SCHOOL OF MEDICINE
North Carolina Translational and Clinical Sciences Institute

CAPSL Request: A dynamic treatment regime estimator for survival outcomes

Jane She[1], Matthew Egberg[2], Michael R. Kosorok[1]
[1]Department of Biostatistics, Gillings School of Global Public Health, UNC
[2]Department of Pediatrics, Division of Pediatric Gastroenterology, UNC

November 2024

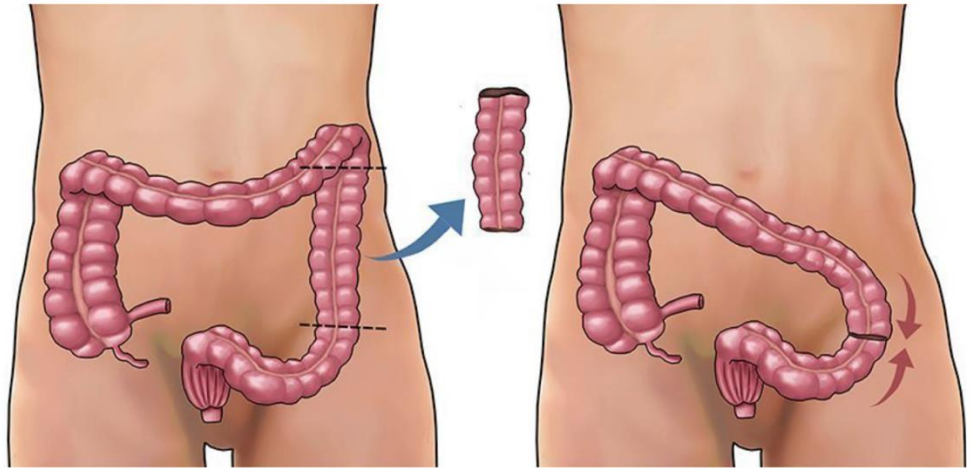
1 Motivation

Crohn's disease (CD) is a chronic autoimmune condition that has not been well characterized in the **pediatric** population due to the limited number of prospective cohort studies. > 50% of patients require surgery within 10 years of diagnosis.

Current postoperative guidelines, to prevent recurrence of CD, is informed by expert consensus rather than evidence-based practice, with significant variance in practice.

2 Research Goal

Estimate an optimal dynamic treatment regime (DTR) to prolong patient survival (time to 2nd resection) for a large number of treatment stages.



3 Simulations

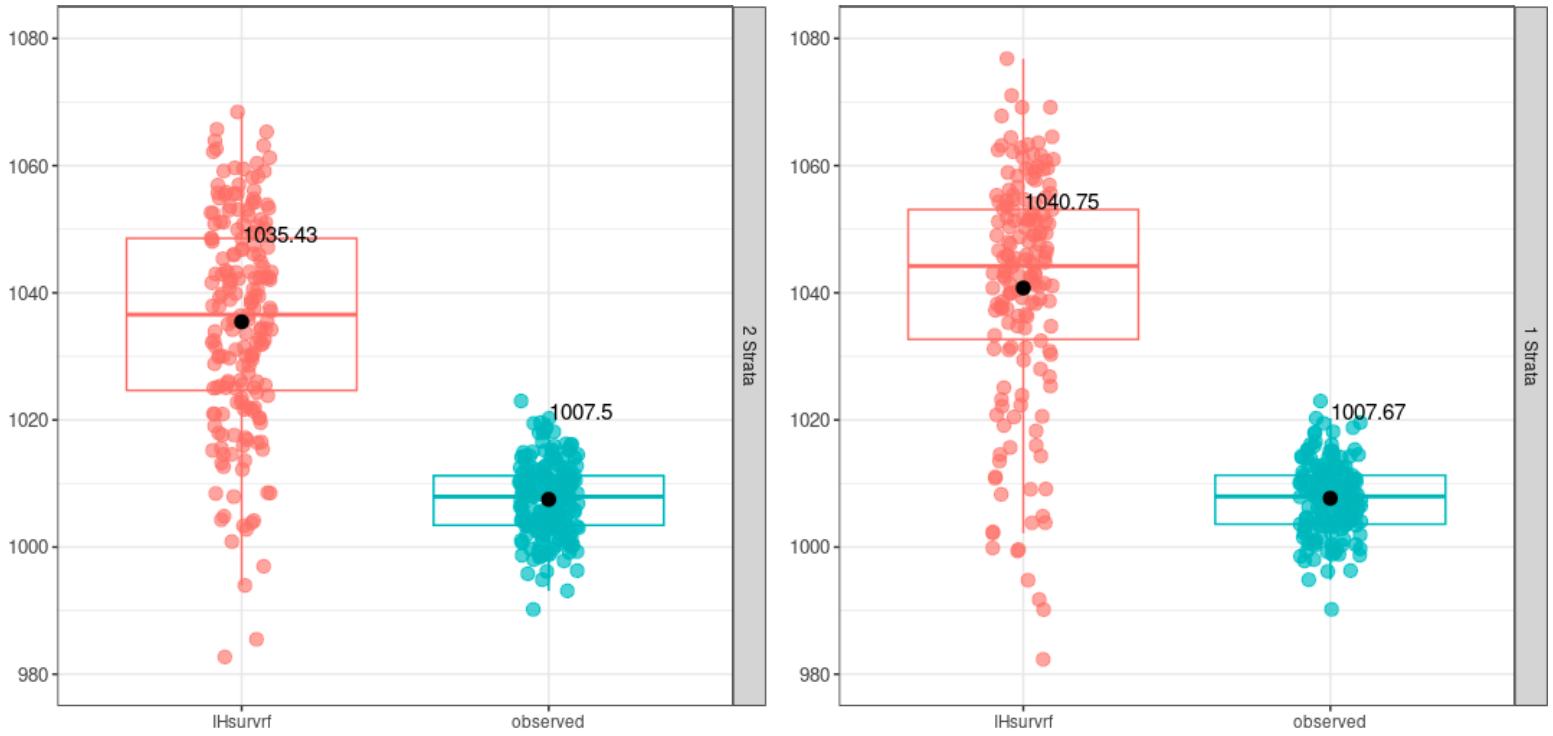


Figure 1: Simulated Example with 15 stages, ~50 % censoring

Here, we have a **sample size of 500** in each setting, with plots differing by the number of strata used.

Under this simulation setting, **A = {0, 1}, T ~ Exp**

Key results:

- The resulting value (mean survival time) of our estimated optimal regime (red) is plotted against the value of the observed regime (blue), with each simulation replicate plotted as a point, and the mean in black.
- Use of our estimator extends survival time in this simulated setting by ~28 days.

4 Application: Pediatric Crohn's Disease

For an application to pediatric CD, we use the IQVIA Pharmetrics dataset which are administrative insurance claims with the following characteristics:

- N = 408
- Maximum number of visits: 20
- % Female (% male): 47.06% (52.94%)
- Treatment: giving any of anti TNF, aminosalicylates, immune modulator, steroid, anti integrin, anti IL, antibiotics

We plan to compare the area under the survival curve of our estimated regime with the observed; higher area under the curve indicates higher average survival time.