European Federation of Statisticians in the Pharmaceutical Industry

Representing Statistical Associations in Europe

EFSPI Newsletter March 2021

In this newsletter:

Scientific

The Scientific Committee is planning for a number of events in 2021. Although none of them have a confirmed date, these are our intended meeting in preparation for 2020:

- A joint EFSPI/BBS virtual event on **Precision Medicine and Health technology Assessment**, **28th June 3-5pm CET**. More details to follow.
- An event on **COVID**, together with the ESIG on Vaccines, planned in second half of 2021.
- A meeting together with the ESIG on **Small Populations** also in the second half of 2021
- An event on **Decentralised trials** in the last quarter of the year.

At this moment all these events are planned to be online, but as soon as we have more concrete information, we will share with you via the Newsletter and our website.

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ESIG News



Regulatory ESIG

DATE FOR YOUR DIARIES: This year's ***EFSPI regulatory statistics workshop*** will take place on three half days during the **13th-15th September 2021**. A first announcement with more details will be shared soon.

ANOTHER DATE FOR YOUR DIARIES: EFPIA in collaboration with representatives from EFSPI, EMA, CTFG, HTA agencies, Patient Advocacy and EORTC will be holding a two half-day workshop on **Accelerating Complex Clinical Trials in Europe and beyond** on the afternoon (GMT) of the **5th and 6th October 2021.** Further details will be shared soon.

Christoph Gerlinger (EFSPI Regulatory Chair), Jurgen Hummel (PSI Regulatory Chair)

Estimands in Oncology

LUNGevity - FDA Oncology CoE - ASA biopharmaceutical section Teleconference (TC)

On 11th February, these three organizations organized another joint TC discussing topics relevant to all of them. The title of this last TC was *Statistical considerations in Oncology clinical trials in the COVID-19 era*, find the agenda attached. The oncology estimand SG had been invited to present on *How can the estimand framework support decentralized trials?* See all the slide decks that can be publicly shared on our webpage. Some takeaways from the TC were:

- The TC was well attended, with regulators from all over the world dialing in and contributing to the discussion.
- In the symposium there was general agreement that the estimand framework is an excellent tool to assess the impact of decentralization on trials.
- The pandemic forced many trials to "decentralize". Building on this experience the FDA seem to push that heavily, especially for "low hanging fruits" such as labs, routine assessments, etc.
- A question that seems to concern FDA more in terms of feasibility are more complicated response assessments and other involved procedures like CAR-T, e.g.
- Although it might not make much of a difference in terms of ability to estimate a relative effect (see this famous paper "Why representativeness should be avoided" https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3888189/), there seems to be push towards trials to be more inclusive. From an industry point of view we can also see this as making sense and would potentially enable us to 1) recruit faster 2) run more RCTs as we get better access to more patients.

- Numbers are supporting that view for certain groups of patients: The speaker from Mayo clinic reported that in their trials they observe more late or missed assessments for women, in metropolitan areas, and for minorities during the pandemic. This might give a hint to what might be expected for decentralized trials as well.

New: Central Statistical Monitoring/Quality Tolerance Limits ESIG

The use of Centralised Statistical Monitoring and Quality Tolerance Limits are fundamental components of Risk Based Quality Management as laid out in ICH-E6(R2) Good Clinical Practice. However, although there has been much commentary published on the operational application of Central Monitoring & QTLs, there appears to be a gap in the literature for the more detailed statistical methodologies that can be utilized. As a result, the new CSM SIG has been formed. Click here to find out more.

New: Real-World Data (RWD) ESIG

The RWD ESIG would like to invite further members to join. To date, there members from 6 companies and one university who have joined the ESIG.

The purpose of the RWD ESIG is to increase collaboration and enhance awareness of strategies and methodologies applied in the utilization of Real-World Data in the pharmaceutical industry. We aim to facilitate sharing of case studies and experiences, to develop best practices with a view to influence industry practice. For more information, see: **https://www.psiweb.org/sigs-special-interest-groups/rwd-sig.**

If you are interested in contributing to the RWD SIG, please get in touch with Anny Stari (GSK).

Small Populations ESIG

The Small Population SIG, with the support of the Scientific Committee is planning to organize a meeting on the topics of Historical Controls, Extrapolation and Meta-Analysis. We are looking for case-studies to be presented during this meeting. If you have a relevant case-study to propose, please contact François Aubin francois.aubin@vennlife.com and/or Andreas Kaiser andreas.kaiser@bayer.com.

