

How to extend OHDSI research from average to heterogeneous treatment effects using causal random forest, enabling identification of patient subgroups with differential benefit and advancing precision medicine.

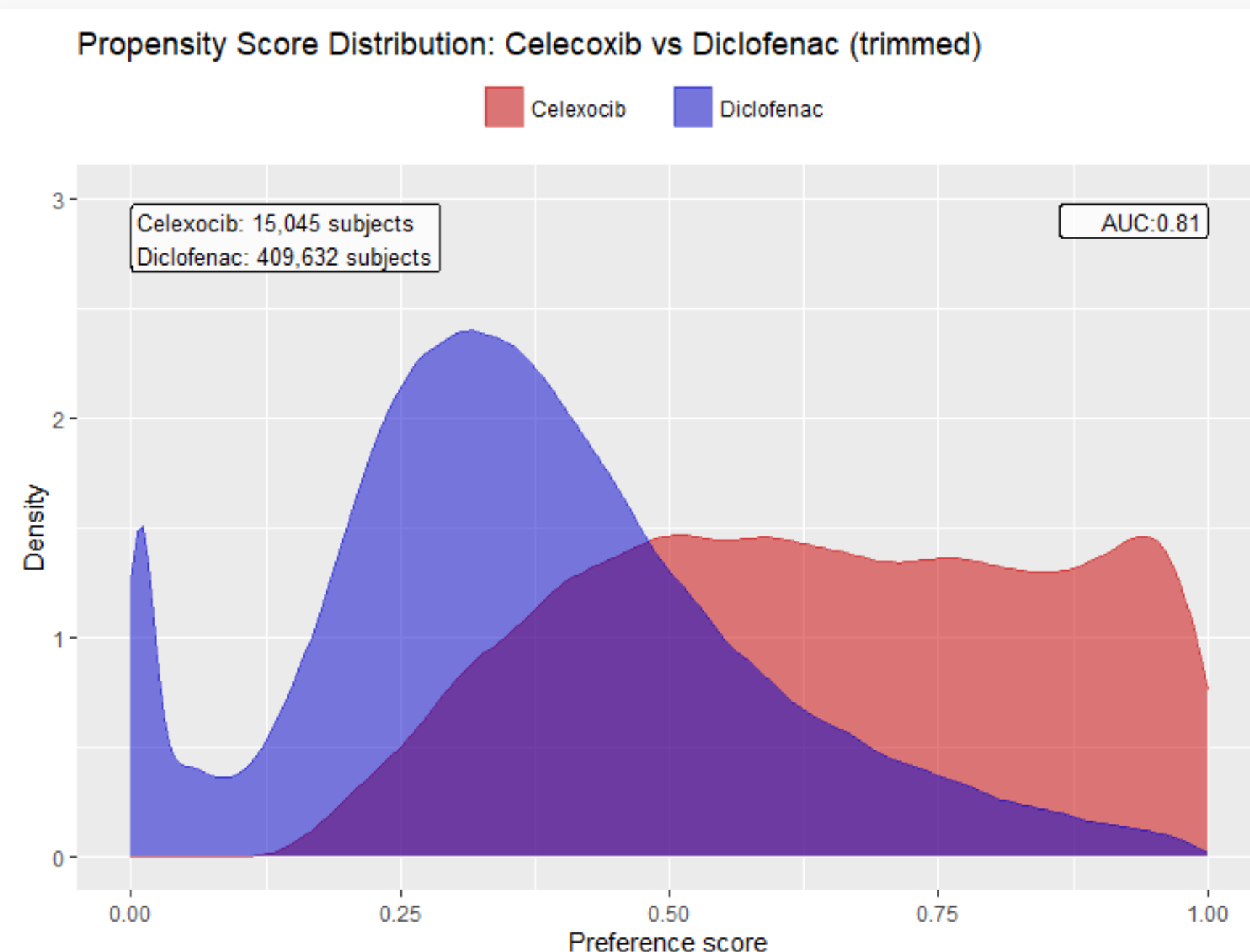
Background: Average treatment effects (ATE) may hide important variability in treatment response, especially in real-world data with complex confounding. Traditional methods like IPTW rely on strong assumptions and may perform poorly in such settings. Doubly robust machine learning algorithm offer a flexible, robust approach to estimate heterogeneous treatment effects (CATE) through **causal random forest**.

