9th EFSPI Regulatory Statistics Workshop
11-13 September 2024
Basel Switzerland

10th September:

Regulatory Dinner: The regulatory members of the Scientific Committee are organizing a regulatory get-together in the margins of the workshop to foster the international exchange within this group. **All regulators and HTA assessors** are cordially invited to this (self-payed) event. The get-together will take place in a relaxed and informal atmosphere in a restaurant on **Sept 10th, 2024 at 19:00 CEST** on the evening before the workshop. Please book your travel accordingly. To register please fill out the following form. Details will follow via email to all who registered. To facilitate the planning please register no later than 28 August 2024. In case of questions please reach out to the organizers Heidi Mestl (Heidi.Mestl@legemiddelverket.no) or Benjamin Hofner (Benjamin.Hofner@pei.de).

11th September 2024, 0830-1700 (+2h wine tasting), Day 1

Time	**Duration (mins)**	**Presentation**
8:30-8:45	15	**Opening remarks** Egbert Biesheuvel (EFSPI President, Viatris, NL)
8:45-10:45	120	**Session 1: Fast to market vs. robustness of the data** *Chairs: Khadija Rantell (MHRA, UK) and Fredrik Öhrn (J&J, SE)* Talk 1: How the pressure to be first, faster, puts pressure on us all and what we can do about it? Speaker: Jenny Devenport (Roche, CH) Talk 2: Conditional marketing authorisation. Speaker: Eva Skovlund (NOMA, NO; CHMP member) Talk 3: CLL11 – a trial tailored to answer questions from many stakeholders efficiently. Speaker: Kaspar Rufibach (CH) Talk 4: Fast and furious to market across Pharma, is it good for HTA? Speakers: Karin Cerri and Lilla di Scala (J&J, CH) Panel discussion with the audience: Speakers and Bergrún Magnusdottir (IMA, IS) and Peter Ahnesorg (Roche, CH)
10.45-11.15	30	Coffee break
11:15-13:15	120	**Short topics** (20 mins per topic): **Present problem on 2-3 slides and receive input from a panel of regulators** *Chairs: Elina Asikanius (fimea, FI; SAWP member) and Kaspar Rufibach (CH)* Panellists: Frank Pétavy (EMA, NL), Benjamin Hofner (PEI, DE), Lukas Aguirre Dávila (PEI, DE; SAWP member), Kit Roes (EMA MWP Chair, NL), Bergrún Magnusdottir (IMA, IS), Andreas Brandt (BfArM, DE), Florian Klinglmueller (AGES, AT), Eva Skovlund (NOMA, NO; CHMP member), Khadija Rantell (MHRA, UK) Topic 1: Judith Anzures-Cabrera, Annabelle Monnet, and

		Alex Strasak (Roche): Estimand Strategies for Handling Deaths in Early-Stage Neurological Disorder Studies Topic 2: James Bell (Elderbrook Solutions GmbH), Thomas Drury (GlaxoSmithKline), Tobias Mütze (Novartis Pharma AG), Christian Bressen Pipper (Novo Nordisk A/S), Marian Mitroiu (Biogen International GmbH), Khadija Rerhou Rantell (MHRA), Marcel Wolbers (Roche), David Wright (AstraZeneca): Estimation methods for estimands using the treatment policy strategy Topic 3: Fredrik Öhrn (J&J): Two trials rule versus pooled trials rule Topic 4: Marc Buyse and Samuel Salvaggio (One2Treat): Testing procedure for multiple treatments and multiple outcomes Topic 5: Kostas Sechidis, Mark Baillie, and Bjorn Bornkamp (Novartis): What are the Quality Standards for Exploratory Analyses? Topic 6: Hong Sun (BMS): Contribution of Sequence
13:15-14:45	90	Lunch break and poster session: ESIGs and EFSPI Working Groups
14:45-17:00	135	**Session 2: Estimands – Celebrating the 5th anniversary of ICH E9(R1) – what have we learned and where do we need to go?** *Chairs: Andreas Brandt (BfArM, DE) and Vivian Lanius (Bayer, DE)* Talk 1: Estimands: Implemented, but not fully embraced. Speakers: Frank Bretz (Novartis, CH, virtual) and Rob Hemmings (Consilium, UK) Talk 2: Clinical Perspectives on Estimand Framework Implementation. Speaker: Miya Okada Paterniti (FDA, US, virtual) Talk 3: Implementation of the estimand framework in the regulatory assessment: How it started and how it's going. Speaker: Laura Rodwell (Medicines Evaluation Board, NL) Panel discussion with the audience Panellists: Speakers and John Johnston (MHRA, UK), Florian Lasch (EMA, NL) and Greg Levin (FDA, US, virtual)
17:00-19:00	120	**Wine tasting** organised by Hans Ulrich Burger (CH) and Emmanuel Zuber (Independent consultant, CH)

