Identifying Heterogeneous Treatment Effects in Aggregated N-of-1 Trials Using Bayesian Clustering

Authors and Affiliation

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Background

N-of-1 clinical trials are used to evaluate treatment effectiveness at the individual patient level by repeatedly measuring outcomes under different treatment conditions. When results from multiple N-of-1 trials are aggregated, Bayesian hierarchical models are commonly used to estimate treatment effects while borrowing information across patients. However, hierarchical models assume that all patients borrow strength from a common population parameter, which may mask important heterogeneity in treatment responses. Identifying groups of patients with similar treatment responses is important for improving individualized treatment decisions and advancing precision medicine.

Objectives

This study compares a traditional Bayesian hierarchical model with a Bayesian clustering approach designed to identify groups of patients with similar treatment effects in aggregated N-of-1 trials.

Methods

We developed a Bayesian clustering model that allows patients with similar treatment responses to share information within clusters rather than across the entire population. Posterior inference was obtained using Markov Chain Monte Carlo (MCMC) sampling. Simulation studies were conducted to evaluate the performance of the hierarchical and clustering methods in estimating individual treatment effects and recovering underlying patient group structures. In addition, the methods were applied to a real-world aggregated N-of-1 dataset evaluating treatment effects on patient-reported outcomes.

Results

Simulation results showed that the clustering method more accurately identified groups of patients with similar treatment responses while preserving heterogeneity in treatment effects. Compared with the hierarchical model, the clustering approach reduced excessive shrinkage toward the population mean when subgroup structures were present. In the real data application, the clustering method identified several patient groups with similar treatment effects that were not apparent under the hierarchical model.

Conclusions

Bayesian clustering provides a flexible alternative to hierarchical models for analyzing aggregated N-of-1 trials and detecting heterogeneous treatment responses. These methods may help identify patient subgroups that respond similarly to treatments and support more personalized treatment strategies in clinical research.