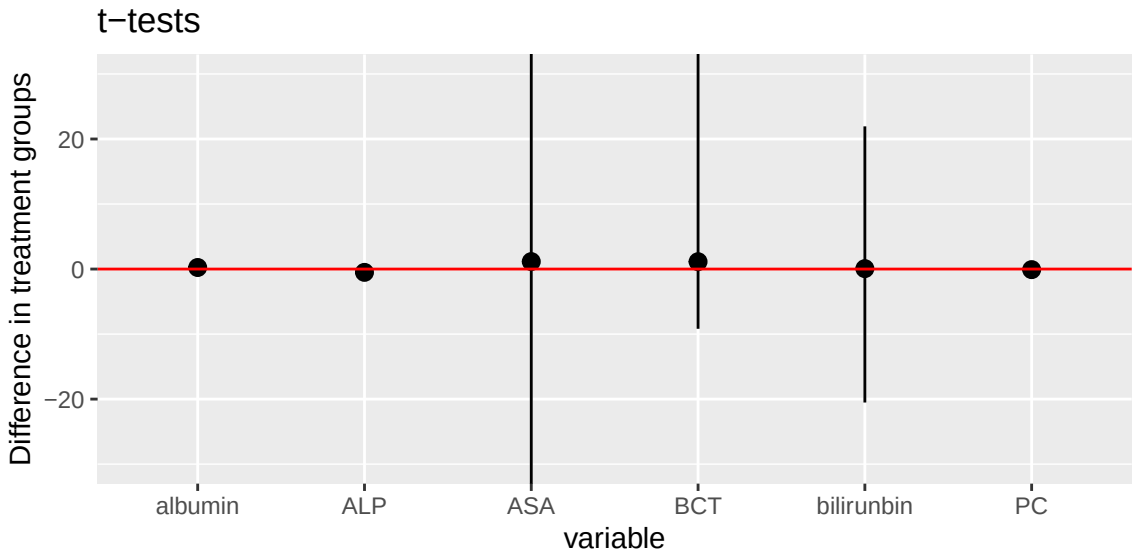


Figure 2: t-tests for blood work