European Federation of Statisticians in the Pharmaceutical Industry

Representing Statistical Associations in Europe

EFSPI Newsletter November & December 2021

Merry Christmas and Happy New Year

In this newsletter

End of Year Remarks and Council News

When looking back over the last year, I must admit that I had not expected to do so from my home office again. But in my country that is the reality like many countries in Europe. Will it be the new normal? Who knows. It could be less hypothetical than a year ago (not necessarily referring to estimand framework though). We'll see.

Like in 2020 this corona year forced EFSPI to organize their regular meetings in a virtual manner. A big thank you to all those organizers whereby it must be mentioned that there is a significant increase in technical skills to be observed. Flawless virtual online meetings like the Stats leaders Meeting and the Annual Regulatory Statistics workshop exemplified that, and all of this brought to you by statisticians that do so on top of their regular job. Chapeau! And except for the typical networking and social interactions in face-to-face setting, both meetings brought everything they are renowned for with current and pressing topics. The Statistical Leaders Meeting discussed career

development, data submissions in regulatory reviews, data science and the flexible workplace. In the Regulatory Statistics workshop Decentralized trials, complex innovative designs and real-world data were the main items. Please visit our website if you want to know more about this all. By the way that is an area of focus for the next year; we want to improve the website realizing the needs to access more easily the information that really matters. Further, in this corona period EFSPI has co-sponsored a record number of webinars organized by European Special Interest Groups (eSIGS) and EFSPI working groups. And like last year we have managed to stay financially healthy and fit for the coming years, hopefully without strict corona measures and a chance to meet each other again face-to-face.

In essence EFSPI is there for you and to promote professional standards of statistics and the standing of the statistical profession in matters pertinent to the European pharmaceutical industry. I feel honoured to have been serving EFSPI as President for the last two years and with great pleasure even though it was all virtual and I did miss the direct interactions. My tenure ends but luckily we found a great successor: Justine Rochon!

You may know, Justine has organized and chaired the Statistical Leaders over the last four years and really took it to the next level. So I am confident that she will do so again with this new role. Justine will be introducing herself in the first Newsletter in January in the new year!

As a consequence we will also have a new Chair of the Statistical Leaders Meeting and we are very privileged that Chrissie Fletcher has taken this on starting from 2022. This year Chrissie ended her role as Communications Officer after more than 10 years great service! We are glad to have her back again in the heart of EFSPI operations. So lots of changes and developments and I am sure this will enforce EFSPI as an organization that counts.

Let me end by wishing you all very good holidays, stay safe, and hope to be seeing some of you again in 2022 face-to-face!

Stefan Driessen, EFSPI President

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ESIG News

Regulatory ESIG

Meetings with Regulatory Statisticians

The Regulatory ESIG participated at the 2nd stakeholder meeting of EMA's Biostatistics Working Party on October 29th. Topics were "Raw data submission" where Uli Burger gave one of the talks and "Use of external control" where Christoph Gerlinger gave one of the talks.

On November 17th the ESIG met with the statisticians from the MHRA. Topics were "Decentralised clinical trials", "Use of external controls", "Noninferiority trials", and "Estimands". For both meetings minutes are currently drafted and will be included in a future EFSPI newsletter.

Please contact Christoph Gerlinger in case of questions or if you have suggestions for future meetings with the regulatory statisticians.

Regulatory Guidance / Publications

The Draft FDA Guidance on Benefit-Risk Assessment will be commented by the Benefit-Risk ESIG.

EMA has published an article on "Real-World Evidence in EU Medicines Regulation: Enabling Use and Establishing Value".

Enabling the use of real-world evidence (RWE) and establishing its value for regulatory decision-making on the development, authorisation and supervision of medicines in Europe by 2025: this is the vision of European regulators as outlined in an article from Peter Arlett, Head of Data Analytics and Methods at EMA, Jesper Kjær, Director of Data Analytics Centre at the Danish Medicines Agency, Karl Broich, President of the Federal Institute for Drugs and Medical Devices (BfArM), and Emer Cooke, EMA's Executive Director, published in Clinical Pharmacology & Therapeutics.

The authors emphasise that delivering this vision, anchored in the Network Strategy to 2025, will support the development and use of better medicines for patients.

