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

Draft program as of 21 July 2024

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 much it matters to be first in class. And how can you catch up? 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 (Roche, 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)
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 (Roche, CH)* Panellists: Frank Pétavy (EMA, NL), Benjamin Hofner (PEI, DE), Lukas Aguirre Dávila (PEI, DE; SAWP member), Heidi Mestl (NOMA, NO; 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: Topic 2: Topic 3: Topic 4: Topic 5: Topic 6: Please send proposals by 15 August to hlyn@novonordisk.com
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: What has improved and what new issues did we uncover and what issues are we still ignoring? Speakers: Frank Bretz (Novartis, CH) and Rob Hemmings (Consilium, UK) Talk 2: Implementation of the estimand framework in the regulatory assessment. How are clinicians and industry involved? Speaker: Laura Rodwell (Medicines Evaluation Board, NL) Talk 3: How has the estimand framework impacted the regulatory process in FDA? Are we discussing the clinical question of interest? What do stakeholders need from statisticians to embrace the framework? Speaker: Miya Okada Paterniti (FDA, US, virtual) Panel discussion with the audience Panellists: Speakers and Florian Lasch (EMA, NL), Greg Levin (FDA, US, virtual) and John Johnston (MHRA, UK)
17:00-19:00	120	**Wine tasting** organised by Hans Ulrich Burger (Roche, CH) and Emmanuel Zuber (Independent consultant, FR)

12th September 2024, 0830-1700, Day 2

Time	**Duration (mins)**	**Presentation**
8.30-10.00	90	**Session 3: Regulatory landscape in China** *Chairs: Kit Roes (Chair of MWP EMA, NL) and Emmanuel Zuber (Independent consultant, FR)* Q&A

10.00-10.30	30	Coffee break
10:30-12:30	120	**Session 4: Patient centricity** *Chairs: Heidi Mestl (NOMA, NO; SAWP member) and Giulia Zigon (GSK, IT)* Talk 1: ICH E22 General Considerations for Patient Preference Studies. Speaker: Dr Francesco Pignatti (Rapporteur (EC), IT) Talk 2: 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 3: Industry case study. How a patient preference trial impacted the approval/SmPC. Speaker: Brett Hauber (Pfizer, US) 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)
12.30-14.00	90	Lunch break
14:00-15:30	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) and Alessandro Gasparini (Red Door Analytics, SE)
15:30-17:00	90	**Session 6: Regulatory and HTA updates** *Chairs:* Talk 1: Speaker: Kit Roes (Chair of MWP EMA, NL) Talk 2: Speaker: Greg Levin (FDA, US, Virtual) Talk 3: 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** *Chairs: Katrin Kupas (BMS, CH)* Speakers: David McConnell (National Centre for Pharmacoeconomics, IE) 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** *Chairs: Antonia Morga (Astellas Pharma, UK)* Speakers: Lara Wolfson (MSD, CH) Wim Goettsch (Utrecht University and SUSTAIN-HTA, NL)
11.15-11.45	30	**Panel discussion** *Chairs: Antonia Morga (Astellas Pharma, UK)* 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)

Confirmed posters:

ESIG/EFSPI Working Group	**Title**	**Author(s)/Presenter(s)**
Estimand Implementation Working Group (EIWG)		
EIWG – reporting sub-team	Realizing the benefits of estimands when reporting and communicating study results – some recommendations	
EIWG – estimands in non-inferiority trials		Sue McKendrick (PPD), David Wright (AstraZeneca), Helle Lynggaard (Novo Nordisk), Chrissie Fletcher (GSK) and Sunita Rehal (GSK)

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

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		
RWD ESIG		
HTA ESIG	Improving Patient Access during Phase 3 Design – Things to Consider	Claudia Nicolay and Michael Schlichting
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
BBS NextGen		
Statistics Methods Leaders		