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Title:

Going multivariate in clinical trial studies: Increasing efficiency using Bayesian adaptive methods for information sharing

Abstract:

Randomized controlled clinical trials (RCTs) are the gold standard to investigate the effectiveness of treatments for (mental) diseases. However, demonstrating the superiority of a treatment is often challenging, in part due to difficulties to enroll a sufficient number of patients. Small samples might lead to premature stopping of the trial, inconclusive results, and potentially the withholding of better treatments from patients in need.

Despite the apparent lack of information in small samples, we often have other sources of information available that could improve decision-making. Especially Bayesian adaptive trial designs lend themselves perfectly to take advantage of combining information from multiple complementary outcomes. Bayesian methods potentially allow for modeling complex dependency structures of multiple outcomes, learning the treatment effects using Bayesian updating, performing optional stopping when evidence is conclusive, and tailoring treatments to patients during the trial; all without compromising error rates.

However, (Bayesian) statistical methods that take advantage of information sharing between multiple outcomes in clinical studies are largely underdeveloped. In this presentation Bayesian (adaptive) methods are proposed for this purpose. Our study demonstrates how the Bayesian adaptive approach benefits from combining multiple outcomes allowing trials to stop earlier, possibly rendering a small sample sufficiently informative.