

9<sup>th</sup> EFSPi Regulatory Statistics Workshop  
11-13 September 2024  
Basel Switzerland

**Draft program as of 8 August 2024**

10<sup>th</sup> 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-payd) event. The get-together will take place in a relaxed and informal atmosphere in a restaurant on **Sept 10<sup>th</sup>, 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](mailto:Heidi.Mestl@legemiddelverket.no)) or Benjamin Hofner ([Benjamin.Hofner@pei.de](mailto:Benjamin.Hofner@pei.de)).

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

| Time        | Duration (mins) | Presentation  |
|-------------|-----------------|---|
| 8:30-8:45   | 15              | <b>Opening remarks</b><br><br>Egbert Biesheuvel (EFSPi President, Viatris, NL)  |
| 8:45-10:45  | 120             | <b>Session 1: Fast to market vs. robustness of the data</b><br><br><i>Chairs: Khadija Rantell (MHRA, UK) and Fredrik Öhrn (J&amp;J, SE)</i><br><br>Talk 1: How much it matters to be first in class. And how can you catch up? Speaker: Jenny Devenport (Roche, CH)<br><br>Talk 2: Conditional marketing authorisation. Speaker: Eva Skovlund (NOMA, NO; CHMP member)<br><br>Talk 3: CLL11 – a trial tailored to answer questions from many stakeholders efficiently. Speaker: Kaspar Rufibach (Roche, CH)<br><br>Talk 4: Fast and furious to market across Pharma, is it good for HTA? Speakers: Karin Cerri and Lilla di Scala (J&J, CH)<br><br>Panel discussion with the audience:<br>Speakers and Bergrún Magnúsdóttir (IMA, IS) and Peter Ahnesorg (Roche) |
| 10.45-11.15 | 30              | Coffee break  |
| 11:15-13:15 | 120             | <b>Short topics (20 mins per topic): Present problem on 2-3 slides and receive input from a panel of regulators</b><br><br><i>Chairs: Elina Asikanius (fimeq, FI; SAWP member) and Kaspar Rufibach (Roche, CH)</i><br><br>Panellists:<br>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 Magnúsdóttir (IMA, IS), Andreas Brandt (BfArM, DE), Florian   |

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|             |     | <p>Klinglmueller (AGES, AT), Eva Skovlund (NOMA, NO; CHMP member), Khadija Rantell (MHRA, UK)</p> <p>Topic 1:</p> <p>Topic 2:</p> <p>Topic 3:</p> <p>Topic 4:</p> <p>Topic 5:</p> <p>Topic 6:</p> <p>Please send proposals by 15 August to <a href="mailto:hlyn@novonordisk.com">hlyn@novonordisk.com</a></p>  |
| 13:15-14:45 | 90  | Lunch break and poster session: ESIGs and EFSPi Working Groups   |
| 14:45-17:00 | 135 | <p><b>Session 2: Estimands – Celebrating the 5th anniversary of ICH E9(R1) – what have we learned and where do we need to go?</b></p> <p><i>Chairs: Andreas Brandt (BfArM, DE) and Vivian Lanius (Bayer, DE)</i></p> <p>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)</p> <p>Talk 2: Implementation of the estimand framework in the regulatory assessment. How are clinicians and industry involved? Speaker: Laura Rodwell (Medicines Evaluation Board, NL)</p> <p>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)</p> <p>Panel discussion with the audience</p> <p>Panellists:<br/>Speakers and Florian Lasch (EMA, NL), Greg Levin (FDA, US, virtual) and John Johnston (MHRA, UK)</p> |
| 17:00-19:00 | 120 | <b>Wine tasting</b> organised by Hans Ulrich Burger (Roche, CH) and Emmanuel Zuber (Independent consultant, FR)  |

12<sup>th</sup> September 2024, 0830-1700, Day 2

| Time       | Duration (mins) | Presentation  |
|------------|-----------------|---|
| 8.30-10.00 | 90              | <p><b>Session 3: Regulatory landscape in China</b></p> <p><i>Chairs: Kit Roes (Chair of MWP EMA, NL) and Emmanuel Zuber (Independent consultant, FR)</i></p> <p>Q&amp;A</p> |

