Utilization of Robust Estimates of Treatment Effect via Semi-Parametric Models in MRCT

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Abstract: Multi-Regional Clinical Trial (MRCT) plays an increasingly important role in global drug development. It serves as an efficient way to accelerate drug development, allowing global simultaneous development and earlier access to new drugs, benefiting patients worldwide. MRCTs provide an opportunity for health authorities to examine robustness and the applicability of a treatment across diverse populations while assessing country specific benefit risk profile. I will highlight some key statistical issues recognized in ICH E17, e.g., increased heterogeneity in trials involving different regions. In addition, despite randomization, there may be a differential treatment effect among different regions, potentially due to confounding region specific factors. Thus, accurate and robust estimates of variation would be especially important to MRCT. Most current methods for the assessment of the consistency or similarity of the treatment effect between different ethnic groups are based on some subjectively specified model. In this talk I will summarize recent advances on robust estimates of global and regional treatment effects in MRCT though semiparametric modeling, and show the asymptotic and finite sample properties of the estimate and how they are applied to real clinical trials. (This work is in collaboration with Ao Yuan, Chaojie Yang, Shuxin Wang, and Shuqi Wang.)