12th September 2024, 0830-1800, Day 2

Time	**Duration (mins)**	**Presentation**
8.30-10.30	120	**Session 3: Regulatory landscape in China** *Chairs: Kit Roes (Chair of MWP EMA, NL) and Emmanuel Zuber (Independent consultant, CH)*

		Talk 1: Opportunities and Challenges in Clinical Research under China's Scientific Regulatory System: Focusing on Innovative Drug Development. Speaker: Dr Duanduan Cong (CDE, CN) Talk 2: Implementation of ICH Statistical Guidelines in China: from the Regulatory Perspective. Speaker: Dr Jianhong Pan (CDE, CN) Talk 3: Intelligent regulation and statistics promote the modern development of regulatory science in China. Speaker: Prof Hou (Peking University, CN) Talk 4: Joint Efforts for Innovative Drug Development in China. Speaker: Dr Xiaoni Liu (Novartis, CN) Q&A
10.30-11.00	30	Coffee break
11:00-13:00	120	**Session 4: Patient preferences** *Chairs: Heidi Mestl (NOMA, NO; SAWP member) and Giulia Zigon (GSK, IT)* Talk 1: Industry case study. How a patient preference study impacted the approval/SmPC. Speaker: Brett Hauber (Pfizer, US) Talk 2: ICH E22 General Considerations for Patient Preference Studies. Speaker: Dr Francesco Pignatti (EMA, NL) Talk 3: Summary of Product Characteristics, section 5.1; what can the industry statistician do to ensure patient relevant data is included? Speakers: Elina Asikanius (fimea, FI; SAWP member) and Mouna Akacha (Novartis, CH) Talk 4: Selecting the treatment – my patient and statistician perspectives. Speaker: Anna Wiksten (CH) Panel discussion with the audience: Speakers and Johan Hellsten (Lundbeck, DK)
13.00-14.30	90	Lunch break
14:30-16:00	90	**Session 5: Openstatsware - How can we build a scalable ecosystem?** *Chairs: Lukas Aguirre Dávila (PEI, DE; SAWP member) and Pierre Mancini (Sanofi, FR)* Talk 1: General GCP principles with focus on software. Speaker: Sarianne Päivike, (fimea, FI) Talk 2: openstatsware, pharmaverse, validation, and Roche filing experience. Speaker: Juha-Pekka Perttola (Roche, CH) Talk 3: Experiences from FDA with open-source submissions. Speaker: Paul Schuette (FDA, US, Virtual) Panel discussion with the audience: Speakers and Benjamin Hofner (PEI, DE), Florian Klinglmueller (AGES, AT), Tobias Fellinger (AGES, AT), Eftychia- Eirini Psarelli (EMA, NL), Alessandro Gasparini (Red Door Analytics, SE) and Steffen Falgreen Larsen (Novo Nordisk, DK)

16.00-16.30	30	Coffee break
16:30-18:00	90	**Session 6: Regulatory and HTA updates** *Chairs: Aysun Cetinyurek Yavuz (Dutch Medicine Evaluation Board, NL) and Julie Jones (Novartis, CH)* Talk 1: EMA Methodology Working Party update – bridge to the future. Speaker: Kit Roes (Chair of MWP EMA, NL) Talk 2: Statistical Updates from the United States Food and Drug Administration (FDA) Center for Drug Evaluation and Research (CDER). Speaker: Greg Levin (FDA, US, Virtual) Talk 3: Update on Methodological Guideline Development from the HTA Coordination Group. Speaker: David McConnell (National Centre for Pharmacoeconomics, IE) Q&A

13th September 2024, 0845-1200, Day 3

Time	**Duration (mins)**	**Presentation**
8.45-9.45	60	**Session 7: Innovative Methods for Indirect Treatment Comparisons in EU HTA: Key Considerations form Trial Design to Implementation** *Chair: Katrin Kupas (BMS, CH)* Talk 1: What innovations in ITC methodology do HTA bodies want to encourage? Speaker: David McConnell (National Centre for Pharmacoeconomics, IE) Talk 2: Considerations for Methodological Innovation for Indirect Treatment Comparisons in EU HTA. Speaker: Antonio Remiro Azócar (Novo Nordisk, ES)
9.45-10.15	30	**Coffee break**
10.15-11.15	60	**Session 8: Opportunities and barriers for innovative methodology in EU HTA** *Chair: Sandro Gsteiger (Roche, CH)* Talk 1: Proper Prior Planning for Pre-specified Post-hoc (Analysis of) PICOs: How Statisticians can address the opportunities and challenges of EU HTA. Speaker: Lara Wolfson (MSD, CH) Talk 2: SUSTAIN-HTA, an EU-wide initiative to build a supporting infrastructure to ensure the ongoing implementation of the latest and fit-for-purpose HTA methodologies and tools in practice. Speaker: Wim Goettsch (Utrecht University and SUSTAIN-HTA, NL)

