Thursday 25 July 2024

09:00-12:30 Mini Symposium 1 (Room 1)

Beyond conventional RCTs: Exploring design options and modeling in drug development

Organizers: Marcia Rueckbeil, Els Goetghebeur, Mouna Akacha in collaboration with the ISCB Sub-Committee "Statistics in Regulatory Affairs" (SiRA)

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Applications of designs with external controls / hybrid designs in drug development

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In drug development, randomized controlled trials (RCTs) are the gold standard for generating evidence of a treatment effect. Yet, in specific situations there may be reasons for deviating from this gold standard. Alternative design options have been developed that come with more or less operational ease and statistical rigor. Trial designs without concurrent control and/or with a purely external control are on the extreme of the spectrum. They are prone to biases and apply mainly to niche situations, such as when the untreated counterfactual is severe and certain (e.g., rapid death). Hybrid designs, however, offer an alternative, more tempered option that can be tuned to a wider range of situations. In these designs, a concurrent control is augmented with external information in a controlled way. In this talk, we explore opportunities and challenges on this spectrum of trial design options. We share our experiences with implementing hybrid designs at scale in a mid-size pharmaceutical company, highlighting the value of generating experience and accumulating efficiency gains. We explore application areas for such designs from exploratory to pivotal development phases.