The creation of the Data Analytics and Real World Interrogation Network (DARWIN EU) will be key to delivering this vision. This EU-wide network will allow to access and analyse healthcare data from across the EU. It will be launched in early 2022 with the establishment of a coordination center to on-board data partners and to drive the conduct of studies requested by medicines regulators and, at a later stage, also requested by other stakeholders.

The article explains plans to establish methods and standards for high-quality collection and use of RWE, in cooperation with stakeholders including patients, healthcare professionals, industry, regulatory and public health agencies, health technology assessment bodies, payers, and academia.

According to the authors, it will be important to advance the debate on the value of RWE compared to randomised clinical trials (RCTs), the gold standard to demonstrate efficacy of a medicine. The vision is that RWE and RCTs should be seen as complementary, each having strengths and weaknesses, with their relative importance depending on the regulatory question. A rigorous and systematic approach to learning from doing will help to identify and establish the use-cases in regulatory decision-making for which RWE will add most value.

In this context, EMA has also contributed to an article that examines when and how RWE was used to support marketing authorisation applications for new products and extensions of indications, submitted to the Agency in 2018 and 2019. The retrospective analysis shows that 40% of initial marketing authorisation applications and 18% of applications for extension of indication for products currently on the market contained RWE. The article describes the characteristics of RWE included in these applications and identifies areas where further research is required.

Both articles aim to support transformation to data-driven regulatory decision-making and to advance patient-centered access to better medicines. They are available through open access:

- Real-World Evidence in EU Medicines Regulation: Enabling Use and Establishing Value
- Marketing Authorization Applications Made to the European Medicines Agency in 2018–2019: What was the Contribution of Real-World Evidence?

For more related content please click here.

A number of draft guidance has been released by FDA relating to real-world data:

FDA Publishes Draft Guidance on Use of EHR and Medical Claims Data to Support Regulatory Decision-Making
The US FDA has published draft guidance on *Real-World Data: Assessing Electronic Health Records and Medical Claims Data To Support Regulatory Decision-Making for Drug and Biological Products*. This draft guidance discusses factors that sponsors and researchers should consider when proposing to use electronic health records (EHRs) or medical claims data in clinical studies to support a regulatory decision on product effectiveness or safety. Covered topics include data source selection, development and validation of definitions for study design elements, and data provenance and quality.

FDA Publishes Draft Guidance on Data Standards for Submissions Containing Real-World Data
The US FDA has published draft guidance on *Data Standards for Drug and Biological Product Submissions Containing Real-World Data*. This draft guidance addresses considerations for the use of data standards currently supported by FDA in applicable drug and biological product submissions containing study data derived from RWD sources. This includes conforming RWD to currently supported FDA study data standards, mapping RWD to study data submission standards, and data transformations

FDA Publishes Draft Guidance on Data Standards for Submissions Containing Real-World Data
The US FDA has published draft guidance on *Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products*. This draft guidance provides further insight into FDA's expectations for sponsors and stakeholders when either proposing to design a registry or using an existing registry to support regulatory decision-making about a drug's effectiveness or safety.

FDA Publishes Draft Guidance on *Considerations for the Use of Real-World Data and Real-World Evidence To Support Regulatory Decision-Making for Drug and Biological Products*
The US FDA has published draft guidance on *Considerations for the Use of Real-World Data and Real-World Evidence To Support Regulatory Decision-Making for Drug and Biological Products*.

One more draft guidance on real-world data is expected in the coming weeks.

Accelerating Adoption of Complex Clinical Trials in Europe and Beyond

Over 400 participants joined an interactive multi-stakeholder workshop on "**Accelerating Adoption of Complex Clinical Trials in Europe and Beyond"** on the 5th-6th October 2021. The objective for increasing understanding of CCTs across stakeholders was achieved with a high level of interaction. Various recommendations were identified to address some of the challenges in designing and conducting CCTs for example: changing from a drug-centric to a systematic patient-centric approach to trial design; seeking consensus on definitions and terminology to facilitate wider understanding; sharing learnings and broaden awareness of CCTs; engaging early with stakeholders and exploring the use of CCTs in confirmatory settings and multi-sponsor studies. Further discussions will continue in 2022 to deliver solutions. See here for the workshop report including the workshop materials.