11.15-11.45	30	**Panel discussion** *Chair: Sandro Gsteiger (Roche, CH)* Panellists: David McConnell (National Centre for Pharmacoeconomics, IE) Antonio Remiro Azócar (Novo Nordisk, ES) Lara Wolfson (MSD, CH) Wim Goettsch (Utrecht University and SUSTAIN-HTA, NL) Anna Wiksten (CH)
11.45-12.00	15	**Closure** Helle Lynggaard (Novo Nordisk, Local Organizing and Scientific Committee, DK)
12.00-13.30	90	**Lunch**

Posters:

ESIG/EFSPI Working Group	**Title**	**Author(s)/Presenter(s)**
EIWG – reporting sub-team	Realizing the benefits of estimands when reporting and communicating study results – some recommendations	Barbara Glocker, Suvi Rajamaki, Vivian Lanius (Bayer AG), Brennan Kahan (UCL), Christian Loesch (UCB), Daniel Bratton (GSK), Francesca Callegari, Melanie Wright (Novartis), Maarten van Dijk (Staburo)
EIWG – estimands in non-inferiority trials	Considerations when Selecting Strategies for Intercurrent Events in Non-inferiority Studies	Sue McKendrick (PPD), David Wright (AstraZeneca), Helle Lynggaard (Novo Nordisk) and Sunita Rehal (GSK)
EIWG – intercurrent events	An Appraisal of the ICH E9(R1) Intercurrent Event Definition with Case Examples	Stefan Englert (J&J), Sue McKendrick (PPD) and Khadija Rantell (MHRA)
Launch & Lifecycle	Data Voyagers: Navigating the Fascinating Universe of Medical Affairs Statistics	Jenny Devenport (Roche) and Yulia Dyachkova (Merck)
Regulatory ESIG	Regulatory Special Interest Group	Alessandro Previtali (BMS), Mark Whitlock (GSK) and Yolanda Barbachano (Biontech)
Openstatsware (Software Engineering) ESIG	openstatsware – let's improve open-source statistical software together!	Alessandro Gasparini (Red Door Analytics)
Subgroup ESIG	Overview of Activities of Subgroup Analysis SIG	B. Bornkamp, B. Ratitch, K. Sechidis, David Svensson on behalf of the Subgroup Analysis European Special Interest Group
Causal inference ESIG	Introducing the Causal Inference Special Interest Group	Alex Ocampo, Kelly Van Lancker, Sanne Roels, Jesper Madsen, and Tim Morris
RWD ESIG	Welcome to the Real World Data SIG!	Elizabeth Merrall and Helen Broadhurst on behalf of the SIG.

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HTA ESIG	Improving Patient Access during Phase 2-3 Design – Things to Consider	Claudia Nicolay (Eli Lilly) and Michael Schlichting (Merck Healthcare KGaA)
Oncology Estimand WG - Conditional and Marginal Effects Task Force	Outcome of Survey on Current Standards and Implementation of Covariate Adjusted and Stratified Analyses.	Sarwar I. Mozumder, Jiawei Wei on behalf of Oncology Estimand WG - Conditional and Marginal Effects Task Force
Biomarker	Biomarkers ESIG – mission and updates	Konstantinos Sechidis
Neuroscience Estimand Working Group	The Neuroscience Estimand eSIG - an overview. Scope, objectives and a look into the future.	Marisa Bacchi, Marian Mitroiu, Paul Delmar, Rachid Abbas, Hans Ulrich Burger, Andrew Hartley, Mette Krog Josiassen, Lars Lau Raket, Peter Quarg, Khadija Rantell, Nikolaos Sfikas
BBS NextGen	Today, Tomorrow and the Future: Summary of BBS Next Generation in 2024 and going forward	Joana Marques Barros, Muriel Buri, Youyou Hu, Antonella Mazzei, Olympia Papachristofi, Ottavia Prunas, Kristina Weber, Lukas Andreas Widmer, Manuela Zimmermann, Hans Ulrich Burger
Statistics Methods Leaders	Statistical Methodology Leaders in Drug Development – a new EFSPI working group	M Akacha, N Best, R El-Galta, H Goette, P Hougaard, J Hummel, C Kunz, V Lanius, T Mielke, N Muhlemann, C Pipper, K Rufibach, M Vandemeulebroecke, D Wright