European Federation of Statisticians in the Pharmaceutical Industry
Representing Statistical Associations in Europe

EFSPI Newsletter October 2018

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Regulatory

On 24/25th September the 3rd EFSPI Regulatory Statistics Workshop took place in Basel. With over 260 registrations (up >60 from last year) the turnout was again excellent. A few highlights from the organizer's perspective were:

- Attendance of industry, academic, and regulatory colleagues, with engaged discussion on a broad range of topics relevant to contemporary drug development.
- An asset of the workshop is how approachable regulators are, on and off stage. And they do not shy away from pronounced statements, e.g. in panel discussions.
- A series of relevant and well-presented talks. All slide decks are available on the EFSPI webpage at https://tinyurl.com/yavgb2j2
- The IDEAS presentations are also available online:
  http://www.ideas-itn.eu/dissemination-workshop/

A few quotes heard at the workshop:

- Benjamin Hofner (PEI): *Regulators are generally open for innovative topics (e.g. platform, basket, umbrella, RWE) – but go and discuss with them early!*
- Anja Schiele, Senior Statistical Advisor NOMA, chair EMA BSWP. *Resistance to single-arm trials is even larger for HTA bodies than for regulators. We prefer an underpowered RCT to a single-arm trial.*

- Rob Hemmings (MHRA): *Randomisation is (really, really) important, isn't it? Did something change? Quality of evidence is paramount. Limited scope for trade off in quality vs. cost. Is supplementing with external data conceivable? Perhaps. Commonly? No. Do not pretend it is easy to use RWD: Use three covariates for PS modelling and you are done does not work.*
- James Roger (LSHTM): *ICH E9 will drive Cox regression out of business!*
- The weather!
- The student posters and presentations from the IDEAS program.

Thanks to the Scientific Committee, all speakers, chairs, panelists, and anyone else who contributed to make this workshop a success for their work!

The local organizing committee for the EFSPI Regulatory Statistics workshop includes: Egbert Biesheuvel, Hans Ulrich Burger, Christoph Gerlinger, Kaspar Rufibach, and Emmanuel Zuber.

The regulatory committee are currently planning to comment on the following drafts:

- EMA draft: "Draft questions and answers on Data Monitoring Committees issues"
- Master Protocols: Efficient Clinical Trial Design Strategies to Expedite Development of Oncology Drugs and Biologics; draft Guidance for Industry
- Adaptive Designs for Clinical Trials of Drugs and Biologics; draft guidance for industry;
- Presenting Quantitative Efficacy and Risk Information in Direct-to-Consumer Promotional Labeling and Advertisements Guidance for Industry

Please contact Christoph Gerlinger (christoph.gerlinger@bayer.com) or Anna Berglind (anna.berglind@astrazeneca.com) if you wish to comment.

The EFSPI/PSI regulatory committee met with MHRA statisticians in September and with EMA's Biostatistics Working Party in October. Summaries of the discussions are currently being finalised and will be included in future newsletters once finalized

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Scientific

EFSPI/PSI Webinar: Do you understand the patient point of view on benefit-risk tradeoffs? Introduction and case studies of stated preference elicitation methods

Tuesday 20th November 2018 **Time:** 15:00 - 16:30 UK Time. **Presenters:** Alexander Schacht (Lilly), Marco Boeri (RTI-HS), Shahrul Mt-Isa (MSD), Brett Hauber (RTI-HS) and Daniel Saure (Lilly)

Do you know what patients value most in a treatment you are developing to reassure that they would best benefit from the drug, having considered what is important to them? For example, if a patient had to choose between a highly effective drug with a bad side effect profile and a less effective drug with minimal side effects, which would they choose? Rooted in traditional economic theory, stated-preference methods can help achieve a better understanding of the patient view point on benefit risk tradeoffs.

This webinar proposes a short primer on stated preference methods and how they can be used to explore the patient preferences for specific drug profiles that are currently available or may be developed in the near future. The theory will be then discussed as applied to two case studies using quantitative preference methods.

Registration for this webinar is free. Click here for further information and to register.

