European Federation of Statisticians in the Pharmaceutical Industry

Representing Statistical Associations in Europe

EFSPI Newsletter May 2017

In this newsletter:

Regulatory

The regulatory committee helped organize the Regulatory Hot topics session at the PSI conference in London May 17. The session was well received with four interesting talks from four fantastic speakers: Professor Armin Koch talked about the draft ICH E17 guidance of multi-regional clinical trials, and interesting methodological issues when one trial form the basis for regulatory decision making in various regulatory regions; Dr Anja Schiel talked about the use of parametric modelling of time to event data in the field of health technology assessments, contrasting to the current thinking in the regulatory setting; Dr Nick Latimer discussed treatment switching in randomized controlled clinical trials, especially around methods for adjusting, the performance of each method and acceptance of methods in health technology assessment; and Dr Lisa LaVange who described the statistics organization at CDER, and the thinking behind statistical policy and guidance at the FDA, where she also share details on comments received on their recent guidance, Multiple endpoints in Clinical Trials (where EFSPI collated comments) , as well as the motivation for issuing a new draft guidance on adaptive designs in lieu of finalizing the 2010 draft guidance.

Data sharing, data transparency and data privacy are areas that continue to evolve. For example, EMA Policy 70 is now effective with proactive publication of submitted clinical overviews and study reports. It is becoming more common for clinical trial data to be used to support scientific questions beyond the objectives of the original study. EFSPI/PSI are holding a webinar on data privacy and data sharing on Wednesday 21st June 2.30-4.30pm (UK time). The aim of the webinar is to provide an outline to pharmaceutical statisticians of key issues and concepts everyone dealing with and sharing patient level data should be aware of. To see the full event flyer and register (free), click here.

The PSI/EFPSI Expert Working Party on the Confidentiality of Interim Results in clinical trials had their inaugural meeting on 11th May 2017 at the PPD offices in Cambridge (see picture below). This Working Party was formed to develop an industry position on this topic to share with the Regulators later this year, and the Working Party will report back to the PSI Membership once this position is developed. If you are interested in getting involved with this Working Party, or have a case study regarding challenges with the confidentiality of interim results to share with us, please contact Jürgen Hummel (co-chair) at Jurgen.Hummel@ppdi.com.

(L to R): Robin Mukherjee (AstraZeneca), Richard Kay (Independent Consultant), Munya Dimairo (University of Sheffield), Erika Daly (ICON), Jürgen Hummel (PPD), Tony Sabin (AstraZeneca), and Adam Crisp (GSK). [Absent are: Simon Day (Independent Consultant), Stuart Pocock (LSHTM), Hendrik Schmidt (Boehringer Ingelheim), and Jorgen Seldrup (Independent Consultant).]

A friendly reminder of a couple of draft regulatory guidances previously released for public consultation for which the regulatory committee are collating comments:

- The EMA published the **Draft guideline on multiplicity issues in clinical trials** intended to provide guidance on how to deal with multiple comparison and control of type I error in the planning and statistical analysis of clinical trials. From the points to consider document published in 2002, aspects related with multiplicity issues in safety, drug-response studies, secondary endpoints, subgroup analysis and estimation were added or updated, and statistical terms were clarified. **Erika Daly (erika.daly@iconplc.com)** is collating comments on behalf of EFSPI. If you would like to comment, please review and send any comments Erika using the attached form by Friday 9th June 2017.

- 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 February 28 2018.

- In addition, please note that the FDA has published the Draft Guidance on **Delayed Graft Function in Kidney Transplant: Developing Drugs for Prevention.** The purpose of this guidance is to assist sponsors in the clinical development of drugs for the prevention of delayed graft function (DGF) in kidney transplantation and although not a stat specific guideline it discusses

statistical matters. Electronic comments can be submitted at https://www.regulations.gov before June 20, 2017.

The 2nd **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. Registration will open on the **1st June 2017**. Specific focus areas include estimands, confidentiality of interim results, subgroups, transparency, and multiplicity. Materials from the first workshop can be found here.

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Scientific

The Scientific Committee is planning for three 1-day scientific meetings in 2017:

The first one will be a joint EFSPI/PSDM meeting on "**Statistical Aspects of Safety Data in Clinical Trials**", to take place on Friday **23 June** in the Netherlands hosted by Astellas. This meeting will focus on three areas, analyses of Adverse Events, Data Monitoring Committees and the presentation of safety data. Information on confirmed speakers and topics can be found here. Registration for this event is open – use this link to register.

