European Federation of Statisticians in the Pharmaceutical Industry

Representing Statistical Associations in Europe

EFSPI Newsletter September 2016

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Regulatory

The **1st EFSPI Regulatory Statistics Workshop** took place on ***September 12th and 13th*** in **Basel, Switzerland**. Approximately 160 delegates attended the workshop. The key themes in the workshop included: estimands; extrapolation; benefit-risk assessments; and statistics in quality attributes. There was also a session allowing individuals from the audience to present on short topics followed by a panel discussion. The materials from the workshop will be available shortly on the EFSPI website.

The EMA have released a new **Draft ICH guideline E17 on general principles for planning and design of multi-regional clinical trials.** The purpose of this document is to outline general principles for the planning and design of multiregional clinical trials with the aim of increasing their acceptability in global regulatory submissions. Aaron Dane (aarondane@danestat.com) is collating comments. If you would like to provide comments on behalf of your organisation please contact Aaron. Comments are due by December 16.

A **cross-functional data transparency workshop** led by EFSPI and the data transparency working group took place on the **27th September** at the **RSS** in **London**. Approximately 70 delegates from Industry, vendors, academics, EMA and a patient representative attended the workshop. The aims of the workshop were to:

- Identify the challenges of maximising data utility whilst minimising data privacy
- Share methods and approaches to anonymise clinical documents/data
- Share methods and approaches for estimating the risk of re-identification
- Understand the practical issues and considerations in anonymising clinical documents/data
- Solicit views/perspectives of linking anonymising patient level data with anonymising clinical documents
- Understand how anonymised clinical data is/could be used by stakeholders

A number of breakout sessions were held where delegates addressed a series of questions in round table discussions. There was useful feedback provided from all stakeholders with many sponsor companies sharing recent case studies. A report summarizing the feedback will be shared in a future newsletter.

On the **30th September** the EFSPI/PSI regulatory committee met with **EMA's Biostatistics Working Party (BSWP).** The key themes discussed during the meeting included: multiplicity issues in clinical trials; statistical methods in quality attributes; controlling type 1 error in bioequivalence studies; treatment switching in oncology; data monitoring committees; confidentiality of results from interim analyses; estimands, and role of non-randomised evidence in regulatory decision making. A summary of the discussions will be shared in a future newsletter.

EFSPI has been asked by EMA to help implementing a set of ISO standards relating to **S**ubstances, **P**roducts, **O**rganisations and **R**eferentials (SPOR) in support of regulatory activities. Please refer to EMA's ISO IDMP web page for more information: click here. If you have any questions please contact Christoph.gerlinger@bayer.com.

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Scientific

Together with the Special Interest Group on Modelling & Simulation, EFSPI is organising a **Webinar on Best Practice in Modelling and Simulation** on **Tuesday October 4th 15:00-16:30 CET**. This webinar will cover recent proposed Best Practice for M&S. Speakers will discuss how M&S can be integrated into the drug development process from discovery to post-marketing, and how M&S practitioners can keep to the appropriate best practice, when applications and impact of M&S vary so much. Speakers include Michael O'Kelly, Chris Jennison, Alun Bedding, Scott Marshall and Tom Parke. These presentations are based on the presentations given at the PSI conference in Berlin in May 2016 and also include new material. Click here to see more details for dialling into the webinar.

A 1-day scientific event on **Evidence Synthesis** will take place on the **22nd November, Brussels** (hosted by BMS). This meeting will discuss methodological considerations relating to synthesizing evidence to support drug development and market access activities. Topics to be discussed include: how to use evidence from network meta-analyses (NMA) to inform clinical trial design; NMA in an ANOVA framework; conducting frequentist NMA in R; conducting NMA using individual

patient level data; and how to compare treatments when the network of evidence is disconnected. Case studies will also be presented. Click here to find out more details and register for this meeting.

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2016 EFSPI Statistics Leaders Meeting

This year 34 people from 8 different countries representing 29 different pharmaceutical companies and CROs attended the 2016 EFSPI Statistical Leaders Meeting. This is a record number of participants and companies in this 7th meeting in a row, this year held in Paris on July 5, kindly hosted by Sanofi.

The purpose of the EFSPI Statistics Leaders meeting is to bring together leaders and managers in statistics groups from the EU pharmaceutical industry to network and discuss strategies to help EFSPI set its strategic objectives for the future. A high level summary will be given here, and you can click here for all the material from the meeting.

The first session on regulatory and public interactions made clear that there is broad EU participation in the regulatory committee and engagement in key regulatory forums with working groups in place on topics as data transparency, estimands, and statistics in quality. Further there are good links with EFPIA. One message from the group was that in the near future more attention should also be given to the communication on consortiums as IMI. In this year's meeting three SIGs were presenting. The relatively new SIG Small Populations has kicked off with still small but active group and expressed that more members can join and a need for case studies. The SIG Modelling & Simulation updated the group on the release of their Best Practice guidance published in Pharmaceutical Statistics and also the SIG will hold a seminar on it. The group was very much invited to use it and give the SIG feedback. The new SIG AIMS (Application and Implementation of Methodologies in Statistics) presented its charter and through a questionnaire of 25 possible topics queried the Stats Leaders group for prioritization and got very helpful feedback to further steer their activities. Especially, the validation of R and graphical application was deemed of high interest.

An interesting discussion was on the topic of Precision Medicine and Biomarkers. How this is supported very much differs between companies (by special expert groups, by early development statisticians, or typical phase2/3 statistician). Not so many success stories are available and that hampers further resourcing this area. Another item mentioned was that collaboration is needed and statisticians with biomarker expertise but also generalists with regulatory requirements in mind. Also the handling of (large) data sets and its validation is important.

