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

EFSPI Newsletter December 2015

In this newsletter:

Regulatory

EMA has published the draft guidance on post approval efficacy studies (see http://www.ema.europa.eu/docs/en_GB/document_library/Scientific_guideline/2015/11/WC500196379.pdf). The expert working to comment is led by Hermann Huss (Hermann-josef.huss@bayer.com). Please contact Hermann if you wish to contribute as additional members of the working group are most welcome. Comments should be sent by the 8th January 2016.

EMA plans a workshop on the extrapolation of adults' safety and efficacy data to the pediatric populations. If you are an expert in this domain and want to contribute please contact Christoph Gerlinger (christoph.gerlinger@bayer.com) by the 15th January 2016.

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Special Interest Groups (SIGs)

This year two new EFSPI/PSI SIGs have been established. In last month's newsletter the SIG Application and Implementation of Methodologies in Statistics (AIMS) was introduced with as contacts: Craig McIlloney (craig.mcilloney@ppdi.com) and Lyn Taylor (TaylorLyn@prahs.com). In this newsletter the new SIG Small Populations is being presented.

SIGs have been very active in 2015. The **SIG Modelling and Simulation** organized a one-day workshop in 2015 and released their Best Practice document (see later in this section). The **SIG**

Benefit-Risk presented at the biometrical colloquium of the IBS (German section) and the UK PSI conference and conducted two webinars with help of PSI/EFSPI. The **SIG Integrated Data Analysis** has a paper accepted for publication on Product Safety Labelling and had a paper published in the Proceedings of the 2016 Joint Statistical Meetings on the Integration of Efficacy Data. The **SIG HTA** presented at the Statistics Leaders Meeting on how statisticians can add value to HTA, and presented at the UK PSI conference on a variety of HTA related topics. A HTA training course is also being developed for Q4 2016. The **SIG Real World Data** has as new chair Maurille Feudjo-Tepie from Amgen (maurille@amgen.com) succeeding George Quartey. Plans for 2016 are being developed with, for example, scientific meetings by the SIGs Benefit-Risk and Medical Devices. We will inform you events through the 2016 Newsletters!

As a reminder, volunteers are always welcome to join the SIGs. It is not necessary to be an expert in the area of interest to be able to join a SIG, rather having a strong interest in the area and being motivated with an eagerness to learn and contribute is highly valuable. To get better insight in what SIGs are doing, please visit the EFSPI website, where a compilation is available of the SIGS as they presented themselves in the EFSPI Newsletters in 2014.

Special Interest Group for Modelling and Simulation presents Best Practice

We sometimes complain about how long it takes regulators to get a new Guideline out. Some EFSPI members can now say with Voltaire “To understand is to forgive all”.

At the EMA-EFPIA Modelling and Simulation Workshop in London in 2011, Rob Hemmings called for a Best Practice document for modelling and simulation. We members of the PSI/EFSPI Modelling and Simulation Special Interest Group (SIG) agreed – we saw the need for such Best Practice in our work. We met with EMA to discuss what they would expect in a Best Practice document. It turned out that for regulators Best Practice means some degree of pre-specification, and the specification should cover assumptions, analyses (including sensitivity analyses) and outputs. There were few surprises in this list! But we SIG members realised that, even in our own work, we have not always covered all these items in a pre-specified plan.

At that 2011 Workshop, EMA had emphasised that from the regulator’s point of view the level of pre-specification could vary depending upon the “importance” of the modelling and simulation in providing evidence for the decision to approve the treatment and the label. Again, this made sense to SIG members. It would be difficult to justify an elaborate pre-specification for a simulation that merely double-checked the formula for a sample size calculation; but detailed pre-specification made sense where, say, PK modelling and simulation was used to seek approval to extend a treatment from adults to paediatric patients.

It is one thing to recognize a good idea, but another to come up with a workable Best Practice document that everyone feels they can use, and that covers all areas of modelling and simulation in the pharmaceutical industry, whether they be of low or high importance. The task was challenging. In addition, statisticians were making efforts outside EFSPI/PSI to agree elements of Best Practice. The EFPIA set up a working group called Model Informed Drug Discovery and Development (MID3). This group has done much information-gathering, and has come up with a list of requirements very close to those of the EMA – see http://www.page-meeting.org/pdf_assets/2935-EFPIA_MID3_PAGE_2014_Final.pdf. However, SIG members found that the proposed Best Practice that we had in mind could complement the work of MID3. The SIG proposals provide a flexible but fairly detailed template that allows the working statistician to create a pre-specification appropriate to the importance of the project. On behalf of SIG members, Michael O’Kelly introduced the document via a poster at the PSI conference this year. Along with O’Kelly, Vlad Anisimov, Chris

Campbell and Sinéad Hamilton are co-authors on behalf of the SIG. PSI/EFSPI members took an active part in getting the document right, though. Chair of the SIG, Chris Campbell, organized a Hackathon in February where SIG members and others PSI/EFSPI members subjected the proposed Best Practice to a practical test set by Professor Chris Jennison (University of Bath) – could the teams specify Chris's modelling and simulation project using the draft Best Practice? The exercise resulted in several improvements to the proposed Best Practice. We now think we have a practical guide to Best Practice for working statisticians who need to use modelling and simulation in their projects. The SIG will use various means to present the Best Practice documents to various groups of interest. The PSI Board has recommended a session based on the Best Practice document at the PSI conference in 2016 in Berlin and to submit the document to a journal. The EFSPI Council has suggested running a webinar on this topic. Meanwhile, the SIG will be happy to send a copy of the proposed Best Practice to those interested – just email mokelly@quintiles.com .

