MATH RESEARCH AT UNC CHARLOTTE 2023

Project: Statistical methods for personalized medicine

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Project description. In recent years, personalized medicine, or precision medicine, has received tremendous attention in clinical practice and medical research, as patients often exhibit heterogeneous responses to treatments. In 2015, the White House launched the Precision Medicine Initiative, a research effort to revolutionize how to improve health and treat disease, further making precision medicine a national priority. Unlike the traditional "one size fits all" medical practice designed for the "average patient," personalized medicine seeks to recommend the right treatment to the right patient at the right time by considering individual differences in genes, environments, and lifestyles. Individualized treatment rules (ITRs) formalize personalized treatment decisions as a function mapping from patient information to a recommended treatment. The optimal ITR is the one that maximizes the mean of a pre-specified clinical outcome when applied to a patient population of interest. Therefore, one important task in personalized medicine is to estimate and discover optimal individualized treatment rules.

Several statistical methods have been proposed to estimate the optimal ITRs using data from randomized trials and observational studies [1, 2, 3]. The main objective of this project is to investigate how to estimate the optimal ITRs under the regression framework. This includes using ordinary linear regression and penalized regression such as lasso. In addition, data from a clinical trial treating chronic depression will be analyzed. If time permits, another task is to investigate other machine learning methods, in particular, how the problem of estimating an optimal ITR is equivalent to a classification problem.

Prerequisite and student's role. We welcome students with a background in probability and statistics to work on this project. In particular, the student must be familiar with the basic theory and methods of linear regression. In the first 2-3 weeks, the student will learn the basic knowledge of penalized regression models and the statistical framework of personalized medicine. Then the student will study and implement algorithms that estimate the optimal ITRs, conduct numerical studies, and analyze the real data.

References

[1] Q. Min, and S. A. Murphy. Performance guarantees for individualized treatment rules. *Annals of statistics*, **39**(2), 1180–1210, (2011).

[2] Y. Zhao, D. Zeng, A. J. Rush and M. R. Kosorok. Estimating individualized treatment rules using outcome weighted learning. *Journal of the American Statistical Association*, **107**(499), 1106–1118.

[3] Y. Pan, Y. Zhao. Improved doubly robust estimation in learning optimal individualized treatment rules. *Journal of the American Statistical Association*, **116**(533), 283–294.