

Summary of the Feb 15 ISCTM Estimands Working Group Meeting

As planned, the Estimands and Missing Data Working Group (WG) Session held on Feb 15, 2023 during the 19th ISCTM Annual Scientific Meeting hosted informal conversations on estimands and their implementation in clinical trials. This session started with a brief overview of the work done by this WG in the past year, presented by Elena Polverejan (WG chair). A manuscript has been developed by this WG; once published (now in the journal review process), it will be shared with the ISCTM Community.

The attendees tackled various topics, including:

- The need to address, within a trial, different questions of interest with their corresponding estimands.
- The importance of continuing data collection after intercurrent events such as treatment discontinuation or treatment switching; this would allow answering various questions of interest, even though they might not be primary or secondary.
- Assumptions for hypothetical scenarios could sometimes be difficult to be proposed and justified, such as how much would patients worsen if rescue medication would not be available; sensitivity analyses are important as they stress-test the assumptions of the main analysis, as well as supplementary analyses as they can capture the treatment effect in different ways.
- Disease specific regulatory guidance documents started to include recommendations on estimands, such as the FDA guidance for Chronic Rhinosinusitis with Nasal Polyps or the EMA Guideline on the clinical investigation of medicines for the treatment of Alzheimer's disease.
- Statisticians are invited to write questions of interest concisely and in a way that can be easily understood by other functions; multiple examples are included in the WG manuscript.

In the past year, the WG main's goal was the finalization and publication of the manuscript. As this is almost done, next steps and potential directions for this WG are being discussed. In the near future, new members will be able to join this WG and periodic meetings will be organized.