Heterogeneous Treatment Effect Analysis Based on Machine Learning Methodology

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INTRODUCTION

- Heterogenous treatment effect (HTE) analysis focuses on examining the variation of treatment effects over the population. A good understanding of HTE information can provide a more accurate advice on the treatment plan to achieve optimal outcome (e.g., for personalized medicine), as compared to average effect analysis.
- One unique challenge of conducting HTE analysis is that the quantity to be estimated (i.e., treatment effect) is often unknown on a given dataset, as in many cases each subject can only be exposed to one condition of treatments. Regression-based models, such as the conventional two-step method, were developed to conduct the HTE analysis, but their model performance would be compromised when dealing with nonlinear and/or high-dimensional data.
- Recently, the machine learning (ML) methodologies have been employed in the HTE analysis, especially with the tree-based approaches. As a latest advances in tree-based HTE method, causal forest [1] – a method based on random forest - has been developed to conduct HTE analysis, exhibiting flexibility to handle complex data while sidest epping potential issues by the single-tree method.

OBJECTIVES

• To conduct simulations to mimic scenarios with different levels of complexity in term of HTE, by which the causal forest and two-step method can be systematically examined with respect to the ability to identify the treatment effect heterogeneity.

METHODS

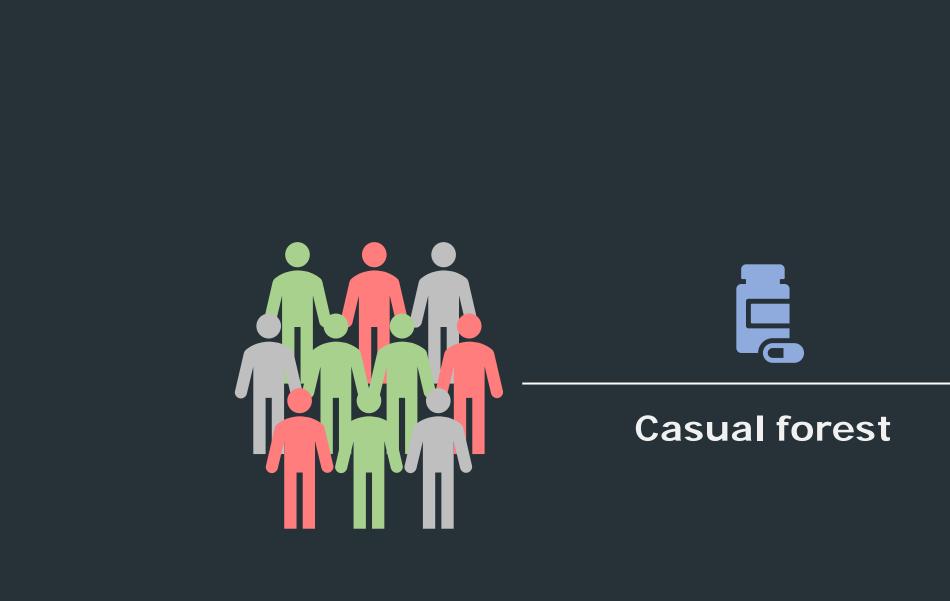
□ Simulation of HTE data

- Interactions between treatment and covariates of subjects can lead to the HTE among the study population.
- By changing the relations of treatment indicator and heterogeneity covariates in the outcome model, various scenarios (models) were created:
 - no heterogeneity covariates
 - inear relationship between heterogeneity covariates
- III. nonlinear relationship between heterogeneity covariates
- IV. high dimensional data

□ ML-based and conventional HTE analysis methods

• We adopted causal forest and the two-step method as the proxies for ML-based and conventional method respectively, for HTE analysis and performance evaluation.

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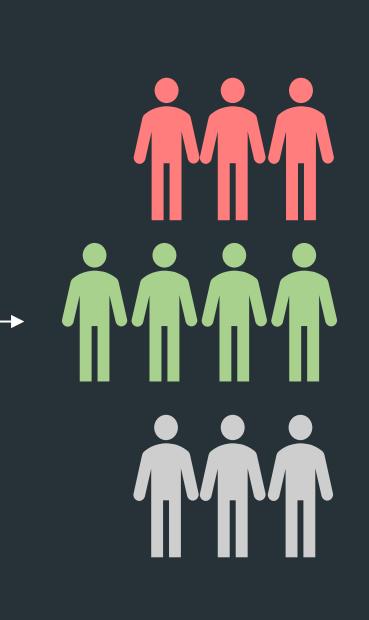


Casual forest, a machine-learningreal-world applications for analysis.

It can potentially be used to improve business intelligence in the Agency.







