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

EFSPI Newsletter

SEPTEMBER 2022

CONTENT

WELCOME

A warm welcome to the September newsletter from EFSPI!

I would call this September "the Swiss month" for EFSPI!

… Why? Because this month we made it to the 7th EFSPI Regulatory Statistics Workshop in Basel. Check out the 'hot of the press news' from this fantastic multi-stakeholder event and gain insights into relevant topics from different perspectives.

In addition, read about the 4th EFSPI Council meeting in 2022 kindly hosted by Roche, and finally get to know the Basler Biometric Section (BBS) which is our EFSPI local association of the month!

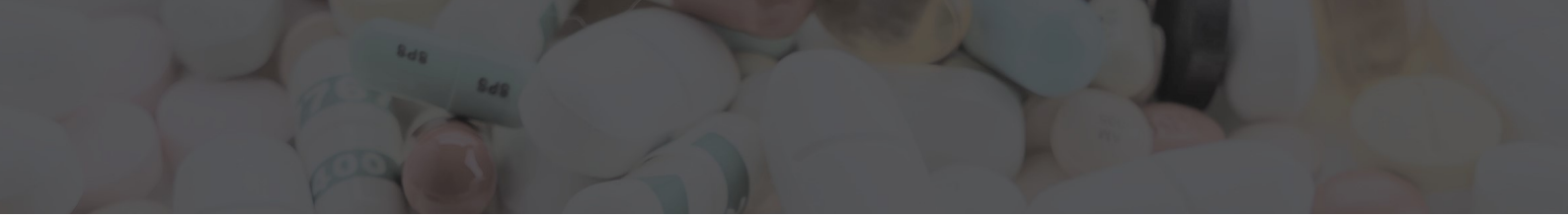
Enjoy the reading and let me know how you liked this newsletter. Please also contact me if you have anything you would like to contribute to our future newsletters and our EFSPI community.

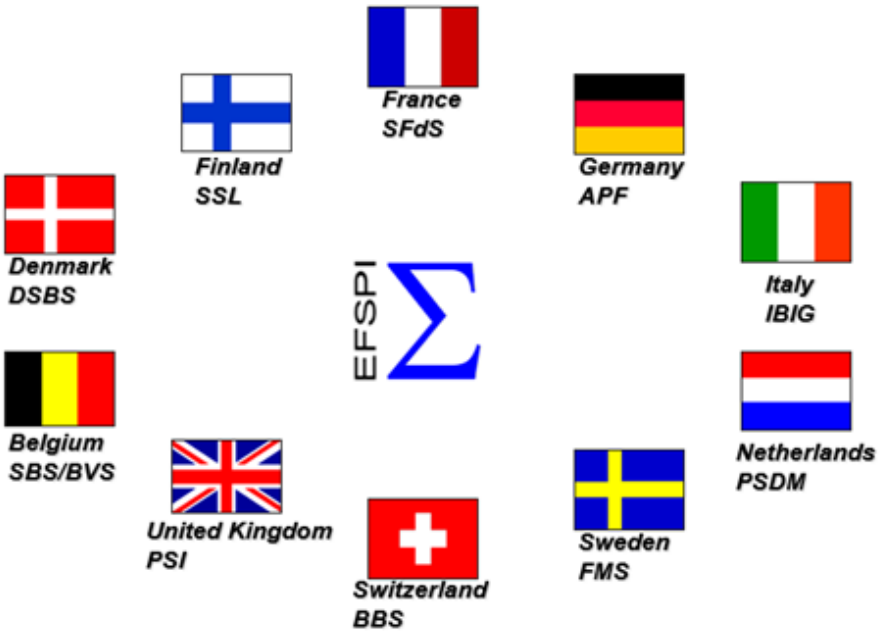
Justine Rochon
EFSPI President and Editor of EFSPI Newsletters

EFSPI COUNCIL NEWS Let's refresh memory!

EFSPI stands for the European Federation of Statisticians in the Pharmaceutical Industry. The federation was officially launched in August 1992 and is registered in Denmark since 2018. EFSPI is a non-profit organization, serving as an umbrella to constituted groups of statisticians. There is no individual membership, but we currently represent 10 national associations including more than 2000 pharmaceutical statisticians in Europe. We promote professional standards of statistics and the standing of the statistical profession in matters pertinent to the European pharmaceutical industry. We offer a collective expert input on statistical matters to national and international authorities and organizations. We exchange relevant information and harmonize attitudes to the practice of statistics in the European pharmaceutical industry and within the member groups. And there is much more we are committed to realise! At the beginning of this year, our EFSPI Council members aligned on priorities for 2022 and beyond. First, we collectively decided to focus on ensuring business continuity. The tradition of annual EFSPI Regulatory Statistics Workshop is one such example. Second, we decided to enhance our branding and visibility. Finally, we committed to further transform EFSPI into a federation that makes a difference today and into the future, and especially invest into the next generation of statisticians and statistical leaders.

