6th EFSPI Regulatory Statistics Workshop
13th – 15th September 2021

Dates and times (CET): Monday, 13rd September 2021, 14-17
Tuesday, 14th September 2021, 14-17
Wednesday, 15th September 2021, 9-13
Webinar: dial-in details will be communicated to registered participants

EFSPI is pleased to announce the 6th regulatory statistics workshop that will take place on 13th-15th September 2021. Given the ambiguity around the COVID-19 situation the scientific committee has decided to make this again a virtual workshop this year, consisting of three webinars. To be globally inclusive webinar times are varied on purpose over the three days.

The workshop will discuss opportunities and challenges of statistical topics between regulators, academics, and industry. We plan for three webinar sessions devoted to

1. Decentralized trials: What is the impact on evidence generation?
2. Complex innovative designs: Where is their place in drug development?
3. Real-world data - using their potential.

To provide some variety we also have shorter sessions on each day:

1. What is on regulator's mind?
2. Same trial but two different estimands for two stakeholders – how is the interpretation different? What is the impact?
3. Short topic session: three topics presented on 2 slides seeking input from regulatory panel.

All sessions feature a panel discussion where participants will be able to ask questions through the chat. Alternatively, you can also share questions upfront by sending an email to kaspar.rufibach@roche.com.

Given that face-to-face interactions were always a key feature of this workshop it is the scientific committee's clear ambition for 2022 to again gather face-to-face, it is by no means our intent to organize this workshop virtually in the future.

Members of the Scientific Committee are: Egbert Biesheuvel, Hans Ulrich Burger, Christoph Gerlinger, Kaspar Rufibach, Emmanuel Zuber, Elina Asikanius, Andreas Brandt, Randi Gron, Cecilia Hedlund, Lorenzo Hess, Armin Koch, Eftychia-Eirini Psarelli, Khadija Rantell, Kit Roes, Anja Schiel, Steven Teerenstra.

Registration: Please register here: https://forms.gle/q3UqoFkMC9NSB2sw5. The registration fee for the entire event (also if you only plan to attend one webinar) is 50 Euro. As part of the registration process we would like to ask you to make a payment of €50 to EFSPI as contribution to the 6th EFSPI Regulatory Statistics Workshop. Please use the reference "Regulatory 21". Due to this informal registration process we can keep the price low. Therefore we cannot accept credit card transfer and we cannot provide you with a formal invoice after the payment. In case you have any question on your payment, please contact Egbert Biesheuvel (Egbert.biesheuvel@danone.com)

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Bank details: European Fed Stat Pharm Ind, **Bank**: HSBC, **IBAN**: GB53HBUK40127677385084, **BIC**: HBUKGB4B, **Reference**: Regulatory 21.

Participation of scientific committee members, speakers & panel discussants, colleagues at regulatory agencies, and students is for free. In addition, Roche and Novartis colleagues are included in a collective registration and also do not need to pay by themselves (but please still register using the above link!). If your company is interested in getting a collective registration (>30 participants) please reach out to kaspar.rufibach@roche.com.

For those who have registered and paid the registration fee a link to a Zoom TC will be shared a few days prior to the event.

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

13th September, 14-17 CET.

Time	Duration	Presentation
1400-1410	10	**Stefan Driessen (EFSPI president)** Opening remarks
1410-1425	15	**EMA regulatory colleague (tba)** Introductory session: What is on European regulator's mind? Where are we with the workplan 2021? What guidelines are planned to be updated? What is industry constantly missing in submissions?
1425-1450	25	**Ralf Herold (EMA)** tba
1450-1515	25	**Mark Levenson (Center for Drug Evaluation and Research, FDA)** FDA Guidance on Decentralized Clinical Trials
1515-1530	15	**James Bell (Elderbrook Solutions)** Concept for evaluation of remote endpoint assessment by integration of an orthogonal crossover equivalence substudy within longitudinal parallel trial designs
1530-1540	10	**Break**
1540-1555	15	**Magalie Hilton (Roche)** alpha-T: a pre-pandemic decentralized trial in oncology
1555-1610	15	**Mira Zuidgeest (UMC Utrecht)** IMI Trials@Home – a head-to-head comparison between remote decentralized, hybrid and conventional trial approaches regarding quality and safety
1610-1630	20	**Anja Schiel (Norwegian Medicines Agency and EMA BSWP & SAWP)** DCT, the new kid on the block. Here to stay?
1630-1655	25	**Panel discussion with all speakers** **Moderator: Khadija Rantell (MHRA) + Emmanuel Zuber (Novartis)**
1655-1700	5	**Emmanuel Zuber (Novartis, local organizing and scientific committee)** Closure

