Status: September 04, 2025

10th EFSPI Regulatory Statistics Workshop

10 – 12 September 2025

Basel Switzerland

Day 0: 09th September 2025, 19:00-22:00

19:00 – 22:00	**Pre-Conference Regulatory Dinner** *All regulators and HTA assessors* are cordially invited to this (self-paid) event, organized by Benjamin Hofner and Heidi Mestl. For more information and registration please visit **link**.

Day 1: 10th September 2025, 08:30-17:00 (+2h get-together)

Time	Presentation
08:30 – 08:45 (15 min)	**Opening Remarks** Egbert Biesheuvel (EFSPI President, Viatris, NL)
08:45 – 10:15 (90 min)	**"Overture": Strategic Priorities in Pharmaceutical Statistics** Speakers will discuss 1. Regulatory priorities in Europe 2. Industry view on regulatory priorities in Europe *Chairs: Bergrún Magnusdottir (IMA, IS), Pierre Mancini (Sanofi, FR)* Talk 1: Kit Roes (Chair of MWP EMA, Radboud UMC, NL): Navigating priorities from regulatory perspective Talk 2: Mouna Akacha (Co-chair EFSPI statistical methodology leaders, Novartis, CH): Strategic priorities in pharmaceutical statistics – A quantitative drug developer's perspective Q&A with the audience, the speakers and regulatory agency representatives: Andrea Manfrin (MHRA, UK) and Rafael Sauter (Swissmedic, CH)
10:15 – 10:45 (30 min)	Coffee break

Day 1: 10th September 2025, 08:30-17:00 (+2h get-together) *<continued>*

Time	Presentation
10:45 – 12:30 (105 min)	**ICH E20 Guideline on "Adaptive Designs for Clinical Trials" – A Critical Discussion from Different Perspectives** Homework: Please read the *draft version* of the ICH E20 guideline on Adaptive Designs for Clinical Trials currently on public consultation to get most out of the session. Discussants will pick and discuss 1 or 2 topics that they found the most thought-provoking. *Chairs: Fredrik Öhrn (J&J, SE), Kit Roes (Chair of MWP EMA, Radboud UMC, NL)* Talk 1: Frank Pétavy (EMA, NL), Khadija Rantell (MHRA, UK): Moving towards harmonisation for (confirmatory) trials with an adaptive design Talks 2 – 5: ICH E20 guideline on adaptive designs for clinical trials: my reflections as a statistician working in … Academia – Christopher Jennison (University of Bath, UK) Industry – Silke Jörgens (J&J/University of Cologne, DE) Regulatory assessment – Maria Grünewald (MWP member, MPA, SE) HTA assessment – Seamus Kent (ESHPM, NL) Moderated discussion with the audience, the speakers and representatives of the **ICH E20 WG**: Hans Ulrich Burger (Medical University of Graz, DE), Jürgen Hummel (Novo Nordisk, UK), Thomas Hiemstra (Novartis, CH)
12:30 – 14:00 (90 min)	Lunch break and poster session: ESIGs and EFSPI Working Groups
14:00 – 15:30 (90 min)	**From Trials to Target Populations: Extending Evidence for Decision-Making** Speakers will discuss issues of transportability and generalisability *Chairs: Anja Schiel (SAWP & MWP member, NoMA, NO), Seamus Kent (ESHPM, NL)* Intro Seamus Kent (ESHPM, NL) Talk 1: Antonio Remiro-Azócar (Novo Nordisk, ES): Evidence synthesis: estimands, transportability and external validity Talk 2: Miguel Hernán (Harvard T.H. Chan School of Public Health, US): Emulation of target trials using observational data: because randomized trials cannot possibly answer all causal questions Q&A and moderated discussion with the audience and the speakers
15:30 – 16:00 (30 min)	Coffee break
16:00 – 17:00 (60 min)	**10th Anniversary of the EFSPI Regulatory Statistics Workshop**
17:00 – 19:00	**Get-together** including wine tasting organised by Emmanuel Zuber (Independent consultant, CH) and Hans Ulrich Burger (Medical University of Graz, DE)

