

Bayesian Borrowing Techniques for Rare Disease Clinical Research

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Course Description: Randomized clinical trials (RCTs) are the gold standard for estimating the effect of a treatment on an outcome. However, RCTs are also resource-intensive and require large samples to estimate moderate or small effect sizes. The resource-intensive nature of RCTs poses particular challenges in the setting of rare diseases where limitations on the number of potential trial participants limit the overall sample size of RCTs. Given these limitations, the design of RCTs in the context of rare diseases places a premium on efficiency and leveraging all available information to evaluate the effect of a treatment on an outcome. One approach to improve the efficiency of RCTs is to leverage external information, in the form of supplemental trial data or real-world data sources (EHR, etc.), through dynamic borrowing. Recent advances in Bayesian methods for dynamic borrowing provide a powerful set of statistical tools to improve the efficiency of RCTs by leveraging data external to the trial. This short course will provide an introduction to Bayesian methods for dynamic borrowing in the setting of rare disease clinical research. Specific topics to be covered include the motivation for leveraging external data in RCTs, an overview of Bayesian methods for dynamic borrowing, including recent advancements in the use of real-world data to augment RCTs, computational tools for implementing these methods, and a general discussion of the strengths and weaknesses of implementing these methods in practice. Throughout the course, methods will be illustrated via case studies from rare disease clinical research.