Dynamic Generalized Odds-Ratio (dGOR): A novel approach to assess Dynamic Treatment Regimes (DTR) with An Ordinal Outcome

Palash Ghosh*

Centre for Quantitative Medicine
Duke-NUS Medical School
National University of Singapore, Singapore

Abstract

Sequential multiple assignment randomized trials (SMART) are used to construct data-driven optimal treatment strategies for patients based on their treatment and covariate histories in different branches of medical and behavioral sciences where a sequence of treatments are given to the patients; such sequential treatment strategies are often called dynamic treatment regimes (DTR). In the existing literature, the majority of the analysis methodologies for SMART studies assume a continuous primary outcome. However, ordinal outcomes are also quite common in clinical practice; for example, the quality of life is often measured in an ordinal scale (e.g., poor, moderate, good). In this work, first, we develop the notion of dynamic generalized odds-ratio ($dGOR$) to compare two dynamic treatment regimes embedded in a 2-stage SMART with an ordinal outcome. We propose a likelihood-based approach to estimate $dGOR$ from SMART data. Next, we discuss some combinatorial properties of $dGOR$ and derive the asymptotic properties of its estimate. We discuss some alternative ways to estimate $dGOR$ using concordant-discordant pairs and multi-sample U-statistic. Then, we extend the proposed methodology to a K-stage SMART. Furthermore, we propose a basic policy search algorithm that uses $dGOR$ to find an optimal DTR within a finite class. A simulation study shows the performance of the estimated $dGOR$ in terms of the estimated power corresponding to the derived sample size. We analyze data from Sequenced Treatment Alternatives to Relieve Depression (STAR*D), a multi-stage randomized clinical trial for treating major depression, to illustrate the proposed methodology. A freely available online tool using R statistical software is provided to make the proposed methodology accessible to other researchers and practitioners.

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*palash.ghosh@duke-nus.edu.sg