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

Draft program as of 20 August 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 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)
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), 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 preferences** *Chairs: Heidi Mestl (NOMA, NO; SAWP member) and Giulia Zigon (GSK, IT)*

		Talk 1: Industry case study. How a patient preference trial 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)
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), Alessandro Gasparini (Red Door Analytics, SE) and Steffen Falgreen Larsen (Novo Nordisk, DK)
15:30-17:00	90	**Session 6: Regulatory and HTA updates** *Chairs: Aysun Cetinyurek Yavuz (NL) and Julie Jones (Novartis, CH)* 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**

		Chair: 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** *Chair: Sandro Gsteiger (Roche, CH)* Speakers: Lara Wolfson (MSD, CH) 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**

Confirmed posters:

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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		
RWD ESIG		
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[1], Marian Mitroiu[2], Paul Delmar[3] , Rachid Abbas[3], Hans Ulrich Burger, Andrew Hartley[4], Mette Krog Josiassen[5], Lars Lau Raket[6] , Peter Quarg[7], Khadija Rantell[8], Nikolaos Sfikas[7] Affiliation: [1]Consultant, [2]Biogen,[3]F.Hoffmann-La Roche, [3]MHRA, [4]PPD, [5]H.Lundbeck A/S, [6]Eli Lilly , [7]Novartis
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