If you want to learn more about our Member Groups, the EFSPI Council, our current priorities and work, please visit our website at www.efspi.org and follow us on EFSPI LinkedIn.





4th EFSPI Council Meeting 2022

We want to thank Roche for kindly hosting the Council on 16 September 2022 in Basel. This was our first in-person meeting since 2019!

Besides regular updates from the EFSPI president and the EFSPI office, this Council meeting was dedicated to strategic considerations and budget review as we are approaching the end of the year.

We also discussed learnings from the two key 2022 EFSPI events: The 13th annual EFSPI Leaders Meeting from July and the 7th EFSPI Regulatory Statistics Workshop from September. In addition, we again took a deeper dive into the ESIG activities and aligned on a funding proposal for future ESIG webinars. The final agenda topic was EMA's Raw Data Submission Pilot. Stay tuned to hear more about EFSPI's role in this project!

EFSPI Regulatory Statistics Workshop

The 7th EFSPI Regulatory Statistics Workshop took place on 14-15 September 2022 as a hybrid meeting, with a focus on in-person attendance at the Biozentrum Basel.

This year, Justine Rochon officially opened the workshop in her role as EFSPI president and welcomed almost 700 registered colleagues from 29 different countries; big pharma and biotech, CROs, academia and several European Health Authorities, FDA, PMDA, and MHRA; by the way not only statisticians but also clinicians and colleagues from other disciplines. Our background, location or current employer may differ but what truly unites us is the same purpose.

The 2-day program was filled with excellent topics provided by excellent speakers and plenty of opportunities to interact with each other.

For an overview of topics for Day 1 and Day 2, see the next pages of this newsletter.

Day 1
In our introductory keynote session of Day 1 we had invited four distinguished speakers: Kit Roes, Frank Bretz, Anja Schiel, and Jasvinder Singh to provide their perspectives on **"What happened in the last to years on the regulatory and HTA landscape"** followed by a panel discussion chaired by Eftychia Eirini Psarelli and Randi Grøn.

All speakers brought points forward that sparked great discussion between the panel and audience on Estimands and PICOs, RCT vs RWE, EUnetHTA21, and the key message that early scientific advice is key from both, the regulatory and the HTA perspectives!

Postbaseline subpopulation analyses: known to be improper, but frequently done. Can we fix them?
This was the title of the afternoon session of Day 1 chaired by Mouna Akacha and Khadija Rantel.

Bjoern Bornkamp and Anja Schiel were invited to present and set the scene discussing relevant scientific questions from different perspectives, sponsor vs regulator vs HTA, including case examples and terminology around proper/improper sub-groups and subpopulations.

After the two talks the speakers and session chairs were joined by Mats Stensrud, Fabrizia Mealli, Kaspar Rufibach, Stephen Ruberg, Wanjie Sun, Florian Klinglmueller, for a hybrid panel discussion. Mouna Akacha opened the dialogue with a question to all in the panel: "Do you think that trial objectives/estimands focusing on postbaseline subpopulations are relevant?".

After a lively discussion, Day 1 of the workshop was concluded with a great **wine tasting and networking event** organized by Emmanuel Zuber and Hans Ulrich Burger.

Day 2
First session of Day 2, chaired by Christoph Gerlinger and Andreas Brandt, dived into **complex innovative designs and how to deal with different priorities for regulators and HTA bodies**. The invited speakers brought a mix of case studies and education pieces:

- Dieter Haering-Flury & Marius Thomas : Neos paediatric trial of Kesimpta and Mayzent in multiple sclerosis
- Theodor Framke: Regulatory view on complex innovative designs
- Anders Viberg : HTA's view on complex innovative designs.

The panel discussion included Anja Schiel, Kit Roes, Katharina Hees, and Franz Koenig.