In the afternoon an interactive workshop was held to discuss the present and future of R&D in drug development and how statistical departments and statisticians may evolve relative to skills sets, expertise, roles and responsibilities. Main items to develop or improve in terms of new and future activities is to take responsibility for evidence generation, be influential, but also be open to change. Operational feasibility (play with data attitude) is key to develop innovation and this should best occur in a collaborative, multi-disciplinary setting for cross-fertilization but still bring to the discussion the role of chance. On areas of influence and organization items to be developed or improved were to increase biostatistics activities outside of traditional phase 2-3 studies, to increase statistical group capacity through training and make use of a more network oriented organization to increase influence. Further areas like risk evaluation and probability of success were brought up as possibly helpful to increase influence.

The meeting was closed with suggestions for topics for the next meeting in 2017 as well as about its organization as the Chair, Stefan Driessen, has indicated to pass on the chair of organizing these successful series of Statistical Leaders Meetings to a successor. Anyone interested in chairing the EFSPI Statistics Leaders meeting please email Stefan Driessen (stefan.driessen@abbott.com).

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Other Events

APF (Germany)

APF is organizing a **German Stats Leader Meeting** hosted by **Parexel** in **Berlin** on the **28th October 2016**.

APF is organizing a Workshop hosted by **Parexel** in **Berlin** on the **25th November 2016** about **Estimands and Prediction of Disease Progression**.

BIAS (Italy)

The upcoming BIAS seminar, "**CDISC Italian User Network Day: data standards and their application**", will revolve around the Traceability and Validation of CDISC datasets and it will be held on **October 21st 2016** in **Milan**, in collaboration with CDISC and with the support of SAS Italy.

Two CDISC Vice Presidents will provide an update on CDISC news, particularly on SHARE and CFAST, and on various therapeutic areas; in addition, a number of heterogeneous speakers will share practical experiences concerning issues in CDISC. At the end of the meeting, there will be a Q&A session. Participants are invited to contact the BIAS Committee (info@biostatistici.it) or Angelo Tinazzi (angelo.tinazzi@cytel.com), as the contact point for the ''Italian CDISC User Network'', to send questions beforehand. The meeting will be in Italian, but some speeches will be given in English. The final agenda of the seminar will be soon available on the website www.biostatistici.it.

FMS (Sweden)

Joint DSBS/FMS Meeting - Every second year the Swedish and Danish societies for biopharmaceutical/medical statistics, FMS and DSBS, arrange a joint meeting and this year's meeting will take place in **Malmö, November 1st 2016**. The theme of the meeting will be ***Statistical analysis of risks and safety data*** and the program will consist of invited speaker sessions and contributing speaker sessions. The planning of the meeting is currently in progress and two examples of topics covered are extreme value modelling of safety data from clinical trials and survival analysis methods in the assessment of safety data.

PSI (UK)

Sample Size Re-estimation - dealing with those known unknowns! 02 Nov 2016, Royal Statistical Society, Errol Street, London. Determining the appropriate sample size is an important part of good clinical trial design. When there is uncertainty about some of the design parameters (e.g. variability, control rate, model parameters), it can be challenging to determine up front the number of subjects required for robust evaluation of the study objectives. The aim of this PSI one day meeting is to present an overview of available methods for sample size re-estimation together with several case studies where such methods have been used in late phase clinical trials. There will be plenty of opportunity for discussion and interaction with other statisticians working in this area. Click here to see more!

Introduction to Simulation 8-9 November, Heathrow, London. The aim of this 2 day course is to provide participants an understanding of simulation techniques. The course, presented by Les Hudson, will include methods for generating samples of univariate, correlated bivariate and multi-variate normal data. Example code for the methods discussed will be provided in both SAS and R and a variety of practical examples will be presented. Click here to see more!

Dose Finding in Drug Development using MCP-Mod 01 - 02 March 2017, Heathrow, London. This two day course will introduce and discuss methods for Phase II dose finding studies, including a review of basic multiple comparisons and modelling methods, as traditionally used in these studies. A unified strategy for designing and analysing dose finding trials denoted MCP-Mod, combining multiple comparisons and modelling, will be the focus of the course. MCP-Mod was the first statistical methodology to receive CHMP Qualification Opinion (2014) and was recently recognised by the FDA under the Fit-for-Purpose Initiative (2016). The course ends with a review of regulatory considerations. Click here to see more!

PSI Conference 2017, Abstract Submission OPEN! In 2017 we are offering a 10% discount on the full 3 day conference rate for anyone selected for a contributed presentation. We welcome abstracts on any statistical topics and have a full list online of those areas of particular interest. For full details on how to submit an abstract please visit the PSI 2017 conference website. Please note the oral presentation deadline is 18th November 2016 and the poster abstract deadline is 27th February 2017.

SSL (Finland)

SSL is organizing a webinar on machine learning on the 27th October. The webinar will be held in Finnish.

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Job Opportunities

Opportunities exist for TA expert statistician in Oncology, TA expert statistician in metabolism, Senior Manager Statistical Programming and multiple positions in quantitative sciences. 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 2,355 organizations across the globe. You can view the current participant and country lists involved in the World of Statistics by going to The World of Statistics website. To see the events and activities planned for 2016, click here.

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Get the latest news and updates about EFSPI by following us on Twitter at @EFSPItweet. Also, when you use Twitter to spread the word about EFSPI, be sure to use the hashtag "#EFSPI". You also can follow developments in EFSPI via LinkedIn.

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And finally.....

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

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.

Enjoy this quote (courtesy of www.goodreads.com)

> "We encounter regression to the mean almost every day of our lives. We should try to anticipate it, recognize it, and not be fooled by it."
> — Gary Smith

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