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

EFSPI Newsletter September 2017

In this newsletter:

Regulatory

The ICH E9 draft addendum [ICH E9(R1)] has been released for public consultation, click here to access the draft addendum. Comments are due by the 28th February 2018. Alan Phillips along with colleagues from the regulatory committee will consolidate comments. Please send comments on the draft addendum using the comments template on the EMA website (see link above) to Alan Phillips (Alan.Phillips@iconplc.com) by the **30th November 2017**. Statisticians are encouraged to distribute the draft addendum to cross-functional stakeholders, in particular clinicians, and to collate comments within their companies/institutions prior to submitting them to Alan.

The EU members of the ICH E9 Working Group (Rob Hemmings, Frank Petavy, Chrissie Fletcher and Frank Bretz) will hold a webinar to present the E9 addendum on **Monday 30th October at 1.30-3.30pm BST.** Please block this date/time in your diaries and inform other cross-functional colleagues within your companies/institutions. Further details about registering for the webinar will follow by mid-October. In addition, a workshop to discuss the addendum is being planned by the regulatory committee to take place in mid-January 2018. Further details about the workshop will be available in the next newsletter.

The second **EFSPI regulatory statistics workshop** will take place on the **5th-6th October 2017** in Basal, Switzerland. This workshop will be dedicated to opportunities and challenges of statistical topics between regulators, academics and industry with dedicated time for interaction and discussion. Click here to view the agenda and to register click here. Specific focus areas include

multiplicity, estimands, predictive biomarkers/companion diagnostics and early development. Development issues in a range of disease areas will be discussed in addition to a contributed short topics session. Materials from the first workshop can be found here.

The EFSPI-PSI regulatory committee will meet with EMA's BSWP in October and with the MHRA statisticians in November. Currently we are working on the discussion points for the agendas. Please send your question or topic to Christoph Gerlinger (Christoph.Gerlinger@bayer.com) or to Anna Berglind (Anna.Berglind@astrazeneca.com).

Reminder: The EMA published the **Draft reflection paper on statistical methodology for the comparative assessment of quality attributes in drug development** providing current regulatory considerations regarding statistical aspects for the comparative assessment of quality attributes in the settings of pre- and post-manufacturing change, biosimilar development as well as generics development. **Bruno Boulanger (Bruno.Boulanger@arlenda.com)** is collating comments on behalf of EFSPI, so please send any comments you have on the reflection paper to Bruno by **15th January 2018.**

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Scientific

A 1-day meeting on **Oncology and Survival Analyses** will take place on **Friday 17th of November** at BMS in Brussels. Survival analysis methods, or 'Time to event' methods originally developed to analyse trial endpoints in oncology are now used in many other indications. At this meeting, you will hear about recent advances in survival analysis methods. Registration is open and more information can be found on our website.

A 1-day EFSPI/PSI **HTA scientific meeting** will take place on **Tuesday 28th November** hosted by **MSD** in **London, UK**. The meeting will provide an update on latest trends in HTA, including: the Real-World Evidence Navigator tool created by the IMI GetReal project; the EUnetHTA Joint Action 3 initiative and methodology being researched; introduction to value-based frameworks and estimands in HTA. Patient perspectives in HTA will be discussed including how to involve patients in HTA. HTA related methodological considerations will be highlighted including approaches to handle treatment switching in HTA. Industry HTA case studies will also be presented. Registration for the meeting will open from September onwards via the PSI website.

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EFSPI Statistics Leaders Meeting

This year 39 people from 10 different countries representing 31 different pharmaceutical companies and CROs attended the 2017 EFSPI Statistical Leaders Meeting, held in Ludwigshafen, Germany, on July 4, kindly hosted by AbbVie. The 8th meeting saw a record number of participants and companies. Topics on the agenda included: Statistics in Decision making, Interaction with Academia, Patented trial designs, and Data Science.

A high level summary of the meeting is provided below, and you can click here for all the material from the 2017 meeting, including a more detailed meeting report.

Meeting Delegates:

In case you are not familiar yet, the purpose of the EFSPI Statistics Leaders meeting is to bring together leaders and senior managers in statistics groups from the EU pharmaceutical industry to network and discuss strategies to help EFSPI set its strategic objectives for the future.

After a warm welcome to all participants by Stefan Driessen, Chair of the Statistical Leaders forum, the first session on **Statistical Decision making**, a follow-up on last year's meeting, was presented by Maylis Coste and Sylvain Nicholas. Several used cases were presented and discussed. The discussion in the group led to a consensus that this topic is of strategic importance and a new SIG would better drive further activities, in particular allowing business cases to be shared and to discuss methodological approaches.

Next Hans-Ulrich Burger kicked of an interactive discussion on the status and desired strategic direction of **Interaction with Academia**. Several questions were asked to the group (for example, do we in Industry want a closer relationship with academia? Do we know what academia want out of increased collaborations?, etc.). From the discussions key themes emerged including a deeper interaction is important if Industry want to be successful. In addition, Industry should put more effort in understanding the needs from Academia and a working group led by EFSPI might be a useful approach to develop stronger relationships (similar to the group who interact with Regulators).

A new item discussed in this year's meeting led by the host Daniele Compagnone from AbbVie, was the interesting topic of **Patented Trial Design**. One example was given (patent existing in US) and discussed in-depth. The Forum deemed such barriers to the use of patented trial design problematic and against the principle of scientific freedom. However, as no other examples are known, the one discussed may be an "outlier".