Toxicology ESIG

We are always on the lookout for new members to join our small committee. Recently we held a webinar for statisticians supporting in-vivo studies and heard how GSK evaluated home cage monitoring systems. If you're interested in joining our monthly meetings and being part of our committee, please contact Gareth Thomas. Click here to find out more.

Please look out for updates and 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

BBS (Switzerland)

Statistical Challenges in CAR-T Cell Therapy Webinar on March 22, 2021

Developing CAR-T Cell therapies has unique challenges not observed with traditional drugs. On top of the manufactory and logistical challenges, clinical development is facing unique statistical questions which need to be resolved for delivering the treatment effect, all of which and more were covered during the BBS webinar on "Statistical Challenges in CAR-T Cell Therapy".

Despite the narrow scope of this topic, we still had around 430 persons registered and with almost 200 people actively joining, interestingly almost 50% of the registrants coming from the USA. It was an excellent program starting off with the clinical perspective and then digging into the regulatory and reimbursement challenges with perspectives coming from academia, industry, and three representatives from Health Authorities (EMA, MHRA & FDA). The program was quite extensive that limited to some extent the time left at the end for the panel discussion.

We received positive feedback from all the speakers as well as participants, and Frank Petavy from EMA who served as one of the panelists, also requested that we should look to organize such a meeting for other advanced therapies.

Overall, it was viewed as a great success, and as a result we will look to organize another webinar / seminar on the same topic at a future date. And, as usual, all the slides from the presentations as well as a recording of the entire webinar is available on the BBS website: http://bbs.ceb-institute.org/?p=1704. Special thanks also go out to Bibiana Blatna from Novartis for helping to coordinate the meeting logistics and ensuring a smooth running and recording of the webinar.

On behalf of the BBS and the CAR-T cell therapy webinar organizers
Roland Marion-Gallois (BMS), Simon Newsome (Novartis), Fred Sorenson (Xcenda)

FMS (Sweden)

On March 25th, the Swedish Society of Medical Statistics, FMS held a Spring meeting. The theme of the meeting was ***Recent advances in medical statistics and machine learning for practicing statisticians***. There were three scientific presentations from young researchers, Linda Vidman, Peter Ström and Sara Ekberg, recently completing their PhDs in Biostatistics. The workshop in machine learning for practicing statisticians was given by Måns Magnusson, Uppsala University.

FMS has instituted a new prize: **The FMS award for best paper by younger statistician**, which was awarded for the first time. The prize was given to Enoch Yi-Tung Chen, Department of Global Public Health, Karolinska Institute for his master thesis - Extrapolating cancer patient survival: a comparison of the flexible parametric model and the rolling-over algorithm, with an award of 5000 SEK.

Anna Torrång

PSI (UK)

PSI Conference 2021

The PSI Conference will be held online from 21 to 23 June 2021 and registration is now open. Registration for the Online Conference includes:

- Access to:
  - exciting, relevant and up to date presentations and sessions over the three live days
  - additional on demand content
  - the latest research in the e-poster portal
- Continued access to all of the Online Conference content for at least 6 months after the conference, allowing you to view content at your leisure
- Opportunity to attend live networking sessions
- Ability to meet and engage with sponsors and exhibitors
- Ability to catch up and connect with colleagues

Companies that sponsor the 2021 PSI Online Conference will receive a large number of complimentary delegate passes that will be issued separately.

Find out more [with link to https://www.psiweb.org/conferences/about-the-conference]

Statistical Excellence in the Pharmaceutical Industry Award

Nominations are being sought for the 2021 Award for Statistical Excellence in the Pharmaceutical Industry, jointly run by PSI and the Royal Statistical Society (RSS).

Nominees can be based anywhere in the world and do not need to be a member of PSI or the RSS. The award will be presented at the annual PSI AGM in July. Click here to find out more.

MEETINGS, WEBINARS AND COURSES

PSI VisSIG Webinar: Rapid Insights to Data

16:00-17:00

Who is this event intended for? This event is intended for all statisticians and statistical programmers interested in data visualization.

What is the benefit of attending? Attendees will focus on the latter two fields of data visualization as it relates to data visualization: neuroscience and cognitive psychology.

Register now...

PSI Non-proportional hazards and applications in immuno-oncology

10:00-16:30

Who is this event intended for? All statisticians from research/academia/ Pharma industries, especially those working in immuno-oncology or other fields where non-proportional hazards may be anticipated.

