EFSPI

European Federation of Statisticians in the Pharmaceutical Industry

Representing Statistical Associations in Europe

4th EFSPI Regulatory Workshop

4th EFSPI Workshop on Regulatory Statistics – draft program

23/24th September 2019 Basel (CH)

After three very successful workshops on regulatory statistics in the past three years, EFSPI is pleased to announce the 4th regulatory statistics workshop taking place in Basel on 23rd and 24th September 2019.

The workshop will be dedicated to the discussion of opportunities and challenges of statistical topics between regulators, academics, and industry with dedicated time for interaction and discussion.

Members of the Scientific Committee are: Egbert Biesheuvel, Birgitte Biilmann Roenn, Andreas Brandt, Hans Ulrich Burger, Christoph Gerlinger, Randi Gron, Benjamin Hofner, Armin Koch, Flavia Lombardo, Frank Petavy, Khadija Rantell, Kaspar Rufibach, Anja Schiel, Emmanuel Zuber.

Monday 23rd September

13:30 **Welcome**

13:40 **Session 1: Real-world data – beyond randomized clinical trials**

15:10 **Coffee break**

Venue

Oekolampad Church
Allschwilerplatz 22
CH – 4055 Basel
Switzerland

Registration Costs

Fee includes lunch & refreshments

Early bird before or on 15th of August

Industry	€250
Academic	€175

After 15th of August

Industry	€300
Academic	€225

Program

	23rd September
13:30-13:40	Welcome
13:40-15:10	Session 1: Real-world data – beyond randomized clinical trials
90min	Chairs: Benjamin Hofner & Christoph Gerlinger
	Kit Roes (Utrecht Medical Center) RWD, RWE, big data, external control, digital biomarker – what do they all mean? (25min) Dominik Heinzmann (Roche) and Simon Wandel (Novartis) BBS spring seminar external controls: summary & what happened since then? (25min) Fabian Model (Roche) & Nikos Sfikas (Novartis) A digital biomarker/endpoint in multiple sclerosis, and regulatory implications (20min) Speaker (Company) An example of a pragmatic trial, and estimand considerations (20min)
15:10-15:40 30min	Coffee break
15:40-17:25	Session 2: Real-world data – applications
105min	Chairs: Frank Petavy & Hans Ulrich Burger
	Kate Taylor (Amgen) Blincyto ODAC experience (20min) Markus Abt (Roche) A digital endpoint in Parkinson (20min) Christoph Gerlinger (Bayer) Using RWD to extrapolate evidence from RCTs (20min) Benjam Hofner (PEI) RWD aspects in a gene-therapy approval (20min) Regulator () What data will regulators have to deal with in the future? (25min)
17:25-18:00 35min	Panel discussion with the chairs and all speakers
17:45-19:00 75min	Reception

Program

	24th September	
9:00-10:30	Session 3: Estimands – are we pushing any boundaries thanks to the ICH E9 addendum?	
90min	Chairs: Andreas Brandt & Emmanuel Zuber	
	Khadija Rantell & Ines Reis (MHRA) How the estimand framework becomes standard practice in applications, and where we still need to learn (20min) Speaker from Oncology and Neuroscience estimand WGs () Case studies and/or experience of working with regulators (20min) Lorenzo Guizzaro (EMA) Regulatory experience with the estimand framework (20min) Panel discussion with the chairs and all speakers (30min)	
10:30-11:00 30min	Coffee break	
11:00-12:30	Session 4: Analysis of safety in clinical trials – or how to bring a statistician out of his comfort zone	
90min	Chairs: Khadija Rantell & Egbert Biesheuvel	
	Tim Friede (University of Goettingen, on behalf of SAVVY working group) Comparison of statistical methods to analyse safety data (15min) Jim Slattery (EMA) (15min) Gian Thanei (Roche) Interactive graphical reporting of safety data, rolled-out globally (15min) Steffen Falgreen Larsen (Novo Nordisk) A shiny app to explore hypoglycemic episodes and adverse events for a pool of trials (15min) Hans Ulrich Burger (Roche) How statisticians deal with the difference between efficacy and safety reporting (10min) Panel discussion with the chairs and all speakers (20min)	
12:30-13:30 60min	Lunch break	

Program

13:30-15:00	**Session 5: Modern approaches for rare disesase**	
90min	Chairs: Armin Koch & Egbert Biesheuvel	
	Speaker from the rare disease EFSPI SIG () (15min) Two speakers (method + content) from Asterix () Goal attainment scaling endpoints in RCTs (15min each) Pierre Demolis () A regulator's view on rare disease drug development (20min) Panel discussion with the chairs and all speakers (25min)	
15:00-15:30 **30min**	Coffee break	
15:30-16:45	**Session 6: Contributed short topics – discussions**	
75min	**Chairs:**	
	Up to 6 topics from practice will briefly be presented (5 min) followed by a 10-15 min discussion of the panel and with audience	**Proposals of topics can be addressed until August 31 to either Armin Koch (koch.armin@mh-hannover.de) or Hans Ulrich Burger (hans_ulrich.burger@roche.com)**
16:45	Official closure of the meeting	

Session 6

- Up to 6 topics from practice will briefly be presented (5 min) followed by a 10-15 min discussion of the panel and with audience
- Proposals of topics can be addressed until August 31 to either Armin Koch (koch.armin@mh-hannover.de) or Hans Ulrich Burger (hans_ulrich.burger@roche.com)
- Great opportunity to get direct HA feedback
- Sessions in the past were great but topics came in always last minute
- Are we able to change?