8th EFSPI Regulatory Statistics Workshop
13th-14th September 2023
Basel Switzerland

13th September 2023, 0850-1700 (+2h wine tasting), Day 1

Time	Duration (mins)	Presentation
8.50-9.00	10	Opening remarks Justine Rochon (EFSPI President and SVP at Boehringer Ingelheim)
9.00-10.30	90	**Session 1: Keynote: Regulatory and HTA update** Chairs: Khadija Rantell (MHRA), Vivian Lanius (Bayer) Talk 1: Kristin Karlsson (Swedish Medical Products Agency, Uppsala University, EMA, Vice-chair MWP) - virtually *EMA Methodology Working Party update* - https://www.ema.europa.eu/en/documents/work-programme/consolidated-3-year-work-plan-methodology-working-party-mwp_en.pdf Talk 2: Eftychia-Eirini Psarelli (University of Liverpool, EMA) *EMA update on submission of individual patient data from clinical trials* Big data | European Medicines Agency (europa.eu) Talk 3: David McConnell (Statistician National Centre for Pharmacoeconomics, Ireland, Methodology Subgroup of HTA CG) *Update on joint HTA work under the EU HTA Regulation* Q&A
10.30-11.00	30	Coffee break
11.00-13.00	120	**Short topics** (20 mins per topic): Chairs: Elina Asikanius (fimea, EMA), Kaspar Rufibach (Roche) 1. Fredrik Öhrn (Janssen): Formal statistical requirements for a pivotal trial to support approval of a combination drug 2. Betty Molloy (Novartis): Use of RWE in drug labelling 3. Patrick Schloemer (Bayer): Hierarchical Composite Endpoints for Chronic Kidney Disease Trials 4. Simon Wandel (Novartis): What's the rule for the pool? 5. Sandro Gsteiger (Roche) and Anders Gorst-Rasmussen (Novo Nordisk, on behalf of the PSI/EFSPI HTA Special Interest Group): Surrogate endpoints - can EMA and EU HTA align on common standards? 6. Susan Robson (Roche): Data Quality issues in IITs
13.00-15.00	120	Lunch break and poster session: ESIGs and EFSPI Working Groups
15.00-17.00	120	**Session 2: Synthetic and other baseline covariates: The promise of smaller and faster clinical trials through prognostic digital twins** Chairs: Benjamin Hofner (Paul-Ehrlich Institut, EMA), Fredrik Öhrn (Janssen) Talk 1: Andreas Brandt (BfArM, EMA) *Covariate Adjustment: Traditional principles and challenges by new approaches* Talk 2: Courtney Schiffman (Genentech Roche) *Strategic considerations and value of covariate adjustment*

		Talk 3: FDA/CDER/OTS/OB/DBIV Daniel Rubin, Ph.D. - virtually *Adjusting for covariates in randomized clinical trials for drugs and biological products* Adjusting for Covariates in Randomized Clinical Trials for Drugs and Biological Products | FDA Open discussion with the audience
17.00-19.00	120	**Wine tasting** organised by Hans Ulrich Burger (Roche) and Emmanuel Zuber (Novartis)

14th September 2023, 0900-1710, Day 2

Time	**Duration (mins)**	**Presentation**
9.00-10.45	105	**Session 3: Complex innovative trials for regulatory decision-making** Chairs: Lukas Aguirre Dávila (Paul-Ehrlich Institut, EMA), Corine Baayen (Ferring Pharmaceuticals) Talk 1: Khadija Rantell (MHRA), Frank Bretz (Novartis), Hans Ulrich Burger (Roche) *Some guidance on when applying adaptive designs* Talk 2: Wolfgang Jacquet (Vrije Universiteit Brussel, EMA) and Benjamin Hofner (Paul-Ehrlich Institut, EMA) *Platform trials in a confirmatory regulatory setting – generating thoughts and directions* Talk 3: Dieter Haering (Novartis) *Experience with and learnings from regulatory interactions around innovative trial designs based on the NEOS study* Panel discussion with speakers
10.45-11.15	30	Coffee break
11.15-13.30	105	**Session 4: How to get most out of Scientific Advice** Chairs: Kit Roes (Radboud Universiteit, EMA), Giulia Zigon (GSK) Talk 1: Tommi Nurminen (fimea, EMA) and Antero Kallio (fimea, EMA) *How to efficiently leverage scientific advice - a joint statistical and clinical perspective* Talk 2: Gergő Merész (Co-Chair of the JSC Subgroup (HTA R)) *HTA Joint Scientific Consultation* Talk 3: David Wright (AstraZeneca) *Tips on a) what questions to ask regulators and HTAs, b) interpreting the answers given and c) what to do when different authorities give conflicting advice*