What is the benefit of attending? Hear about potential strategies to handle non-proportional hazards and delayed treatment effects from experts in the field.

Register now...

PSI Missing Data in Clinical Trials - Past, Present and Future

4 May: 10:30-13:30, 5 May: 9:00-12:00

Who is this event intended for? Statisticians involved in designing and analysing clinical trials.

What is the benefit of attending? Gain awareness of the increasing importance of appropriate missing data handling in clinical trials by learning about state-of-the-art methodology and recent case studies.

Register now...

PSI Training course: R for SAS users

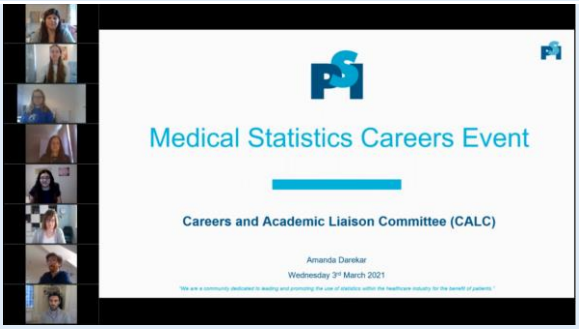
9:45-12:00

Who is this event intended for? Statisticians who are experienced with other software languages (such as SAS) and would like to increase their proficiency in R.

What is the benefit of attending? Attendees will learn how to: understand data manipulation in R; produce R graphics with ggplot2; and perform & interpret statistical analyses in R.

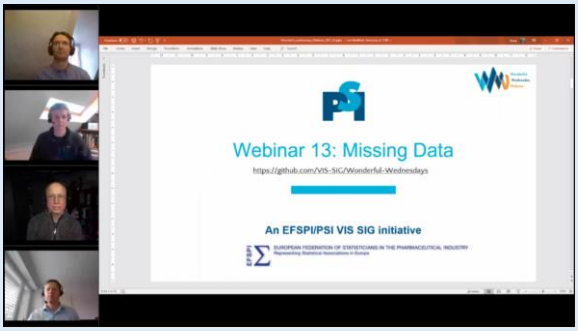
Register now...

Podcasts & Webinars



PSI Careers Event 2021 Panel Discussion
An introduction to the event from the chair of PSI CALC and Panel Discussion featuring company volunteers.

PSI Webinar: Wearable Technologies - Challenges and Opportunities
Wearable technologies and digital health data offer great opportunities for studying patients functionally in real life settings. Actigraphy, for example, can be used as part of clinical trials to collect continuous movement data, but the frequency of data collection results in dense datasets requiring extensive processing and signal detection. In this webinar, a panel of expert speakers will discuss how such aspects can be addressed to help realize the promise of these technologies.



PSI VisSig Wonderful Wednesday 13: Missing Data
Steve Mallet leads the discussion on ways to display data that is actually missing. The example data was based on a study with multiple measurements of pain that were partially incomplete. They presented visualisations which can help to describe the amount, the nature and the impact of missing data on the study outcome.

Data monitoring committees for clinical trials and the role of the statistician

David Kerr and I were talking with a niche part of clinical research many statisticians never get actively involved in: DMCs. Join us while we discuss DMC, its role for DMC statisticians, and the best skills and traits of a DMC statistician.

The Chimp Paradox

Stuart and I got a recommendation to read a book entitled “The Chimp Paradox: The Acclaimed Mind Management”. This book is an incredibly powerful mind management model that can help you become a happy, confident, healthier, and more successful person. Join us in this episode while we discuss how to manage your inner chimp, and more.

The power of simulations for designing clinical studies and beyond

Aiden Flynn has invested an enormous amount of time to leverage simulations for understanding the designs of studies and development programs. In this episode, we discuss Why is trial simulation necessary, What design features does he assess, and how does he get a good stability in terms of the simulation precision.

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6 steps to prepare before you want to convince your business partner

Maybe you want to improve a process or implement a new analysis approach. Whenever we want to change something, we usually need others to agree to the next steps. Listen to this to know the 6 steps to prepare before convincing your business partner.

A virtual summer academy for statisticians

In this episode, Qiqi and I talk about BI-UConn Summer Academy, an initiative we started 3 years ago to help students understand and learn the world of clinical development better and bring them networking opportunities.

Listen to these podcast episodes now and share it with others who might learn from it.
Ciao and be an effective statistician!
Alexander Schacht

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Job Opportunities

Opportunity for a Director and Team Leader, Biostatistics.

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 Communications Officer

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