Methods

To identify patient subgroups with differential treatment effects between new users of celecoxib and diclofenac on the 30-day risk of gastrointestinal bleeding, we conducted the following analyses using the IPCI database:

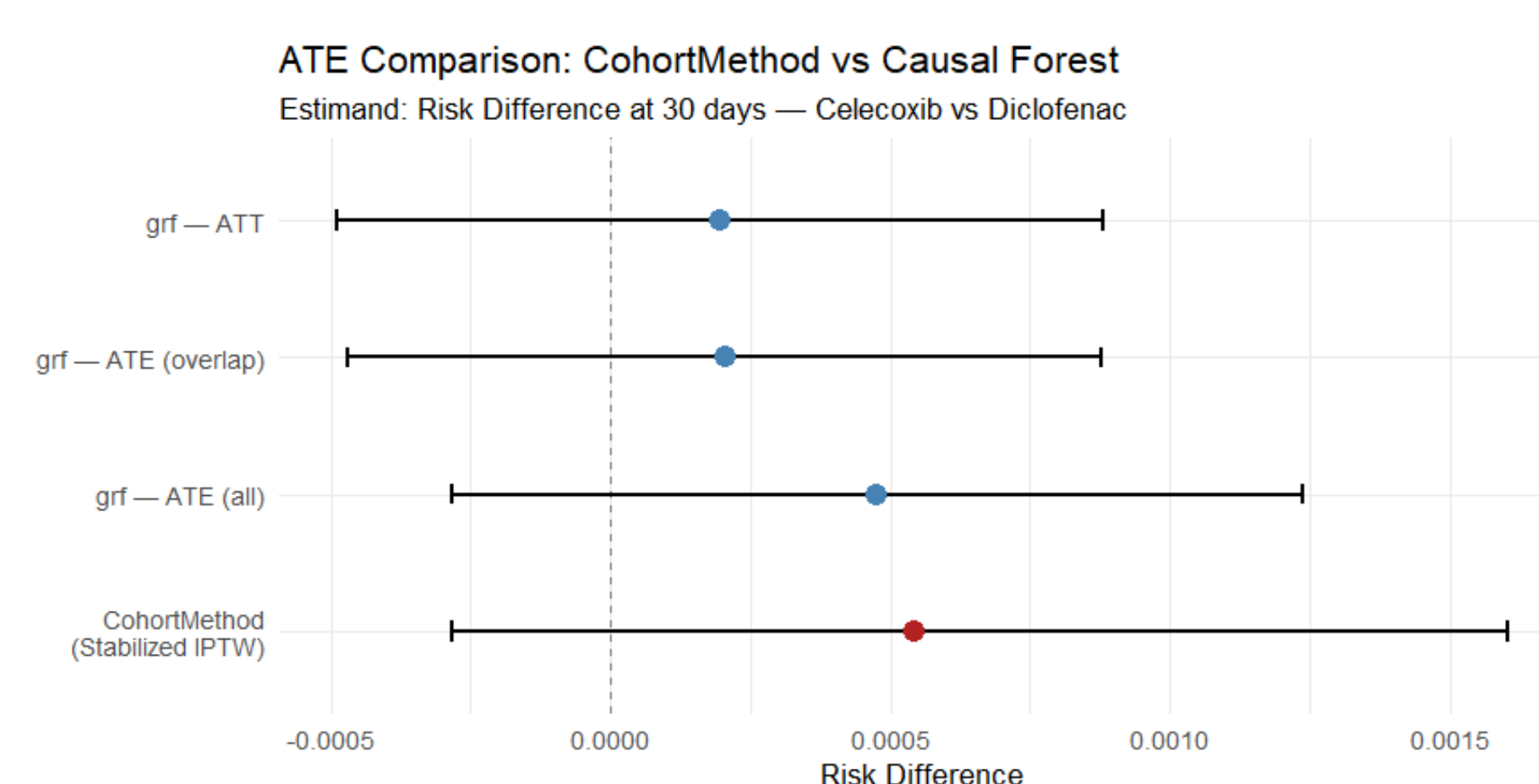
- 1 ATE estimation** using the OHDSI standard **CohortMethod** with a new-user cohort design, we calculated the stabilized *IPTW* and defined ATE as the **Risk Difference** at 30 days
- 2 Implementation of the causal forest** using **grf** R package with the same cohort definition and same covariates, including PS from CohortMethod
- 3 ATE comparison** (CohortMethod vs grf) with a forest plot and estimation of **CATE** to explore heterogeneity in treatment effects across patient subgroups.