Christoph Gerlinger **and** ***Jurgen Hummel***
(EFSPI Regulatory Chair) ***(PSI Regulatory Chair)***

VIS ESIG

2021 - a great year for data visualization

2021 marks a year in which the focus and awareness about data visualisations have reached a peak. A community of statisticians have worked on data visualisations for the last couple of years within PSI and have organised various sessions at the last conferences. In 2021 data visualisation became the main topic for the PSI conference. Keynote speakers highlighted the importance of data visualisation in communicating COVID data to the public and many of the concepts the data visualisation community has been talking about for years have been coming to the fore-front of people across the industry.

The VIS SIG (https://www.psiweb.org/sigs-special-interest-groups/visualisation) has been vividly active with the successful monthly Wonderful Wednesday Webinar series (https://www.psiweb.org/sigs-special-interest-groups/visualisation/welcome-to-wonderful-wednesdays) delivering 12 interactive challenges to improve data visualisations. Everybody has access to the recordings on the video-on-demand platform of PSI (https://www.psiweb.org/vod). Furthermore, the visualisations together with discussions and the corresponding code are available for everybody to use on the VIS SIG blog (https://vis-sig.github.io/blog/).

Beyond the 12 Wonderful Wednesday Webinars, the VIS SIG has also organised:

- a conference session
- a scientific event "Generating Insights through Modern Applications of Data Visualisation" spread over 2 half days (https://www.psiweb.org/vod/item/psi-scientific-meeting-generating-insights-through-modern-applications-of-data-visualisation)
- a webinar on the Grammar of Graphics (https://www.psiweb.org/vod/item/psi-vissig-webinar-grammar-of-graphics---theory-to-implementation)
- a webinar on Rapid Insights Into Data (https://www.psiweb.org/vod/item/psi-vissig-webinar-rapid-insights-to-data)

In 2022, the VIS SIG is going to continue to run the Wonderful Wednesday Webinar series.

Register here for these upcoming events:

https://zoom.us/webinar/register/WN_xeAHuUEXQzynhUXzUw23-Q

Also, the VIS SIG organises a further session at the conference. The VIS SIG discusses other formats to increase the capabilities of statisticians around data visualisations and raise the awareness of data visualisations.

Alexander Schacht, Zachary Skrivanek, Lorenz Uhlmann, Steve Mallett, Bodo Kirsch on behalf of the VIS SIG

Pre-clinical ESIG

The Pre-Clinical SIG organised the following webinars in 2021:

-Beyond the looking glass: Interpreting animal welfare & behaviour by monitoring & assessing mice activity data (Eloisa Brook, Joanna Moore and George Birkbeck, GSK)
-Update from the Carcinogenicity working group (Steve Bailey, Pfizer)

N.B. These are all available on the PSI website under Video on Demand.

We plan on running our 10th Workshop in April 2022. This will be the first time this event is run virtually. For more information please consider joining our distribution list by emailing eloisa.i.brook@gsk.com.

Eloisa Brook (GSK)

Estimands in Oncology

The oncology estimand SIG again had another very busy and successful year. Key activities and achievements are listed here. We also keep updating the group's webpage www.oncoestimand.org. It now features a tab Further resources with links to other estimand SIGs and a section I have never heard of estimands - where should I start? This might be a good landing page to point out to interested colleagues who'd like to learn more.

Kaspar Rufibach (Roche)

Data Sharing ESIG

The Data Sharing ESIG have had an open access article published in Discover Artificial Intelligence on "Synthetic data use: exploring use cases to optimise data utility".

https://trebuchet.public.springernature.app/get_content/6badbd95-0836-4146-89d1-3a6ad23411bf

Rebecca Sudlow (Roche)

Please look out for updates and other ESIG news at https://www.psiweb.org/sigs-special-interest-groups/sigs

Adam Crisp (PSI SIG liaison)  ***and Gaëlle Saint-Hilary (EFSPI SIG liaison)*** 

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Country News

APF (Germany)

APF held its German statistics leaders meeting on November 25th. Topics included "Usage of R in Pharma Development" and APF'S efforts to engage with students. On November 26th the annual workshop had the topic "Breakthrough Therapies" with a keynote talk by Benjamin Hofner from the German PEI.

