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)

Co-chairs: Marcia Rückbeil and Tim Friede

Voilà: The European collaborative project INVENTS

Sarah Zohar (INSERM, Paris, France)

The evaluation of new medicines for rare diseases (RD) including paediatric RDs is challenging for several reasons, among which are the small patient sample sizes, heterogeneity of patients and diseases and heterogeneity in disease knowledge. Due to these difficulties, access to effective treatments and the number of treatment options are often limited in RDs.
INVENTS is a European collaborative under Horizon Health Cluster aiming at providing clinical trial trialists, researchers and regulators with a global framework encompassing methods, workflows and evidence assessment tools to be implemented in RD drug development. The project kicked-off in January 2024 and it ambition is to significantly improve the evaluation of evidence and regulatory decision-making through the development and validation of: refined longitudinal model-based diseases trajectories and treatment effect, improved extrapolation models, in silico trials (e.g., virtual patient cohorts), optimised model-based clinical trial designs and evidence synthesis methods. These methods and models will be evaluated through simulation studies and challenged on extensive data from a range of use cases provided by our industrial partners Roche and Novartis and Real World data from the French National RD registry, they will also contribute to the scientific aspect of this project. The INVENTS framework will improve consistency and efficiency of the drug evaluation process for RD by augmenting clinical evidence without compromising its scientific integrity and providing regulators assessment credibility criteria.

At the end of this 5 years project, the RD trialist community will be able to exploit novel and improved clinical trial designs, in silico trials and RWD analysis approaches supporting drug development in RD. The European Medicine Agency and European national regulators (including Health Technology Assessment bodies) will be supplied with a general framework allowing better informed decision making. Most importantly, RD patients will benefit from an increased and faster access to efficacious and safe treatments.

INVENTS is funded by the European Union's Horizon Europe Framework programme under grant agreement 101136365. INVENTS is also funded by the Swiss State Secretariat for Education Research and Innovation (SERI).