



**BBS Workshop:
Estimands in Early Development (ED)
across Therapeutic Areas**

Date: Monday 16th October 2023,
14:00 – 17:30 CET

Venue: VIRTUAL event, free of charge
Dial-in details will be communicated to registered virtual participants.

The topic of estimands has been largely discussed (spurred on by the ICH E9 Addendum) with a focus on late development and examples already exist of late-stage studies adopting the framework, well within regulatory acceptance. We wish to extend the conversation to Early Development across therapeutic areas.

We will be focusing on the questions:

- a. When/how to raise awareness on the topic of estimands also in early-stage development (will it depend on disease area, patient population, endpoint)?
- b. What value lies in introducing the language of estimands in protocols and analysis plans in ED?
- c. Can estimands aid in the goal of dose optimization?

The event will feature contributions from statisticians in industry and regulators. The Organizers are Fredrik Öhrn, Andrew Wright, Stefan Englert and Lilla Di Scala.

Slides and recordings of the talks will be made available after the event on the [BBS webpage](#), both pending speaker approval.

Please follow this link for the registration: [BBS Workshop: Estimands in Early Development \(ED\) across Therapeutic Areas \(google.com\)](#). In case of issues with the registration tool, please send an email to laurence.guillier@roche.com.

**Basel Biometrics Section
Basel, 2023**

Agenda

- 14:00 – 14:15 Lilla Di Scala (Janssen, BBS)
Welcome, scene setting and “Let’s extend the conversation on estimands to Early Development”
- 14:15 – 14:45 Stefan Englert (Janssen): Why jeopardize clarity, consistency, and coherency in early phase?
A plea for introducing estimand focused discussions to early development acknowledging similarities and differences as compared to late development.
- 14:45 – 15:00 Case Study 1: (Novartis, Karin Meiser, “Using estimands in PoC studies for infectious diseases: what did we consider?”)
- 15:15 – 15:30 Case Study 2: (AZ, David Wright, “Examples of opportunities to use the estimand framework in early Phase studies”)
- 15:30 – 15:45 Case Study 3: (Roche, Francois Mercier, “Dealing with treatment discontinuation in dose escalation Phase 1 oncology clinical trials”)
- 15:45 – 16:00 Break
- 16:00 – 16:20 Rob Hemmings, Consilium Scientific (former EMA) “Estimands in Early Development – an external perspective”
- 16:20 – 16:40 Thomas Gwise (former FDA), “Optimizing Estimands with Optimus”
- 16:40 – 17:30 Panel Discussion (Andrew Wright, Fredrik Öhrn and all speakers)
- 17:30 Next BBS webinars and closure

We look forward to your participation!