SIG Toxicology

A two-day workshop was organized by the SIG Toxicology in 2015. There were 19 attendees and hot topics discussed included: Analysis of ADA Assays, Analysis of large datasets (including telemetry data), Experimental Design and Benchmark dose approaches.

Most companies have very few statisticians supporting the toxicology area and the Toxicology SIG provides an excellent forum for sharing problems and proposing solutions. Under the Toxicology SIG umbrella seven papers have been published. Our aim is to now progress work on the following topics, have further discussions (via web meetings and/or teleconferences) and publish our proposals/recommendations on each of the following:

- Approaches to combining sexes in analyses
- Anti-Drug Antibody Assay analyses
- Carcinogenicity data and the use of transgenic study data

If you are interested in contributing to one of these areas/publications and/or are interested in joining the Toxicology SIG, please contact Gareth Thomas (gareth.thomas@envigo.com).

EFSPI/PSI are pleased to announce that the new

Small Populations
Special Interest Group

will have its first telecon meeting on **29th January 2016 from 2pm - 3.30 pm** CET, dial in details will soon be made available on the EFSPI website)

The purpose of this SIG is to provide a forum for statisticians working in the Pharmaceutical Industry engaged in the topic of small populations and/or rare diseases.

Among others the aims of this SIG are:

- *To exchange information and share case studies of statistical/methodological challenges faced in the area of small populations,*
- *To collaborate and discuss strategies and methodology being applied in this area of research,*

- *To create visibility on biostatistics activities for small populations, and to promote and highlight opportunities for statisticians to make a positive impact,*
- *To form a working expert group within industry identified by, and interact with the external community, like the FP7 programs (IDEAL, INSPIRE and ASTERIX),*

If you want to know more or are interested to participate, please feel free to contact Egbert Biesheuvel (egbert.biesheuvel@danone.com)

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Scientific

The Scientific Committee is already planning for a few scientific events in 2016.

The first scientific event is planned for the *second half of June2016* on **Biomarkers and Subgroups** and will take place in Leiden, the Netherlands. More information on this event and the other events will follow.

If you have ideas or suggestions for scientific meetings for 2016, please e-mail Egbert Biesheuvel (egbert.biesheuvel@danone.com).

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7th EU Statistical Leaders meeting 2016

Plans are underway for the next EU Statistical Leaders Meeting. The date of the meeting is still to be confirmed but will be in the June-July timeframe. The meeting will be held in France, kindly hosted by Sanofi. Possible themes for the meeting are: modeling in drug development, personalized medicine and biomarkers, risk-based monitoring, data science, leadership skills for statisticians, and statisticians' engagement in regulatory policy and in public-private initiatives.

We are very much looking for people that want to sit in the Organizing Committee and help with the agenda and the set-up of the meeting. If you are interested, please contact Stefan Driessen **(stefan.driessen@abbott.com).**

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Other Events

A calendar of events that each local association is planning for 2016 is in development and will be available on the EFSPI website in early 2016.

BBS (Switzerland)

Disease and Product Registries - This Seminar being held on the 13th January at Novartis, Basel, Switzerland, presents new statistical and epidemiological approaches along with case studies that involve using registry data to improve accuracy in estimating and predicting outcomes in the real-

world setting that will serve to benefit manufacturers, healthcare systems and patients alike. For further information see the EFSPI website.

PSI (UK)

PSI Careers Fair - The Careers and Academic Liaison Committee (CALC) are planning the annual Careers Fair Event on Wednesday 3rd February 2016, to be hosted at the University of Reading. As well as MSc students, the invitation is extended to final year BSc and PhD students with an interest in pharmaceutical statistics and statistical programming.

The **PSI 2016 annual conference** will be taking place on 22nd- 25th May 2016 at the Pullman Hotel, Berlin. The theme of the 2016 Conference is "Promoting Statistical Insight and Collaboration in Drug Development" and registration will open soon. Sessions will include an update on estimands, subgroups, dose exposure modelling, decision criteria, use of biomarkers in translational activities, clinical registries and many more with speakers from industry, academia and regulatory agencies. The deadline for poster abstracts is 29February 2016 - please visit the website for more information. http://www.psiweb.org/events/2016-conference/2016-conference-abstracts. For further information please go to http://psiweb.org/events/2016-conference.

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Job Opportunities

Opportunities exist for a Principal/Senior Principal Biostatistician, a Senior Epidemiologist, and a Senior Quantitative Safety Scientist. 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 2,372 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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Follow us on Twitter and LinkedIn

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…..

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.

Enjoy these festive jokes (courtesy of http://achristmasnew.esy.es/christmas-jokes-for-kids/):

Who hides in the bakery at Christmas?

A mince spy!

Activity village .co.uk © 2000 - 2012

What is the best Christmas present in the world?

A broken drum – you can't beat it!

Activity village .co.uk © 2000 - 2012

Who delivers presents to baby sharks at Christmas?

Santa Jaws!.

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How many presents can Santa fit in an empty sack?

Only one, after that it's not empty any more!

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What carol is heard in the desert?

Camel ye faithful

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What did one snowman say to the other snowman?

Can you smell carrot?

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What do you sing at a snowman's birthday party?

Freeze a jolly good fellow!

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Who gives puppies Christmas presents?

Santa Paws

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EFSPI wishes everyone a Happy Christmas and New Year.

Chrissie Fletcher
EFSPI Communications Officer