Decision making in Clinical Development, December 12

On Wednesday December 12 the scientific meeting on '*Decision making in Clinical Development*' will take place at Servier in Paris. *Taking decisions during the development of a new drug requires combining many and varying pieces of information. Decision-makers need quantitative tools to support informed decisions, with transparent processes that synthesize the whole available information in order to evaluate the success associated to different options.* More information can be found on the flyer, which is available on our website, and the registration is open.

For the first time, we will have a poster session. If you wish to present a poster, please send an abstract to Gaëlle Saint-Hilary (gsainthilary@gmail.com) by October 31st 2018. The notification of acceptance will be provided by November 9th 2018.

2019 Meetings

The Scientific Committee is planning for three 1-day scientific meetings for 2018. The first one day meeting will be on "Recent developments in biomarkers and subgroups in drug development", which will take place in March hosted by AstraZeneca in Göteborg, Sweden. Exact date and more information on the program will follow. In addition, we are starting with planning two additional 1-day events, one before summer, one after. More information will follow.

The fourth EFSPI regulatory statistics workshop will also take place in Basel in the fall 2019.

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Country News

APF (Germany)

The next APF Statistics Leaders meeting is taking place on the 22nd November 2018 in Berlin, hosted by Paraxel. Registration for the annual fall meeting on Visualization taking place on the 23. November 2018 in Berlin, click here.

A two day workshop on Bayesian methods in the development and assessment of new therapies will take place on 06-07 of December at University of Goettingen, Germany. The aim of the workshop is to provide a space for presentations and discussions of recent developments in the application of Bayesian methodology in early pharmaceutical research and health technology assessment. Invited speakers include (among others) Ralf Bender (IQWiG), Thomas Jaki (Lancaster University, Heinz Schmidli (Novartis) and Sibylle Sturtz (IQWiG).
Please find additional information at http://www.biometrische-gesellschaft.de/arbeitsgruppen/bayes-methodik/workshops/2018-goettingen.html

IBIG (Italy)

An Italian Biostatistics Group (IBIG) Forum will be organized in Padova on 22-23 November including the following topics: Bayesian Statistics in Clinical Trials, Micro-Randomized Trials and Expedited Approval Programs. The forum will take place at the NH Hotel in Padova, Italy. For more details click here.

PSI (UK)

Abstract Submission OPEN for the 2019 PSI Conference

PSI are pleased to announce that the count down to the PSI Conference 2019 has now begun! **The Conference will be held in London on the 2-5th June 2019 with Data Driven Decision Making in Medical Research as the theme.**

Please submit now any of your contributed oral or poster abstracts. We accept abstracts on any topic, but have provided a list of topics we are particularly interested in including; Data Science, Decision Making, Bayesian, Causal Inference, Pre-clinical, Future trends, PROs and many more. You can see the full list and download the abstract template here! The deadline for oral abstract submissions is **23rd November 2018**. Remember, anyone selected for an Oral presentation, will be eligible for **10% off the 3-day conference price**. If you need any final persuasion then please listen to the episode from The Effective Statistician podcast which interviews Paul Terrill, the current chair of the PSI Scientific Committee, on the benefits of submitting an abstract and his logistical and practical tips for doing so. Click here to listen to the podcast or search in your podcast app for The Effective Statistician.

FEATURED VIDEO: PSI Conference 2018: Introduction to machine learning for longitudinal medical data

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In the era of big data, there has been a surge in collected biomedical data, which has provided ample challenges for distributed computing but also posed novel inference questions. Classical machine learning techniques, such as logistic regression, neural networks, support vector machine and Gaussian processes performed very well in non-temporal prediction tasks but typically relied on the independence assumption.