Preliminary results



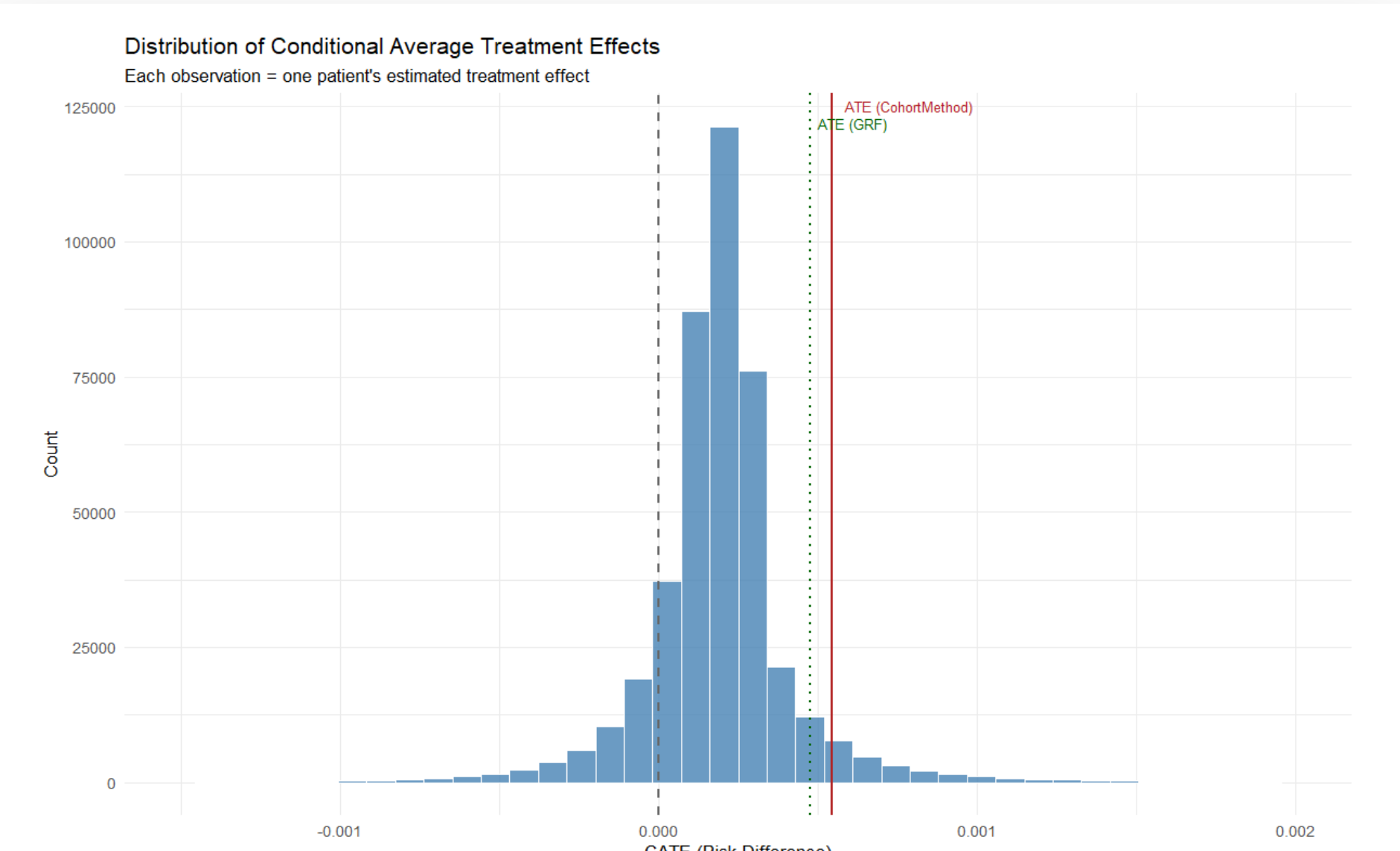
Result 1: PS overlap

Adequate overlap between groups was observed after trimming, despite some imbalances in the tails



Result 2: ATE comparison

Causal forest reproduced ATE estimates from CohortMethod



Result 3: CATE distribution

Despite a modest average effect, variability across patients suggests differences in treatment benefit

Conclusion & future perspective: Starting from these preliminary results, we aim to further investigate how we can assess heterogeneity using observational data using causal machine learning algorithms. While the proposed method shows potential in capturing heterogeneity, further work is needed to better understand its performance in applied settings.

This framework may enable researchers to move beyond average effects and identify patient subgroups most likely to benefit from specific treatments in routine clinical practice using real-world data.

