4th EFSPI Workshop on Regulatory Statistics

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, 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**
15:40	**Session 2: Real-world data – applications**
17:10	**Panel discussion with all speakers**
18:00	**Reception**

Tuesday 24th September

09:00	**Session 3: Analysis of safety in clinical trials – or how to bring a statistician out of his comfort zone**
10:30	**Coffee break**
11:00	**Session 4: Estimands – are we pushing any boundaries thanks to the ICH E9 addendum?**
12:30	**Lunch break**
13:30	**Session 5: Modern approaches for rare disease**
15:00	**Coffee break**
15:30	**Session 6: Contributed short topics – discussions**
16:45	**Official closure of the meeting**

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

Hotel Rooms

Bildungszentrum 21

http://www.bildungszentrum-21.ch

(mention EFSPI workshop)

To Register Please Go To
www.efspi.org

Or contact:

EFSPI Secretariat
Tel: +44 (0)1625 664549
efspi@kingstonsmith.co.uk

For information on the scientific content, contact the Scientific Committee

Proposals for short topics for Session 6, please contact either Armin Koch (koch.armin@mh-hannover.de) or Hans Ulrich Burger (hans_ulrich.burger@roche.com) by August 31st

4th EFSPI Workshop on Regulatory Statistics

	23rd September
13:30-13:40	**Welcome**
13:40-15:10 90min	**Session 1: Real-world data – beyond randomized clinical trials** **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)** Development of a digital endpoint in Multiple Sclerosis - challenges and opportunities (20min) **Hendrik Schmidt (Boehringer-Ingelheim)** A look on Best Practices in Pragmatic Trials (20min)
15:10-15:40	**Coffee break**
15:40-17:25 105min	**Session 2: Real-world data – applications** **Chairs:** Frank Petavy & Hans Ulrich Burger
	Kate Taylor (Amgen) Single-arm study plus a historical comparator equals two historic regulatory approvals – my experiences with the Blincyto MRD filings (20min) **Stanislas Hubeaux (Roche)** Development of a smartphone based monitoring tool for people with Multiple Sclerosis - challenges and opportunities (20min) **Christoph Gerlinger (Bayer)** Using RWD to extrapolate evidence from RCTs (20min) **Benjam Hofner (PEI) and Khadija Rantell (MHRA)** RWD aspects in a gene-therapy approval (20min) **Stephen Evans (London School of Hygiene and Tropical Medicine)** How far can we trust the Real World? (25min)
17:25-18:00 35min	**Panel discussion with the chairs and all speakers**
17:45-19:00	**Reception – German and French wine tasting!**

	24th September
09:00-10:30 90min	**Session 3: Analysis of safety in clinical trials – or how to bring a statistician out of his comfort zone** **Chairs:** Khadija Rantell & Kaspar Rufibach
	Tim Friede (University of Goettingen, on behalf of SAVVY working group) Comparison of statistical methods to analyse safety data (15min) **Gian Thanei (Roche)** Pooling and harmonizing of safety data for a robust statistical analysis (15min) **Steffen Falgreen Larsen (Novo Nordisk)** A shiny app to explore hypoglycemic episodes and adverse events for a pool of trials (15min) **John Johnston (MHRA)** Matching up (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)**
10:30-11:00 30min	**Coffee break**
11:00-12:30 90min	**Session 4: Estimands – are we pushing any boundaries thanks to the ICH E9 addendum?** **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) **Evgeny Degtyarev (Novartis)** How the estimand framework pushes boundaries in oncology (15min) **Georg Kralidis (Gruenenthal) and Marcel Wolbers (Roche)** Treatment policy and hypothetical strategies for intercurrent events in chronic pain and Parkinson's disease (20min) **Lorenzo Guizzaro (EMA)** Regulatory experience with the estimand framework (15min) **Panel discussion with the chairs and all speakers (20min)**
12:30-13:30	**Lunch break**

13:30-15:00 90min	**Session 5: Modern approaches for rare diseases** **Chairs:** Armin Koch & Egbert Biesheuvel	
	Lukas Aguirre Davila (Hannover Medical School) Observational vs. randomized analyses of digoxin-mortality in the DIG trial (20min) **Charlotte Gaasterland (Kennisinstituut van Medisch Specialisten)** Goal Attainment Scaling: Validation & use for rare disease (20min) **Hilke Zander (PEI)** A regulator's view on rare cancer drug development: Histology independent indications (20min) **Panel discussion with the chairs and all speakers (30min)**	
15:00-15:30 30min	**Coffee break**	
15:30-16:45 75min	**Session 6: Contributed short topics – discussions** **Chairs:** Armin Koch & Hans Ulrich Burger	
	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**