The second meeting will be on **Oncology and Survival Analyses**, planned for November in Brussels, and a third joint meeting together with the PSI on **HTA** is planned for the fourth quarter of 2017. More information will follow.

Asterix

EFSPI is one of the partners in the FP-7 projects, called Asterix, on new innovative methodology for rare diseases. This consortium is reaching its final stage, and as a result this consortium is organising the **Asterix End Symposium** on **September 18 and 19** in **Zaandam** (the Netherlands) **** SAVE THE DATE ****. At the symposium key topics such as novel novel approaches to randomization, sequential designs, multiple endpoints, meta-analyses and Goals Attainment Scaling by using examples and explanations in common, non-technical language will be discussed. Special attention is dedicated to patient involvement in general and the Patient Think Tank.

More information can be found on the Asterix website and on the flyer.

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EFSPI Statistics Leaders Meeting

For the 8th time EFSPI will have its EU Statistics Leaders Forum in 2017 on the 4th July in Ludwigshafen, Germany, hosted by AbbVie. This year the meeting will discuss the role of statistics in decision making, increasing collaboration with Academia, the rise of "Data Science" and revisit the workshop outcomes of 2016 to further define strategic directions for EFSPI, the leader's forum and

for the statisticians working in the pharmaceutical industry in general. We expect over 30 participants again this year and look forward to a very good and interactive meeting. Note that this meeting is on personal invitation only. If you want to know more about it, please contact Stefan Driessen **(stefan.driessen@abbott.com).**

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Country News

BBS (Switzerland) /PSI (UK)

One-day Event on Cancer Immunotherapy 15th June 2017 Roche, Basel. Immunotherapy treatments use parts of a person's immune system to fight disease. In the recent past, immunotherapy has become an important part of treating some types of cancer e.g. melanoma, NSCLC. Studies of these treatments have resulted in a number of observations that have implications for the statistician e.g. delayed treatment effects, long term survivors etc. This joint Basel Biometric Society / PSI one day meeting will present an overview of the science and potential statistical challenges across a range of topics covering early and late phases of development, regulatory and health technology assessments. The speakers from Academia, Regulatory Bodies, and Pharma will share their thoughts, ideas and experiences, including case studies. There will be plenty of time for questions and interactions with colleagues. Further details can be found here.

BIAS (Italy)

The 1st "Fundamentals of R for statisticians" course was held on April 3rd in Milan and it was sold out. Professor Dario Gregori and Corrado Lanera (University of Padua) introduced the basic concepts of R that is becoming very popular and extensively used also in the pharmaceutical industry as it represents a less expansive and high quality alternative to SAS.

The third edition of the "SAS for data managers" course was held on May 12th in Milan. Glauco Cappellini (Quintiles) and Francesco Maccari (Parexel), senior statistical programmers, presented the essentials of SAS programming with some tips and tricks on the management of data using SAS.

The next national BIAS congress will be organized in Parma on September 28th and 29th. This edition will be focused on "***Biostatistics beyond clinical trials: epidemiology, pharmacoeconomics, personalized medicine, machine learning and other hot topics"***. The Epidemiology session will involve Italian experts from both the Academia and the Industry, and it will aim to depict how observational and real world studies can play a strategic role in the development of new drugs and their importance in the surveillance of marketed products. A panel of experts leaded by Eva Pagano (AOU Città della Salute e della Scienza, Torino) will talk about pharmacoeconomy and its growing importance in the decision making process of the health authorities. Presentations on machine-learning (Dario Gregori, University of Padua), personalized medicine and estimands (Mouna Akacha, Novartis Pharma AG) complete the scientific program of the Congress.

PSI (UK)

PSI Webinar - Patient Engagement in Clinical Trials 13th June 14:30 - 15:30 UK Time. Patients have

long been an important part of clinical drug development - without them, there would be no new medicines. Recently, there has been a fundamental shift in their involvement in the drug development process. Today, patients are highly active in engaging in discussions about their disease, what they look for in new treatments, and how clinical trials are designed and conducted. Following on from the successful and thought-provoking presentation at last year's PSI conference by Paul Wicks from PatientsLikeMe, this webinar will continue to explore the ways in which patients are influencing the design of new clinical trials. Please click here for more information and to register (free).