Second session of Day 2, chaired by Kaspar Rufibach and Kit Roes, dived into the important discussion around **Generalisability and external validity: How to generate evidence about treatment effect?**

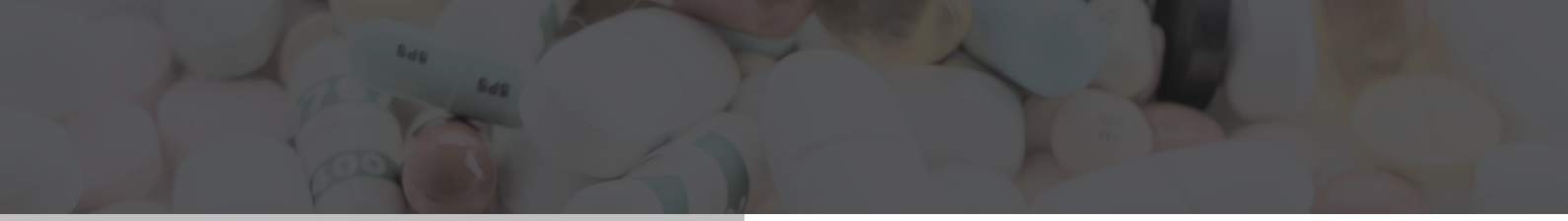
- Stephen Senn: Clinical trials are about comparability, and not generalisability
- Florian Klinglmueller: Regulatory view on generalisability
- Joshua Ray: Let them in or build a wall? Transporting inferences across borders

After the three talks Robert Hemmings joined the speakers in a panel discussion that again sparked a lot of questions and comments from the audience.

The afternoon session on Day 2 was chaired by Emmanuel Zuber and Benjamin Hofner. EFSPI felt honored to welcome such distinguished guest speakers from FDA, PMDA and MHRA, and learn more about various perspectives on the "**Role of statistics / quantitative science in regulatory decision making**"!

- Aloka Chakravarty (FDA): Generating Actionable Insights Using RWD during COVID-19 pandemic.
- John Johnston (MHRA): What role should statistics play in regulatory decision-making?
- Yuki Ando (PMDA): Role of biostatisticians in regulatory decision making: the current discussion in Japan.
- Rajeswari Sridhara (FDA): Experience with the project SignifiCanT.

After the presentations, the speakers were joined by Kit Roes, Anja Schiel and Robert Hemmings for a panel discussion underlying once again the importance of good communication and good science:
"If you can't explain it simply, you don't understand it well enough." (Albert Einstein.)



Do you have a question or problem that you would like regulatory feedback to?

Again this year we concluded the workshop with a short topics session including

- Oliver Sailer, Stephan Wojciekowski, Dietmar Neubacher: Bayesian borrowing from simulated paediatric patients with type 2 diabetes mellitus
- Bjoern Holzhauer: Procova approach to build a super-covariate' based on AI models
- Andrea Schulze: Methodological aspects of the war in Ukraine for clinical trials
- Armin Koch: My Type-1 Error
- Vivian Lanius: Causal(?) estimands for time-to-event endpoints

This is always an interesting session and we thank our five presenters and all the regulatory colleagues for their valuable contributions to the success of this session!

Kudos to the Scientific Committee and the local organizers for making this workshop possible and a truly memorable experience!

Special thanks go to Kaspar Rufibach, the 'face' behind the EFSPI Regulatory Statistics Workshop! Just when we thought it couldn't get any better, it did. Together with the Scientific Committee and the local team, Kaspar showcased what it truly means to have a constructive multi-stakeholder dialogue. Thanks for going beyond pure statistics, bringing the right people together and enabling the EFSPI community to work towards the same shared goal!

For further information, see the materials available from the workshop at the EFSPI webpage.

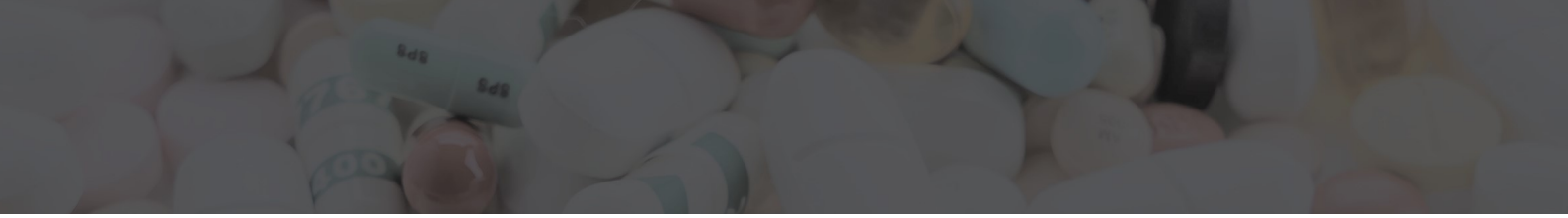
CELEBRATION TIME !!!