expected to be harmed by treatment

expected to derive penefit from treatment

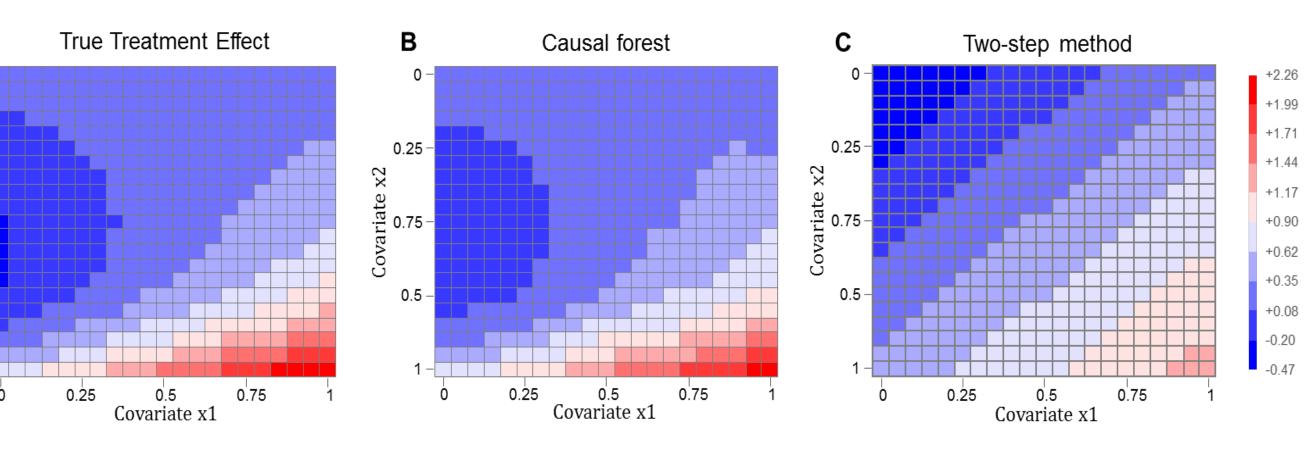
expected to have no treatment effect

based method, is a promising tool in heterogenous treatment effect (HTE)

Take a picture for more details

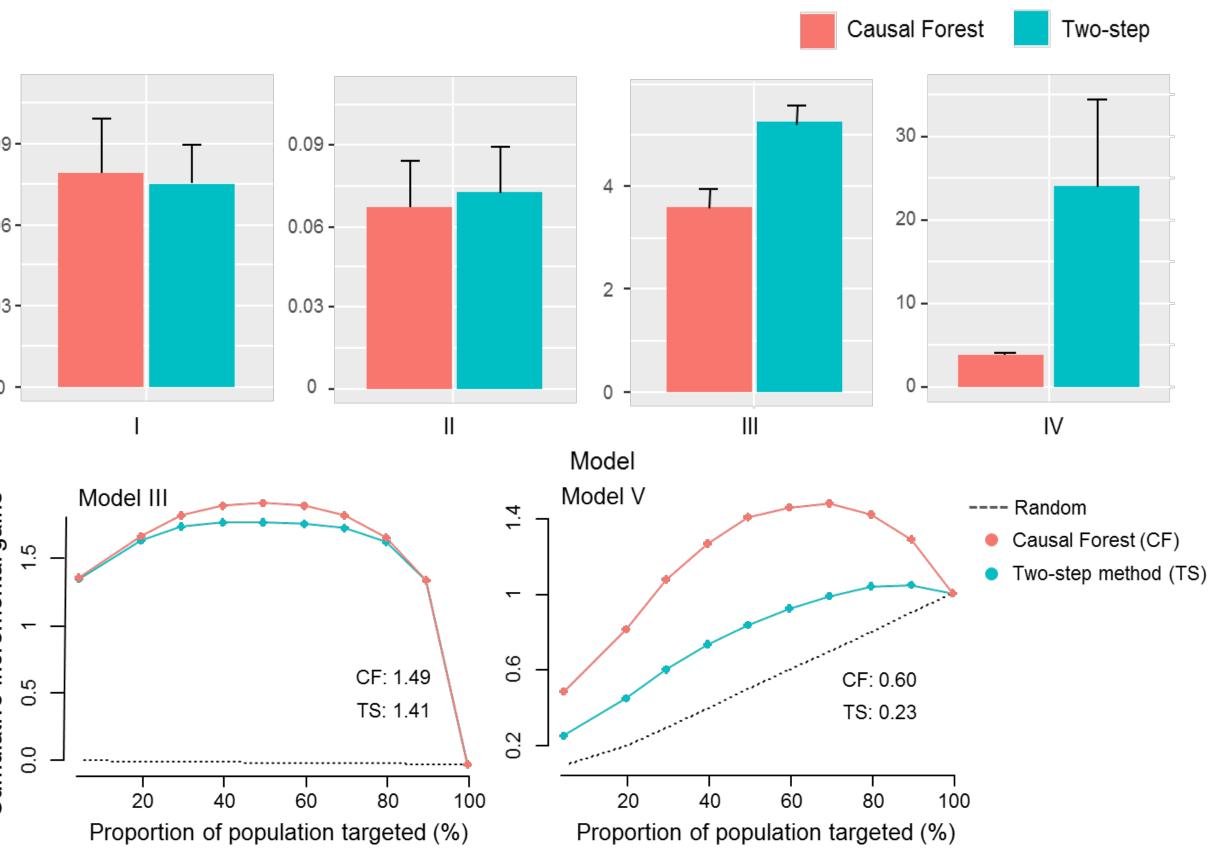
RESULTS

Exemplary Case: treatment effect of a dataset with nonlinear relationship between the two covariates x1, x2 and treatment effect.



Simulations based on Model I-IV

Causal forest and two-step method were applied on simulation data to perform HTE analysis. Prediction performance were evaluated in terms of root mean square error (RMSE) and incremental gains curve.



Causal forest can outperform the linear regression-based two-step method for HTE analysis with nonlinearity and high-dimensionality present in the data.

REFERENCES

1) Wager, S. and S. Athey, *Estimation and inference of heterogeneous* treatment effects using random forests. J Am Stat Assoc, 2018. 113(523).

DISCLAIMER

The opinions expressed in this poster are those of the authors and should not be interpreted as the position of the U.S. Food and Drug Administration.

