

Exam Practice Questions for PBC Data Analysis

1. Treatment Effect on Survival

Is there any difference in time-to-death (or transplant) between the D-penicillamine and placebo groups?

2. Prognostic Biomarker Panel

Which baseline labs best predict survival? Use penalized Cox models (e.g., horseshoe prior) and compare to LASSO-Cox.

3. Missing-Data Sensitivity

How sensitive are hazard ratios to different imputation strategies for missing labs (e.g., mean imputation vs. Bayesian joint-MVN vs. multiple imputation)?

4. Effect Modification by Stage

Does the effect of treatment vary by histologic stage? Fit a Cox model with an interaction term.

5. Two-Year Mortality Logistic Model

Predict death/transplant within 2 years using logistic regression on age, sex, ascites, bilirubin, albumin, etc. Evaluate with AUC.

6. Cluster Patients by Biochemical Profile

Identify latent subgroups via Gaussian mixture or Dirichlet-process clustering on labs and compare survival across clusters.

7. Natural History vs. Trial Analysis

Replicate the trial's null result in the treated cohort, then fit a multivariable survival model on the pooled cohort without treatment.