We are delighted to announce that EFSPI has reached 1,000+ followers in September 2022. This is a great success given the short time span since the EFSPI page was launched on LinkedIn. We could not be happier about this!

A huge thanks to all of you who follow, like, share and comment on our content. We appreciate your support, and we love engaging with you! Your engagement with us inspires us to do better with every post that we share with you. We endeavor to bring insightful and meaningful content that adds value to you. Therefore, let us know what we should keep, start, or even stop doing when creating our posts.

And lastly, if you are not already following EFSPI, join us today! Following us on LinkedIn will allow you to keep up to date with our latest news and events. By following us, you will get the opportunity to learn from others and become part of an awesome community!

Your EFSPI communication team:
Randi Grøn (EFSPI Communication Officer)
and Justine Rochon (EFSPI President).



Multi-stakeholder workshop on patient experience data in medicines development and regulatory decision-making

Dear EFSPI community, I would like to share with you some first insights from a workshop on patient experience data in medicines development and regulatory decision-making workshop I attended at the EMA on 21 September 2022.

EMA's Regulatory Science Strategy to 2025 recognises the need to identify optimal approaches for engaging patients in medicines development and benefit-risk assessments, including the development of standards for designing, conducting, analysing and reporting relevant studies incorporating patient experience data for regulatory submission, and to elucidate how such data can best inform regulatory decisions. Patients have valuable insights and perspectives from living with a condition and its treatment. This includes symptoms, natural history, quality of life, unmet needs, which outcomes are important and preferences for future treatments. Input from patients, as users of medicines, can inform medicine development, enhance regulatory decision making and result in more patient-relevant outcomes.

This multi-stakeholder workshop brought together patients, healthcare professionals, academia, regulators, and industry to discuss ways to improve the collection and use of patient experience data to achieve patient-centred medicine development and regulation. I was invited to this multi-stakeholder workshop in my role as EFSPI president.

The aim of this workshop was to discuss together how to ensure that the patients' voice is systematically included throughout the life cycle of each medicine. Emer Cooke (Executive Director of EMA) opened the workshop with a call to action: "We need active contribution, we need arguments, we need debate!" Why? "Because we still don't have the patient experience systematically included in all aspects of medicines development and regulations. If we want our outcomes to be meaningful, we need to improve the status quo."

The workshop consisted of six sessions:

Session 1: Patient Engagement including an introduction of the EMA framework for engagement and presentations on how patient engagement can contribute to the development and approval of medicines and safety monitoring of medicines

Session 2: Patient Preference Elicitation including a definition of patient preferences, a deeper dive into patient preference research, and learnings from use-cases

Session 3: Patient Reported Outcomes including presentations on PRO contribution to the development and approval of medicines and PRO data generation in practice

Session 4: Digitalization for patient-generated health data including presentations on European Health Data Space, tools to collect patient-generated data, and data platforms

Session 5: Guidance on collection and use of patient data including presentations on qualification of novel methodologies and ICH Patient-Focused Drug Development (PFDD) initiative

Session 6: Summary and next steps

My key take away message from the workshop: This is not a 'tickbox' exercise. Instead, genuine patient engagement is required (and expected!) from all stakeholders. In addition, there were some common topics in all presentations and discussions that are relevant for EFSPI: Multi-stakeholder dialogue and collaboration, importance of data quality, methodology, study design, analysis, and evidence generation. It became clear that there is still room for greater progress in guiding stakeholders on what constitutes sufficient evidence to enable the use of patient experience data to support regulatory decision-making, labelling claims, health technology assessment, and eventually patient access. I am pleased to share with you the link to the published recording and presentations.

Justine Rochon, EFSPI President

ESIG News

Do you know ESIGs?

It is European Special Interest Groups (please note the capital for the "E" as it stands for European). They are sponsored jointly by EFSPI and PSI, and therefore should be cited as ESIG, no mention of PSI nor EFSPI which are included in the "E". Our ESIGs play a key role and foster connections across disciplines, industry, academia and countries/regions. The ESIGs provide a forum for members to discuss topics of mutual interest, keep updated on developments in a particular area of industry, to organize events on their specialist field and/or to collaborate on developing the science of that field.

What's new?

