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

**Draft program as of 28 June 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**<br><br>Egbert Biesheuvel (EFSPI President, Senior Director Viatris) |
| 8:45-10:45 | 120 | **Session 1: Fast to market vs. robustness of the data**<br><br>Talk 1: How much it matters to be first in class. And how can you catch up? Speaker: Jenny Devenport (Roche)<br><br>Talk 2: Conditional marketing authorisation. Speaker: Eva Skovlund, NOMA<br><br>Talk 3: CLL11 – a trial tailored to answer questions from many stakeholders efficiently. Speaker: Kaspar Rufibach (Roche)<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)<br><br>Panel discussion with the audience |
| 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**<br><br>Confirmed panellists:<br>Frank Pétavy, Benjamin Hofner, Lukas Aguirre Davila, Heidi Meistl, Kit Roes, Bergrun Magnusdottir, Andreas Brandt, Florian Kilngmuller, Eva Skovlund, Khadija Rantell<br><br>*Chairs: Elina Asikanius (fimea, EMA) and Kaspar Rufibach (Roche)*<br><br>Topic 1:<br><br>Topic 2:<br><br>Topic 3:<br><br>Topic 4: |

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| | | Topic 5:<br><br>Topic 6:<br><br>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?**<br><br>Talk 1: What has improved and what new issues did we uncover and what issues are we still ignoring? Speakers: Frank Bretz, Novartis and Rob Hemmings, Consilium<br><br>Talk 2: Implementation of the estimand framework in the regulatory assessment. How are clinicians and industry involved? Speaker: Laura Rodwell, EMA<br><br>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, MD, FDA<br><br>Panel discussion with the audience<br>Panellists:<br>Florian Lasch, EMA<br>Greg Levin, FDA |
| 17:00-19:00 | 120 | **Wine tasting** organised by Hans Ulrich Burger (Roche) and Emmanuel Zuber (Novartis) |

12th September 2024, 0830-1700, Day 2

| **Time** | **Duration (mins)** | **Presentation** |
|---|---|---|
| 8.30-10.00 | 90 | **Session 3: How can statisticians navigate the interface between the EU Joint Clinical Assessment and EMA regulatory assessment?**<br><br>Panel discussion with the audience |
| 10.00-10.30 | 30 | Coffee break |
| 10:30-12:30 | 120 | **Session 4: Patient centricity**<br><br>*Chairs:*<br><br>Talk 1: ICH E22 General Considerations for Patient Preference Studies. Speaker: Dr Francesco Pignatti, Rapporteur (EC)<br><br>Talk 2: Summary of Product Characteristics, section 5.1; what can the industry statistician do to ensure patient relevant data is included? Speaker: Elina Asikanius, fimea.<br><br>Talk 3: Industry case study. How a patient preference trial impacted the approval/SmPC. Speaker: Brett Hauber, Pfizer |

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| | | Talk 4: Selecting the treatment – my patient and statistician perspectives. Speaker: Anna Wiksten<br><br>Panel discussion with the audience |
| 12.30-14.00 | 90 | Lunch break |
| 14:00-15:30 | 90 | **Session 5: Openstatsware - How can we build a scalable ecosystem?**<br>Talk 1: General GCP principles with focus on software. Speaker: Sarianne Päivike, (fimea)<br>Talk 2: Introducing openstatsware: improving software engineering for biostatisticians. Speaker: Daniel Sabanés Bové, Roche<br>Panel discussion with the audience<br>Panelists:<br>Benjamin Hofner, PEI<br>Tobias Fellinger, EMA |
| 15:30-17:00 | 90 | **Session 6: Regulatory updates**<br>Talk 1: Speaker: Kit Roes Radboud University, Netherlands, EMA<br>Talk 2: Speaker: Greg Levin, FDA<br>Q&A |

13th September 2024, 0830-1200, Day 3

| **Time** | **Duration (mins)** | **Presentation** |
|---|---|---|
| 8.30-9.45 | 75 | **Session 7: Regulatory landscape in China**<br><br>Q&A |
| 9.45-10.15 | 30 | **Coffee break** |
| 10.15-11.45 | 90 | **Session 8: The role of innovative statistical methods in pan-European HTA**<br><br>Panel discussion with the audience |
| 11.45-12.00 | 15 | **Closure**<br>Helle Lynggaard (Novo Nordisk, Local Organizing and Scientific Committee) |

**Confirmed posters:**

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| **ESIG/EFSPI Working Group** | **Title** | **Author(s)/Presenter(s)** |
|---|---|---|
| Estimand Implementation Working Group (EIWG) | | |
| EIWG – reporting sub-team | | |
| 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 | | |
| Launch & Lifecycle | | 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) |
| Statistics Methods Leaders | | |