However, many recent applications have longitudinal context in the form of short- and long-term dependencies. Hidden Markov Models proved popular to model longitudinal data but increasingly become less computationally feasible for a large number of hidden states. Recently, advances in parallel computing led to widespread use of deep learning approaches, such as recurrent neural networks and convolutional networks, and attracted attention due to their impressive results on sequence data. Finally, we will look in more detail at a case study from healthcare analytics which infers disease type from multiple irregularly sampled longitudinal observations,
such as blood pressure, heart rate and blood oxygen saturation

Two new exciting episodes of The Effective Statistician podcast, created in association with PSI

In these two episodes, we cover very different topics. You will learn about practical problem solving with multiplicity problems from one of the world-class experts: Alex Dmitrienko. Having worked within a big pharma company, a large CRO and running his own consulting business, you will benefit from both his technical but also practical experience. Please find the episode below:

- **Understand and master multiplicity in practical situations - Interview with Alex Dmitrienko**

Benjamin and Alexander talk about something that many statisticians struggle with - explaining achievements to others. We all have been in situations where awesome achievements weren't appropriately recognised. Listen to the latest episode on how to avoid this frustrating situation for you:

- **How to sell your achievements - actionable advise**

Search for The Effective Statistician in your podcast app and subscribe now!

PSI Webinar: Avoiding Pitfalls in Supervised/ Unsupervised Learning

Thursday 29th November 2018, 14:00 - 15:30 UK Time
Presenters: Ilya Lipkovich (IQVIA), Alexander Schacht (Lilly) and Andy Nicholls (GSK)

As the availability of big data increases and statisticians assist with predicting outcomes or understanding patterns in an ever-wider variety of scenarios then supervised and unsupervised learning methods become increasing called upon. Such machine learning algorithms offer the opportunity to understand potential predictors or clusters amongst large datasets, but are also subject to the risks of overfitting or over-interpretation. This Webinar seeks to introduce ideas and share experiences in this field.

The talks will introduce several supervised and unsupervised learning methods and cover data-driven subgroup identification in clinical trials, and case studies of implementation clustering algorithms.

Click here for further information and to register.

Toxicology Special Interest Group Free Webinars

The Toxicology Special Interest Group holds quarterly free webinars. Our upcoming webinars are on the following topics:

Tuesday 11th December 2018 - Thomas Steger-Hartmann on Big Data

Tuesday 19th February 2019 - Professor Malcolm Macleod on Data Quality and Pre-Clinical Research

Tuesday 16th April 2019 - TBC

Tuesday 18th June 2019 - TBC

All take place at 14:00 UK time. Full details will be released closer to each webinar, or contact Gareth Thomas (gareth.thomas@envigo.com) to be kept up to date on all ToxSIG activities.

New Emerging Topics around Estimands and ICH Addendum

Tuesday 29th January 2019, IQVIA, Reading, UK

The draft ICH E9 addendum on estimands and sensitivity analysis was released for public consultation at the end of August 2017 and more than 1200 comments were submitted. All stakeholders are gaining the necessary experience and familiarity with estimands along with the associated challenges and methodologies. The language and thinking behind causal inference is well suited to this area. This one day meeting aims to share and discuss new emerging topics around estimands and the ICH addendum, including:

- Sharing the feedback from the public consultation on the draft ICH E9 addendum
- Exploring the estimand concept within health technology assessments
- Describing how casual inference fits into the area of estimands
- Presenting case studies illustrating the implementation of the estimand framework and the use of causal inference methodology

Click here for further information and to register.

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Job Opportunities

Opportunities exist for **Biostatistician Project Leaders – Early Phases, and Medical Affairs**, and for Principal and Senior Statisticians, and Principal and Senior Modelling and Simulation Statisticians.

For all current recruitment adverts and more information on how to submit recruitment adverts, please visit the EFSPI website: Job postings. If you are currently seeking to hire a statistician and wish to post a job advert, EFSPI are offering one free advert for every 3 adverts posted on the website.

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The World of Statistics

The World of Statistics is comprised of 2, 199 organisations across the globe. Participating organizations in The World of Statistics include national and international professional statistical societies, colleges and universities, primary and secondary schools, businesses, government statistical agencies, and research institutes. You can view the current participant and country lists involved in the World of Statistics by going to The World of Statistics website.

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Chrissie Fletcher
EFSPI Communications Officer