The ESIG poster session at the 7th EFSPI Regulatory Statistics Workshop was a great success (from the upper left corner in clockwise direction): Juergen Hummel presented the Regulatory ESIG ("probably the longest serving ESIG, dating back to at least 2006"). An Vandebosch talked about the work of the Vaccines ESIG. Lara J Wolfson and Anders Gorst-Rasmussen (HTA ESIG) gave answers to "What is EU HTA?" and "Why should statisticians care about EU HTA?". Stefan Englert gave an update from the Estimands in Oncology ESIG and Jenny Devenport (Launch & Lifecycle ESIG) asked the thought-provoking question "Is medical affairs the wild west of statistics?". The poster session was completed with two more posters from the Data Sharing ESIG and the Estimand Implementation Working Group.

Do you want to contribute to our ESIGs? For the names and contact details of our ESIG leads, please see our ESIG Lead Contact Page.

Do you want to become our ESIG of the month? Then please contact Emmanuel Pham (EFSPI SIG liaison) or Adam Crisp (PSI Board SIG liaison lead) to be featured in one of our next newsletters

LOCAL ASSOCIATION OF THE MONTH

The Basler Biometric Section (BBS) of the Austria-Switzerland Region (ROes) is part of the global organization, the International Biometric Society (IBS). BBS is an independent, non-profit organization which offers a platform for sharing information and exchange of opinions on statistical methods applied to various areas of medicine, including drug development, biology, epidemiology, and more.

The current BBS president is Hans Ulrich Burger from Roche, and he represents the BBS together with Marisa Bacchi (JnJ) at the EFSPI Council. The BBS Council has representation from most of the Swiss based pharma and biotech companies, as well as the University of Basel and the Swiss Institute of Tropical Medicine.

The BBS is very active, and the Council meets regularly to discuss scientific/statistical topics and to organize seminars, training sessions and other events, often in collaboration with EFSPI. More information can be found on the BBS website http://bbs.ceb-institute.org

Marisa Bacchi, Swiss representative in the EFSPI Council

OTHER COUNTRY NEWS

PSI (UK)

MEETINGS, WEBINARS AND COURSES:

13 October 2022: PSI Scientific Meeting: Adaptive Designs and their Application

17-18 October 2022: PSI Scientific Meeting: Decentralised Clinical Trials

20 October 2022: PSI Vaccines SIG Webinar: Assessing Population Level Vaccine Effectiveness Under Different Study Designs

25 October 2022: PSI Webinar Series: Showcasing R use in Pharma

9 November 2022: PSI Careers - MEDMathS: Medicine Empowered by Data, Maths and Statistics

14 November 2022: Joint PSI & EFSPI HTA SIG Webinar: Statistics in EU HTA - PICOs, Estimands & More

The Effective Statistician Podcast

Listen here!

Listen to these episodes and share them with your friends and colleagues who might learn from it.

Ciao and be an effective statistician! Alexander Schacht

SFdS (France)

SnB 2022 has been held in Cité universitaire Internationale, in Paris, 19-21 September. It has been a very successful event, with around 200 attendees!

The main theme was **When Machine Learning meets Statistics for Drug Development and Evaluation** with four sessions:

- Big data to the rescue of drug development challenges?
- Statistics and Machine Learning: friends or foes
- Getting medicines to patients faster - the role of innovative designs
- Lessons learnt from the COVID-19 experience.

There was also a half day dedicated to debate, which happened to be really successful.

Please feel free to have a look at SnB 2022 - Statistics & Biopharmacy

ABSTRACT SUBMISSION OPEN FOR THE PSI CONFERENCE 2023!

PSI are pleased to announce that the countdown to the PSI Conference 2023 has now begun!

We are now taking submissions for any of your contributed oral or poster abstracts. The deadline for oral abstract submissions is 18 November 2022. As a reminder you can submit individual abstracts or abstracts for a full session. You can see the full list of abstract topics and download the abstract templates on the website. Remember, anyone selected for an oral presentation, will be eligible for 10% off the three-day conference price!

This year we are adding a new option to give a TED-style talk. These talks should be only 5-10 minutes in length and can focus briefly on a big idea, a small idea, an issue etc. Please indicate on your abstract form if interested. Conference registration will open soon, we look forward to seeing you in London!

JOB OPPORTUNITIES

Nowadays, job opportunities for statisticians and data scientists are excellent!

For information on how to submit recruitment adverts, please visit the Job postings on our EFSPI website. 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.

AND FINALLY...

To add your email address to the EFSPI mailing list, click on "Sign up to our newsletter" on our landing page. To view all newsletters please see the "News" area on the EFSPI website.

We value your feedback, so let us know what should we keep, start or even stop doing when creating the EFSPI newsletters?

We would also like to learn from you how we can further improve our EFSPI communication and branding.

Please share your thoughts with Justine Rochon and Randi Grøn.