Day 2: 11th September 2025, 09:00-17:30

Time	Presentation
09:00 – 11:00 (120 min)	**Short Topics**: **Present a problem on 2-3 slides and receive input from a panel of regulators** (≤ 24 minutes per topic) *Chairs: Elina Asikanius (SAWP & MWP member, fimea, FI), Kaspar Rufibach (Merck KgaA, CH)* Panelists: (*MWP member, #[alternate] SAWP member) Angelika Geroldinger (AGES, AT), Anja Schiel*# (NOMA, NO), Florian Lasch, Frank Pétavy (EMA, NL), Katharina Hees, Lukas Aguirre Dávila# (PEI, DE), Khadija Rantell (MHRA, UK), Kit Roes* (Radboud UMC, NL), Maria Grünewald* (MPA, SE), Tommi Nurminen (fimea, FI), Xiaofei Liu (BfArM, DE) Topic 1: Gaelle Klingelschmitt, Jianmei Wang, Audrey Boruvka, Jenny Devenport (Roche, CH): Surprises matter! Limiting broad disclosure of futility analysis criteria to preserve trial integrity Topic 2: Rob Hemmings (Consilium, UK): Accessing accumulating data in open-label studies Topic 3: Franco Mendolia, Katja Brandau (Bayer, DE): The future of per protocol set analyses in non-inferiority trials Topic 4: Thomas Hoffelder, Beate Presser (Boehringer Ingelheim, DE), Christian Schmid (Roche, DE) on behalf of the CMC-Network EU SIG: How can we strengthen statisticians' impact in CMC related ICH guidelines? - Special focus on ICH M13b concerning dissolution profiles Topic 5: Bohdana Ratitch, Alfredo Farjat (Bayer, CA/NL): Acceptability of prognostic covariate adjustment and Targeted Maximum Likelihood Estimation (TMLE) methods for the primary and key analyses of pivotal clinical trials in absence of prior clinical knowledge about strong predictive factors
11:00 – 11:30 (30 min)	Coffee break
11:30 – 12:30 (60 min)	**From Black Box to Pandora's Box: Navigating AI in Clinical Trials** *Chairs: Benjamin Hofner (PEI, DE), Jenny Devenport (Roche, CH)* Talk 1: Tim Friede (University of Göttingen, DE): AI in clinical trials: Is there a role for statistics? Talk 2: Gabriel Westman (MWP member, MPA, SE): AI in clinical trials – a regulatory perspective Talk 3: Chris Harbron (Roche, UK): Will industry need statisticians in an AI world? Moderated discussion with the audience, the speakers, Florian Klinglmüller (MWP member, AGES, AT) and Andrea Manfrin (MHRA, UK)
12:30 – 14:00 (90 min)	Lunch break

Day 2: 11th September 2025, 09:00-17:30 *<continued>*

Time	Presentation
14:00 – 15:30 (90 min)	**Characterizing the Effect of Treatment Using Hierarchical Composite Endpoints – Risks and Benefits of Win Statistics and Beyond** HCEs are proposed across therapeutic areas with the intent to characterize treatment effects by combining different outcomes using Generalized Pairwise Comparisons (e.g., win statistics), Desirability of Outcome Ranking, or Ordinal Longitudinal Models for State Occupancies. Speakers will discuss the importance of a clear clinical question, methodological strengths and limitations, and their role in regulatory decision making. *Chairs: Heidi Mestl (SAWP member, NoMA, NO), Patrick Schlömer (Bayer, DE)* Talk 1: Henrik F. Thomsen (Novo Nordisk, DK) and Mickaël De Backer (UCB, BE): Hierarchical composite endpoints: more nuance, more insight and … more confusion? Talk 2: Lukas Aguirre Dávila (alternate SAWP member, PEI, DE) Hierarchical composite endpoints – time to untie the not's? Moderated discussion and Q&A with the audience, the speakers, Andreas Brandt (BfArM, DE) and Anja Schiel (SAWP & MWP member, NoMA, NO)
15:30 – 16:00 (30 min)	Coffee break
16:00 – 17:30 (90 min)	**Keeping it Real or Losing Control? Adventures in Target Trial Emulation** Goal: to explore how Target Trial Emulation (TTE) can improve transparency and methodological rigor when designing, evaluating, and assessing clinical trials incorporating observational data. Stakeholders will gain insights into regulatory expectations, industry experiences, and how TTE enhances alignment and communication between regulators and pharmaceutical companies. This includes how TTE clarifies assumptions, addresses biases, and facilitates robust sensitivity analyses and evaluation criteria through structured links to randomized trials. *Chairs: Anouk Neven (EMA, LU), Pierre Mancini (Sanofi, FR)* Talk 1: Olaf Klungel (MWP member, University of Utrecht, NL): Bridging the Target Trial Emulation Framework and the Estimand Framework Talk 2: Rima Izem (Novartis, CH): Target Trial Emulation meets clinical trial design: two case studies Talk 3: Angelika Geroldinger (AGES, AT): From a regulatory perspective: what can we gain from TTE? Moderated discussion and Q&A with the audience and the speakers, Elina Asikanius (SAWP & MWP member, fimea, FI) and Simon Newsome (Novartis, CH)