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

14th September, 14-17 CET.

Time	Duration	Presentation
1400-1410	10	**Egbert Biesheuvel (Danone, local organizing and scientific committee)** Welcome and scene setting
1410-1430	20	**Michael O'Kelly (IQVIA)** Two estimands: double trouble or just the same old same old?
1430-1455	25	**Anja Schiel (Norwegian Medicines Agency and EMA BSWP & SAWP) & Armin Koch (Medizinische Hochschule Hannover & EMA BSWP & SAWP)** Is there something like "too much innovation"?
1455-1515	20	**Kirsty Wydenbach (MHRA)** MHRA experience with CIDs and recommendations derived from it
1515-1530	15	**Jiawen Zhu (Roche)** Label-enabling dynamic borrowing for OS in DLBCL - FDA CID pilot example
1530-1540	10	**Break**
1540-1555	15	**Don Berry (Berry Consultants) and Alexandra Vaury (Novartis)** Regulatory feedback and pharmaceutical partner's perspective on Precision Promise phase 3 platform study design.
1555-1610	15	**Philip Hougaard (Lundbeck)** Designing the EPAD (European Prevention of Alzheimer's Dementia) Platform trial: Key issues
1610-1630	20	**Kit Roes (Radboud university medical center and EMA BSWP)** Complex innovative versus comprehensive assessment for benefit risk
1630-1655	25	**Panel discussion with all speakers + Kit Roes: Trials should be as simple as possible and as complex as needed** **Moderator: Eftychia Psarelli (EMA) + Kaspar Rufibach (Roche)**
1655-1700	5	**Kaspar Rufibach (Roche, local organizing and scientific committee)** Closure

Webinar 3: Real-world data - using their potential.

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15th September, 9-13 CET.

Time	Duration	Presentation
0900-0905	5	**Christoph Gerlinger (Bayer, local organizing and scientific committee)ber** Welcome and scene setting
0905-0925	20	**Chantal Quinten (EMA)** Opportunities and Challenges of RWE to Support Regulatory Decision-Making
0925-0945	20	**Khadija Rantell (MHRA)** Meeting the expectation of patients with rare diseases: examples of high quality medicines and timely approval
0945-1005	20	**Tim Williams (MHRA) and Dipak Kotecha (University of Birmingham)** Pragmatic trials in the real world: DaRe2THINK – a novel approach to healthcare-embedded clinical trials
1005-1020	15	**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
1020-1035	15	**Christoph Gerlinger (Bayer)** The use of external controls: To what extent can it currently be recommended?
1035-1045	10	**Break**
1045-1100	15	**Marianne Cunnington (GSK)** Evolving strategies in generating medication safety data in pregnancy – an industry perspective
1100-1125	25	**Yuki Ando (PMDA)** RWD - PMDA's view on it
1125-1150	25	**Panel discussion with all speakers + Anny Stari (PSI / EFSPI RWD SIG chair) + Corinne de Vries (EMA)** **Moderator: Elina Asikanius (FIMEA) + Christoph Gerlinger (Bayer)**
1150-1200	10	**Break**
1200-1250	50	**Uli Burger & Armin Koch + panel with all regulators** Short topic session: three short topics, presentation on 2 slides, get input from panel please send proposals until 16th August to kaspar.rufibach@roche.com
1250-1300	10	**Uli Burger (Roche, local organizing and scientific committee)** Closure