PSI (UK)

UK Medical Statistician Level 7 Apprenticeship Scheme

The Medical Statistician Level 7 (MSc integrated) Apprenticeship Scheme is now live. See https://www.instituteforapprenticeships.org/apprenticeship-standards/medical-statistician for more detail of how to register for an apprentice to join your company and a summary can be found here: https://www.psiweb.org/careers/educationandresources/medical-statistician-apprenticeship

The apprenticeship is made up of an "Occupational Standard" which describes the occupation, duties, knowledge, skills and behaviours expected, and an Medical Statistician assessment plan which describes how the scheme would work in practice and how the scheme is examined. Both documents are available at the government website above. Information about the road to a quality apprenticeship can also be found here: https://www.apprenticeships.gov.uk/employers/the-road-to-a-quality-apprenticeship

Well done to the working group led by Jim Saul (Lapcorp) and Lyn Taylor (Phastar) to achieve this milestone.

Registration is now open!

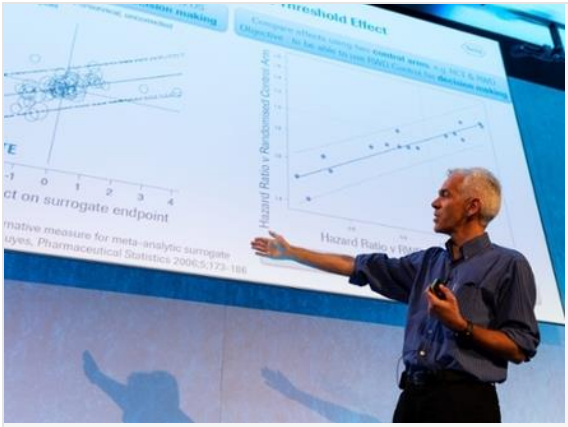
Join us at the PSI Conference 2022 in Gothenburg, Sweden face-to-face from **Sunday 12 to Wednesday 15 June 2022**.

Registration for the conference is **now open**. Click below to see more details and register now to qualify for the Early Bird discount. Group registration discounts are also available if your company is sending multiple attendees.

Register here

David Lawrence PhD, PSI Conference Chair

Upcoming Events



PSI Training Course: Use of Historical D...

Jan 24 - 27, 2022 (GMT+0)

PSI Medical Statistics Careers Event

Mar 2, 2022
(10 AM - 5 PM) (GMT+0)

ON-DEMAND WEBINARS AND PODCASTS

PSI VisSIG Wonderful Wednesday 20: Competing risk data example

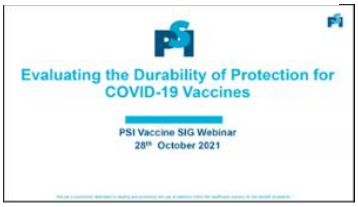
Bodo Kirsch discusses an approach to display data on competing risks based on an issue recently published. All visualisations are available on the Wonderful Wednesday blog.

Watch here

PSI Subgroup Analysis SIG Webinar: Modern Approaches to Subgroup Identification

In this webinar we look at some recent advances in statistical methods for identifying treatment effect heterogeneity in clinical trials. This ranges from identifying baseline biomarkers likely to influence the treatment effect (ranking) to provide novel biomarker 'signatures' (subgroups) with associated estimated enhanced effect (Individual Treatment Effects).

Watch here



PSI Vaccine SIG Webinar: Evaluating the Durability of Protection for COVID-19 Vaccines

Here, we show how to estimate potentially time-varying placebo-controlled vaccine efficacy in this type of staggered vaccination of participants. In addition, we compare the performance of blinded and unblinded crossover designs in estimating long-term vaccine efficacy.

Watch here

The 4 pillars to boost your career

Interview with Olaf Kapinski. Olaf has a really interesting character. He is an IT guy and he went up through the ranks of different companies, had a great career and learned quite a lot about leadership. He was applying a lot of these learnings and now he’s training others in that regard as he has his very own leadership program.

How to combine a control arm with RWE data

Listen to an interview with Margaret Gamalo about how to combine a control arm with real world evidence data. She has been sharing her knowledge for a long time and has gained lots of opportunities this way.

Helping others will help you in the long-run.

Statistical Engineering

In this episode, Sam Gardner together with Roger, Ron, and Geoff will dive deep into Statistical Engineering and will discuss about ISEA, SE and its examples.

Listen to these episodes and share them with your friends and colleagues.

Ciao and be an effective statistician!

Alexander Schacht

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Job Opportunities

See here for more details on the multiple roles available. For 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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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.

Chrissie Fletcher, EFSPI Newsletters 

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