Estimating Sample Sizes in Clinical Trials Training Course 28 - 29 Jun, 2017, London UK. The course describes calculations for sample size estimation in the design of clinical trials. It will be highlighted how the objectives of a clinical trial will impact on sample size calculations. The course is a practical course and all methods will be illustrated with examples and case studies. Please click here for more information and to register.

PSI One Day Meeting: Career Young Statisticians 19th June 2017 QuintilesIMS, Reading, UK. The aim of this meeting is to provide a relaxed environment for career young statisticians where they can present and discuss various statistical topics and interact/network with other statisticians in similar positions to themselves. In addition to presentations from fellow statisticians there will be a soft skills workshop on explaining statistics to non-statisticians and ample opportunity to connect with colleagues across the industry. Please click here for more information and to register.

The Introduction to Industry Training Course needs you! NEXT COURSE STARTS OCTOBER 2017: £1050+VAT. This course aims to describe the drug development process from research right through to research, toxicology, data management & role of the CRO, clinical trials, product development & manufacture and marketing. Please click here for more information and to register.

We want your brains! - Volunteering opportunity to share your knowledge and experience The PSI External Affairs Committee and Cancer Research UK's (CR UK) Centre for Drug Development (CDD) are looking for individuals who would be willing to volunteer their time and knowledge for a variety of opportunities. These include:

- Being part of a team helping to create and run a 1 day workshop for CDD staff on key statistical considerations for early phase development oncology trials.
- Or as an individual volunteering 1 or 2 days to:
    - contribute to the review of CDD's outline and full protocol templates to refine stats and analysis sections
    - advise and input into the development of outline & full protocols for upcoming trials
    - participate as a statistical reviewer in a session of the independent CDD protocol review committee

If you are interested in finding out more about supporting CRUK in this way, please get in touch with Rebecca Sudlow (rebecca.sudlow@roche.com)

ITIT and Training Committee are seeking new members! The Introduction to Industry Training (ITIT) and Training Committee are seeking new committee members. We are looking for individuals who are enthusiastic, good organisers, eager to contribute and play a key role in the continued success of PSI training courses. The ITIT committee organise a series of six two day training courses for PSI members new to industry. The training committee organise several one and two day meetings for statisticians at all career levels. For more information please contact Alex Godwood for ITIT (godwooda@medimmune.com) or Mary Elliott for training (maelliot@amgen.com)

Pharmaceutical Statistics May/June is now available on the Wiley website. This issue has five papers on a diverse range of topics including QTc prolongation, use of biomarker analysis, response adaptive designs and Bayesian sequential designs.

View the replay of the recent webinar by the Benefit-Risk Special Interest Group This webinar is now online and can be viewed here. You can find the abstracts of the presentations with bios of the speakers here.

SFdS (France)

The **International Meeting Statistical Methods in Biopharmacy** will be held on **the 14-15th September 2017** with the theme "***The future of Biostatistics in an emerging world of data sciences***". Key themes for the meeting include: regulatory statistics and beyond, statistical inference of biostatistics of the 21st Century, successful marriage between bioinformatics and biostatistics, and recurrent event analyses. More details to register will follow in future newsletters.

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Other news/events

EFPIA Clinical Trial Design Taskforce

The Clinical Development Expert Group (CDEG) are forming a small taskforce to map out ongoing and planned initiatives in relation to novel and innovative trial designs. Novel trial designs has been recently identified as a priority area for future IMI initiatives as well as a key focus area for EFPIA's work in digital health. The taskforce will be led by Chrissie Fletcher (Amgen and CDEG co-chair). Please email Chrissie (fletcher@amgen.com) for further information.

International Clinical Trials Day

Industry, regulatory and academic institutions across the world celebrated International Clinical Trials Day on the 20th May 2017. Statisticians are recognised as having a critical role in the design, conduct, analysis and reporting of clinical trials. Click here to read EFPIA's views on why this is an important occasion for the Pharmaceutical Industry to celebrate and acknowledge this day.

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The World of Statistics

The World of Statistics is comprised of 2, 188 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 3 adverts posted on the website.

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Chrissie Fletcher
EFSPI Communications Officer