		Open discussion with the audience
13.30-15.00	90	Lunch break
15.00-17.00	120	**Session 5: Use of non-RCT studies in regulatory decision-making** Chairs: Eftychia-Eirini Psarelli (University of Liverpool, EMA), David Wright (AstraZeneca) Talk 1: Kit Roes (Radboud Universiteit, EMA) *Single Arm Trials: The EMA Reflection Paper* EMA draft reflection paper: https://www.ema.europa.eu/en/establishing-efficacy-based-single-arm-trials-submitted-pivotal-evidence-marketing-authorisation (April 2023) Talk 2: Pallavi Mishra-Kalyani and Mark Levenson (FDA) - virtually *Regulatory Issues with the Use of External Controls and the US FDA RWE Program* FDA draft guidance: https://www.fda.gov/regulatory-information/search-fda-guidance-documents/considerations-design-and-conduct-externally-controlled-trials-drug-and-biological-products (February 2023) Talk 3: Emmanuel Zuber (Novartis) *External control for approval and labeling: Two case studies trying to figure out when it is worth it* Talk 4: PD Dr Med Stefan Lange (IQWiG) *HTA view on the EMA draft reflection paper and the FDA guidance* Open discussion with the audience
17.00-17.10	10	**Closure** Helle Lynggaard (Novo Nordisk, Local Organizing and Scientific Committee)

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Confirmed posters:

ESIG/EFSPI Working Group	Title	Author/Presenter
Scientific Affairs/Training Academy	Scientific Affairs & Training Academy – Forward-looking in 2024 and beyond	Egbert Biesheuvel
Estimand Implementation Working Group	How the 'Estimand Implementation Working Group' brings together statisticians and clinicians to support the estimand journey	Khadija Rantell, Amel Besseghir, Chrissie Fletcher, Nanco Hefting, Armin Schueler, Pepa Polavieja, Helle Lynggaard, Judith Anzures Cabrera, Stefan Englert
Next Generation Group in BBS	Future of the Next Generation Statisticians and Quantitative Scientists	Lars Andersen, Joana Marques Barros, Muriel Buri, Youyou Hu, Antonella Mazzei, Kristina Weber, Lukas Andreas Widmer, Hans Ulrich Burger, Olympia Papachristofi
HTA ESIG	What's the HTA ESIG doing to prepare for EU HTA? 1. What's the HTA ESIG doing to prepare for EU HTA?	Emma Crawford, Anders Gorst-Rasmussen, Sandro Gsteiger, Fred Sorenson
HTA ESIG	EU HTA - how can statisticians help navigate the problem of multiplicity?	Emma Crawford, Anders Gorst-Rasmussen, Sandro Gsteiger, Fred Sorenson
Oncology-Estimands ESIG	Conditional and Unconditional treatment effects in randomized clinical trials: Estimands, Estimation, and Interpretation	Björn Bornkamp, Jiawei Wei, Sarwar Mozumder
Regulatory ESIG	Regulatory ESIG	Christoph Gerlinger
Biomarker ESIG	The Biomarker Europe Special Interest Group: where are we now?	Esha Mohamed, Guillaume Desachy and Nicole Krämer
Launch & Lifecycle ESIG	Launch and Lifecycle SIG	Jenny Devenport & Alexander Schacht
Historical Data ESIG	Beyond the classical type I error: Bayesian metrics for Bayesian designs using informative priors	Nicky Best and Simon Wandel