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| 10.00-10.30 | 30  | Coffee break  |
| 10:30-12:30 | 120 | <p><b>Session 4: Patient centricity</b></p> <p><i>Chairs: Heidi Mestl (NOMA, NO; SAWP member) and Giulia Zigon (GSK, IT)</i></p> <p>Talk 1: ICH E22 General Considerations for Patient Preference Studies. Speaker: Dr Francesco Pignatti (Rapporteur (EC), IT)</p> <p>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)</p> <p>Talk 3: Industry case study. How a patient preference trial impacted the approval/SmPC. Speaker: Brett Hauber (Pfizer, US)</p> <p>Talk 4: Selecting the treatment – my patient and statistician perspectives. Speaker: Anna Wiksten (CH)</p> <p>Panel discussion with the audience: Speakers and Johan Hellsten (Lundbeck, DK)</p> |
| 12.30-14.00 | 90  | Lunch break   |
| 14:00-15:30 | 90  | <p><b>Session 5: Openstatsware - How can we build a scalable ecosystem?</b></p> <p><i>Chairs: Lukas Aguirre Dávila (PEI, DE; SAWP member) and Pierre Mancini (Sanofi, FR)</i></p> <p>Talk 1: General GCP principles with focus on software. Speaker: Sarianne Päivike, (fimea, FI)</p> <p>Talk 2: openstatsware, pharmaverse, validation, and Roche filing experience. Speaker: Juha-Pekka Perttola (Roche, CH)</p> <p>Talk 3: Experiences from FDA with open-source submissions. Speaker: Paul Schuette (FDA, US, Virtual)</p> <p>Panel discussion with the audience: Speakers and Benjamin Hofner (PEI, DE), Florian Klinglmueller (AGES, AT), Tobias Fellingner (AGES, AT), Alessandro Gasparini (Red Door Analytics, SE) and Steffen Falgreen Larsen (Novo Nordisk, DK)</p>   |
| 15:30-17:00 | 90  | <p><b>Session 6: Regulatory and HTA updates</b></p> <p><i>Chairs: Aysun Cetinyurek Yavuz (NL) and Julie Jones (Novartis, CH)</i></p> <p>Talk 1: Speaker: Kit Roes (Chair of MWP EMA, NL)</p> <p>Talk 2: Speaker: Greg Levin (FDA, US, Virtual)</p> <p>Talk 3: Speaker: David McConnell (National Centre for Pharmacoeconomics, IE)</p> <p>Q&amp;A</p>   |

13<sup>th</sup> September 2024, 0845-1200, Day 3

| Time | Duration (mins) | Presentation |
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| 8.45-9.45   | 60 | <b>Session 7: Innovative Methods for Indirect Treatment Comparisons in EU HTA: Key Considerations form Trial Design to Implementation</b><br><br><i>Chairs: Katrin Kupas (BMS, CH)</i><br><br>Speakers:<br>David McConnell (National Centre for Pharmacoeconomics, IE)<br>Antonio Remiro Azócar (Novo Nordisk, ES)      |
| 9.45-10.15  | 30 | <b>Coffee break</b>   |
| 10.15-11.15 | 60 | <b>Session 8: Opportunities and barriers for innovative methodology in EU HTA</b><br><br><i>Chairs: Antonia Morga (Astellas Pharma, UK)</i><br><br>Speakers:<br>Lara Wolfson (MSD, CH)<br>Wim Goettsch (Utrecht University and SUSTAIN-HTA, NL)   |
| 11.15-11.45 | 30 | <b>Panel discussion</b><br><br><i>Chairs: Antonia Morga (Astellas Pharma, UK)</i><br><br>Panellists:<br>David McConnell (National Centre for Pharmacoeconomics, IE)<br>Antonio Remiro Azócar (Novo Nordisk, ES)<br>Lara Wolfson (MSD, CH)<br>Wim Goettsch (Utrecht University and SUSTAIN-HTA, NL)<br>Anna Wiksten (CH) |
| 11.45-12.00 | 15 | <b>Closure</b><br><br>Helle Lynggaard (Novo Nordisk, Local Organizing and Scientific Committee, DK)   |

**Confirmed 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 |  |
| EIWG – estimands in non-inferiority trials |   | Sue McKendrick (PPD), David Wright (AstraZeneca), Helle Lynggaard (Novo Nordisk), Chrissie Fletcher (GSK) 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)  |

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|--|--|---|
| 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          |
| Biomarker  | Biomarkers ESIG – mission and updates  |   |
| Neuroscience Estimand Working Group                                |  |   |
| BBS NextGen  |  |   |
| Statistics Methods Leaders   |  |   |