ID de Contribution: **15** Type: **Non spécifié**

Integration of External Data in the Design and Analysis of Clinical Trials for Rare Diseases

jeudi 16 novembre 2023 12:15 (25 minutes)

Background: In the rare neurological disease field, it is challenging to demonstrate treatment effect in the clinical trials for new treatments. Some neurological diseases have very few patients available for recruitment. As a result, a sufficiently powered randomized clinical trial (RCT) is not feasible. The importance of incorporating historical data into the planning and analyses of clinical trials is on the rise, particularly for extending the control group of a new trial. However, the use of external data also introduces the risk of potential bias,as the historical control population may be rather different from the RCT.
Method : There are several approaches to eliminate or reduce the bias due to population difference. The methods used during the internship are either frequentist (propensity score matching (PSM) and inverse probability of treatment weighting (IPTW)), or a combination of frequentist and Bayesian methods (PSM or IPTW combined with power prior or commensurate prior).
Purpose: The aim is to compare the operational characteristics of various methods allowing to borrow information from historical data, in the context of a new trial with a 2:1 randomization ratio. The analyses are conducted using simulated data from a new clinical trial combined with an external cohort acting as historical data. In order to take into account discrepancies between historical and current data, different scenarios of natural change in the progression of the disease (called drift) and imbalance in patient group characteristics are implemented.
Results: Methods of borrowing historical data can increase power without creating too much bias or inflation of the type I error. Whether it is really beneficial depends on the hypotheses and the settings.

Auteur principal: Mlle JOUNOT, Emilie (Servier)

Co-auteur: Mme TRUFFAUT-CHALET, Lucie (Servier)

Orateur: Mlle JOUNOT, Emilie (Servier)

Classification de Session: Statistique bayésienne