Day 3: 12th September 2025, 08:30-12:10

Time	Presentation
08:30 – 10:00 (90 min)	**The Added Value of Bayesian Methods for Pivotal Clinical Trials – Just a Communication Issue?** *Chairs: Claudia Dallinger (Boehringer Ingelheim, DE), Elina Asikanius (SAWP & MWP member, fimea, FI)* Talk 1: Katharina Hees and Florian Krach (PEI, DE): Bayesian approaches in clinical trials: a discussion on regulatory expectations Talk 2: Nicky Best (GSK, UK): Communicating the value of Bayesian approaches in clinical trials: is it just a prior issue? Reflections of the panelists: Aysun Cetinyurek Yavuz (MEB, NL), Simon Wandel and Frank Bretz (Novartis, CH), Frank Pétavy (EMA, NL), and Tobias Mielke (J&J, DE) Moderated discussion and Q&A with the speakers, the audience and the panelists
10:00 – 10:30 (30 min)	Coffee break
10:30 – 12:00 (90 min)	**How to Deal with Assumptions in Drug Development? What Insights Can We Gain from ICH M15?** *Chairs: Claudia Dallinger (Boehringer Ingelheim, DE), Rafael Sauter (Swissmedic, CH)* Talk 1: Flora Musuamba Tshinanu (SAWP & MWP member, University of Namur, BE): From risk assessment to optimal regulatory decision: first having the question right Talk 2: Oliver Sailer and Valerie Nock (Boehringer Ingelheim, DE): Bridging disciplines with ICH M15: a case study on assumption testing for pharmacometric-enhanced Bayesian borrowing Talk 3: Tobias Mielke (J&J, DE): ICH-M15 to support credibility assessment for Bayesian modelling Moderated discussion and Q&A with the audience, the speakers and Norbert Benda (University of Göttingen, DE)
12:00 – 12:10	**Closure** Vivian Lanius (UCB BioSciences GmbH, Local Organizing and Scientific Committee, DE)
12:10	Lunch

Poster session of ESIGs and EFSPI Working Groups on Day 1 (10th September 2025, 12:30 – 14:00)

ESIG/EFSPI Working Group	Title	Author(s)/*Presenter(s)* *on behalf of the SIG / group
Causal Inference SIG	Causal Inference Special Interest Group	*Sanne Roels*, *Alex Ocampo*, Kelly Van Lancker
CMC Statistical Network EU SIG	Introduction to CMC statistical network Europe and areas for priority	*Beate Presser*, *Christian Schmid*, Jens Lamerz, Kevin Lief, Martin Motava
EFSPI Scientific & Training Academy	Scientific and Training Academy – Past and Present	Jonas Häggström
EFSPI/EFPIA EIWG with phuse sub-team	Estimands in safety analytics	*Armin Schüler*, *David Wright*, Amel Besseghir, *Khadija Rantell*, Andreas Sashegyi, Katarina Hedman, George Kordzakhia, Mike Colopy, Liangcai Zhang
EFSPI statistical method. leaders	Statistical methodology leaders in drug development, an EFSPI group	*Mouna Akacha, Kaspar Rufibach* *
HMA-EMA Big Data Steering Group	Clinical study data submission in Europe: An EMA-CHMP proof-of-concept pilot	*Frank Pétavy*, Eftychia Eirini Psarelli, Marie Annie Orre
Historical Data SIG	Historical Data: A PSI/EFSPI Special Interest Group	*Oliver Sailer*, Monika Jelizarow *
HTA SIG	"Adaptive Clinical Trials" – what are the challenges and opportunities from an HTA perspective?	Maximo Carreras, Chrissie Fletcher
HTA SIG	Characterizing the effect of treatment using hierarchical composite endpoints – risks and benefits of win statistics and beyond in the HTA context	*Fred Sorenson*, Anders Gorst-Rasmussen, Shahrul Mt-Isa
HTA SIG / RWE SIG	Target trial emulation and incorporation of observational data into clinical trials from an HTA perspective	*Katrin Kupas*, Min-Hua Jen, Orlando Dohring, *Rima Izem*
HTA SIG	From trials to target populations: extending and extrapolating evidence for HTA decision-making	Ash Bullement, Peter Pemberton-Ross, Grammati Sarri, Orlando Dohring
Launch & Lifecycle SIG	Updates from the Launch & Lifecycle SIG	*Lada Mitchell, Cornelia Dunger-Baldauf*, Jenny Devenport, Yulia Dyachkova
openstatsware / Software Engineering SIG	openstatsguide: checklist for good statistical software packages	*Audrey Yeo*, Daniel Sabanés Bové, Alessandro Gasparini, *Nils Penard*
PSI / EFSPI Biomarkers SIG	The Biomarkers ESIG	*Denis Engemann*, Laura Schlieker, Mathias Cardner
Real World Data SIG	Welcome to the Real World Data SIG!	Josephine Wolfram, *Rima Izem*, Elizabeth Merrall, Helen Broadhurst *
Regulatory SIG	EFSPI/PSI regulatory European Special Interest Group	*Christoph Gerlinger* *
Small populations SIG	The PSI/EFSPI small population Special Interest Group	Giles Partington, *Maeva Dupuis*, *Aysun Cetinyurek Yavuz*
Treatment Effect Heterogeneity SIG	Data-driven evaluation of treatment effect heterogeneity	*Bjoern Bornkamp*, Kostas Sechidis, David Svensson, Ashwini Venkatasubramaniam