Welcome to the 6th EFSPi Regulatory Statistics Workshop 2021

Webinar 3: Real-world data and short topics session

We have put you on mute.
We are waiting for other attendees to join.
We will start soon.
6th EFSPI Regulatory Statistics Workshop
13th – 15th September 2021

• Three webinar sessions devoted to:

  Monday  1. Decentralized trials: What is the impact on evidence generation?

  Yesterday  2. Complex innovative designs: Where is their place in drug development?

  Today  3. Real-world data and short topics session

• Organization: EFSPI and organizing committees
  THANKS!

• Host: Roche (in particular Kaspar Rufibach)  THANKS!
What is EFSPI

• EFSPI = European Federation of Statisticians in the Pharmaceutical Industry
• Founded in 1992
• A federation of National European Groups
• Now have 10 national groups
• No individual members
• EFSPI is an “umbrella”, non-profit making organisation
• Our national organisations collectively represent 2200 members
• Each organisation has 2 members on the EFSPI Council

• Website: www.efspi.org
6th Regulatory Statistical Workshop!

- 2nd time virtual workshop

- 2022 live meeting???
High number of participants

from 200+ live workshop (2019), ≈ 700 people in webinar (2020)
More than 1200 participants now!!
Worldwide representation

1262 registrations from 42 countries

- Switzerland: 291 (23%)
- China: 252 (20%)
- Germany: 143 (11%)
- United States of America: 97 (8%)
- India: 59 (5%)
- Denmark: 87 (7%)
- Netherlands: 73 (6%)
- Canada: 34 (3%)
- Sweden: 34 (3%)
- France: 31 (2%)
- Ireland: 31 (2%)
- Finland: 22 (2%)
- Japan: 22 (2%)
- Italy: 19 (2%)
- Greece: 14 (1%)
- Spain: 11 (1%)
- Other: 8 (1%)

Other countries have registrations ranging from 1 to 8%.
Big thank you to the organizers!

Local organizing committee

- Egbert Biesheuvel (Danone)
- Hans Ulrich Burger (Roche)
- Christoph Gerlinger (Bayer)
- Kaspar Rufibach (Roche)
- Emmanuel Zuber (Novartis)

Scientific committee

- Elina Askanius (Finland)
- Andreas Brandt (BfArM)
- Maria Grünwald (Swedish Medical Products Agency)
- Randi Grøn (Novo Nordisk)
- Cecilia Hedlund (Swedish Medical Products Agency)
- Lorenzo Hess (Swissmedic)
- Benjamin Hofner (Paul-Ehrlich Institute)
- Armin Koch (Medizinische Hochschule Hannover)
- Eftychia-Eirini Psarelli (EMA)
- Khadija Rantell (MHRA)
- Kit Roes (Radboud UMC)
- Aldana Rosso (Danish Medicines Agency)
- Anja Schiel (Norwegian Medicine Agency)
- Steven Teerenstra (Radboud UMC)
Upcoming events with EFSPI involvement

28.09.2021  DIA webinar: Estimands How and Why - A Real Life Case Study in Oncology (co-organized by EFSPI / PSI WG estimands in oncology) link

21.10.2021  Joint PSI, EFSPI and ASA BIOP Webinar: Complex Innovative Designs link
Housekeeping for today

• If time allows clarification questions between the presentations will be discussed.

• Panelists please stay on mute and turn off video when not speaking.

• Participants please ask questions using the chat or in Q&A. Questions will be picked up for the panel discussion or responded to in writing after the event.

• If applicable please indicate to whom you want to ask question.

• Slides and recording will be made available on EFSPSI webpage pending approval of all speakers.
Real-world data - using their potential

- **Chantal Quinten (EMA)** Opportunities and Challenges of RWE to Support Regulatory Decision-Making
- **Khadija Rantell (MHRA)** Meeting the expectation of patients with rare diseases: examples of high-quality medicines and timely approval
- **Tim Williams (MHRA) and Dipak Kotecha (University of Birmingham)** Pragmatic trials in the real world: DaRe2THINK – a novel approach to healthcare-embedded clinical trials
- **Qing Wang (Roche)** Use of RWD to contextualize post-hoc analysis to support regulatory submission: an example from the ORATORIO Trial and the Long-Term MSBase Registry
- **Christoph Gerlinger (Bayer)** The use of external controls: To what extent can it currently be recommended?

break

- **Marianne Cunnington (GSK)** Evolving strategies in generating medication safety data in pregnancy – an industry perspective
- **Yuki Ando (PMDA)** RWD - PMDA’s view on it
- **Panel discussion with all speakers + Anny Stari (PSI / EFSPi RWD SIG chair)**