The afternoon was largely spent on the by now traditional interactive workshop in this meeting on a special topic. This year it was **Data Science**. Data Science is an emerging, multi-functional area, that is on many people's radar (e.g. in academia, companies, governments) with the ongoing digitalization in healthcare. An excellent introduction to the topic was given by James Weatherall together with Andy Garrett. The results of a survey performed prior to the Statistical Leaders Forum were presented indicating a high level of engagement and interest in this area. The survey suggests there is variability in the maturity and number of activities in Data Science being pursued amongst the companies who participated in the survey. Nevertheless, a general trend was interest to monitor how data science continues to mature with increasing focus and activities in areas such as Big Data,

and IoT (Internet of Things). The four break-out groups came back with numerous ideas on how to proceed in the area and the statisticians role in it (see summary on our website, including link to 'get started" paper by the RSS Data Science section). The consensus was that Data Science is an area that will continue to grow and increase in importance, and statisticians and EFSPI should embrace it, in particular by engaging and promoting statistical rigor. Definitely, this topic will need follow-up by the Statistical Leaders group.

The meeting closed with discussing EFSPI's priorities for the coming years. The directions of the last years with more outgoing communication and interaction were re-emphasized (regulators, academia, health organizations) and focus areas such as Data Science were brought up to form part of the strategic direction of EFSPI.

Any one more interested in the EFSPI Statistics Leaders meeting, please contact Stefan Driessen (stefan.driessen@abbott.com).

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Special Interest Groups

The opening session for new SIG on **Decision-making support** will take place in mid-October. Anyone wishing to participate in this opening session can email Maylis Coste maylis.coste@servier.com and Sylvain Nicolas Sylvain.Nicolas@sanofi.com.

The purpose of the **Decision-making support SIG** is to share and promote quantitative tools to evaluate, compare and optimize drug development plans and business strategies in the pharmaceutical industry, in terms of success and/or risk.

The SIGs main aims are:

- To share (anonymised) cases studies of how quantitative decision-making methods have been used within pharmaceutical companies, including but not limited to statistical methodologies for Go/No-Go decisions (probability of success, …), comparisons of development plans, optimal allocation of resources (operations, number of patients, trial duration, …) and portfolio value assessment
- To discuss and make recommendations on existing methodologies in terms of approach and interpretation
- To develop new methodologies or practices where needed
- To promote the role of the statistician in supporting decision-making in pharmaceutical companies and/or other stakeholders
- To propose trainings, public meetings or publications to share methods and experience

A series of articles relating to **Benefit assessment in Germany: requirements & challenges** have been written by colleagues at Pfizer, Germany and shared with the **HTA SIG**. Topics covered include: Introduction; Impact of label on benefit assessment; Health related quality of life; Validation of surrogates; Handling of missing values; Indirect comparisons and network meta-analyses; and Health economic evaluation. The articles have been collated into a report and this is available to read in the HTA SIG area on the PSI Website. Click here to view the report. Any comments or questions can be sent to Friedhelm Leverkus (Friedhelm.Leverkus@Pfizer.com).

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Country News

PSI (UK)

Abstract Submission OPEN for the PSI Conference 2018 PSI are pleased to announce that the count down to the PSI Conference 2018 has now begun! The conference will be held in **Amsterdam** on the **3 - 6th June 2018** and the theme is Breaking Boundaries in Drug Development. We are now taking submissions for 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; Machine Learning, Pre-clinical, Real World Data, Safety Data, Genomics, Bayesian and many more. You can see the full list and download the abstract template here! The deadline for oral abstract submissions is **24th November 2017**. We hope to see you in Amsterdam!

PSI Toxicology Special Interest Group The Toxicology SIG is a small group of statisticians who mainly work, or have an interest in, toxicology data. We also expand our discussions to include nearly all areas of pre-clinical development, including Safety Assessment, Safety Pharmacology, Genetic Toxicology, Carcinogenicity, use of historical control data and general assay supporting, including Anti-Drug Antibody assays. We are in the process of putting together a regular series of Webinars on such topics, and organising our next workshop for **April 2018** following the success of our last workshop in March 2017. If you work in these areas, or know someone who is, please get in touch so that we can share our thoughts with you. We welcome anyone across the globe to get involved in our work. For more information and to be added to our email distribution list, please contact gareth.thomas@envigo.com.

Do you have colleagues who look baffled when you talk about statistics? Statistics Fundamentals for Clinical Trials for Non-Statisticians (or 'How to speak stats in a day!'), **14th November, 2017, Reading, UK**. Presented by Gemma Hodgson this is a 1-day course, aimed to introduce statistics to people who work on Clinical Trials, but who are not Statisticians. No previous knowledge of Statistics is assumed as we start right at the beginning with the basics. For more information on the agenda and how to register please click here.

One-day meeting: Use of Extrapolation methods 22nd November, 2017, GSK, Stevenage, UK. Use of extrapolation techniques is playing an increasingly important role in the development of new medicines particularly with regard to special populations such as paediatrics and rare diseases. This meeting will include speakers from industry, academia and regulatory (including Rob Hemmings from MHRA). Please look out for an eNews update on this meeting in the autumn with full speaker details.

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The World of Statistics

The World of Statistics is comprised of 2, 196 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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And finally.....

To add your e-mail address to the EFSPI mailing list, click on "Sign up to our newsletter" on the homepage of the EFSPI website.

To view previous newsletters please see the EFSPI website in the "News" area.

For 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 three adverts posted on the website.

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Chrissie Fletcher
EFSPI Communications Officer