



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

EFSPi Annual Report 2014

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Highlights from the EFSPI President & Vice President



EFSPI met for the first time with the CHMP's Biostatistics Working Party at the EMA in London for an in-depth discussion of current biostatistics topics. EFSPI commented on the draft guideline on subgroup analyses and was invited to give an oral presentation of our comments at a workshop at the EMA in London. EMA published its final policy on clinical data transparency and the EFSPI data transparency working group will continue its efforts to develop white papers in related topics in 2015.

EFSPI held their 5th EFSPI Statistics Leaders Meeting in 2014. Topics at this meeting were: EFSPI strategic objectives 2013-2015, recap 2013 meeting, Data Sharing, SIG updates, new SIG IDA presentation, SIG Pharmaco-Epidemiology achievements and plans, and finally Recent Developments in Data Science.

A number of the Special Interest Groups began to work together on areas of mutual interest with a number of joint sub-teams being formed, e.g. benefit-risk in HTA.

The EFSPI membership fees remained the same in 2014. The operations board continued to monitor expenses throughout the year.

Chrissie Fletcher (UK)

President

Egbert Biesheuvel (the Netherlands)

Vice-President

Finance



EFSPI had planned to make a small loss in 2014 due to a planned one-off spend on improving the EFSPI website. A slightly larger loss was realised overall in 2014 primarily driven by reduced revenues from reduced scientific meeting activity although the reduced revenues was slightly offset by reduced expenses. The planned spend on upgrading the EFSPI website was not fully realised in 2014 and some of the cost will carry-over into 2015. Careful oversight of expenses required to run EFSPI enabled costs to be below our budget expectations. Despite realising a slightly larger loss, our reserves remain at a sufficient level.

Birgitte Biilmann Rønn (Denmark)

EFSPI Treasurer

EFSPI Income and Expenses 2014

	Actual €	Budget €	Variance €
Income			
Membership Fees	12.514	12.511	3
Scientific Meetings	7.190	27.000	(19.810)
Recruitment Web Advertisement	1.750	-	1.750
	<u>21.454</u>	<u>39.511</u>	<u>(18.057)</u>
Expenses			
Executive Office Hours	13.952	14.000	48
Attending Meetings	868	2.400	1.532
Web Development & Hosting	2.967	4.500	1.533
Office Costs	908	500	(408)
Bank Charges	1.621	2.000	379
Scientific Meetings	2.448	18.000	15.552
	<u>22.764</u>	<u>41.400</u>	<u>18.636</u>
Net result for the year	<u>(1.310)</u>	<u>(1889)</u>	<u>579</u>

Balance

	2014 €	2013 €
Current assets		
Debtors	450	1.882
Prepayments	398	1.381
Accrued Income		1.883
Bank - €	39.102	40.989
Bank - £ (Converted to €)	214	2.989
	<u>40.164</u>	<u>49.064</u>
Current liabilities		
Creditors	833	2.285
Accruals	10	6.148
	<u>843</u>	<u>8.433</u>
Revenue reserves		
Balance brought forward	40.6317	34.767
Result for year to date	(1.310)	5.865
	<u>39.322</u>	<u>40.631</u>

Birgitte Biilmann Rønn (Denmark)

EFSPI Treasurer

Regulatory Affairs



2014 has been a busy year for the PSI/EFSPI regulatory committee with the following key events:

- The committee met with CHMP's Biostatistics Working Party at the EMA in London for an in depth discussion of current biostatistics topics. This was the first time that a CHMP working party met with their scientific counterparts from industry.
- The CHMP draft guideline on subgroup analysis was released for consultation. EFSPI commented and was invited for an oral presentation of our comments at a workshop at the EMA in London. Following the EMA workshop a small working group will continue to investigate and discuss aspects relating to statistical methodology and interpretation of subgroup analysis. Of particular interest is how these approaches will relate to the criteria applied to conclude consistency across subgroups.
- EMA published its final policy on clinical data transparency. Several major comments made by EFSPI and other key stakeholders have been taken into consideration by EMA. At present, the EMA policy only concerns the publication of study reports, clinical summaries and overviews. The transparency for individual patient data will be discussed further between EMA and the stakeholders.
- An expert group on estimands has been set up to provide a discussion forum on this topic and provide input into the ICH E9(R1) working group creating the addendum to E9. The first meeting will take place in mid-February 2015.
- An expert group on Post-Authorisation Efficacy Studies (PAES) has been set up to provide a discussion forum on the statistical and methodological issues pertaining to PAES. This group will collaborate with EFPIA and their working group and will support the review of the PAES guidance developed by EMA when it is released for consultation (expected in 2015).

Christoph Gerlinger (Germany)

Regulatory Chair

Scientific Affairs



EFSPI held one European Statistical meeting in 2014:

- Health Technology Assessment hosted by Bayer in Berlin, Germany on September 25. Key themes included recent trends in HTA e.g. parallel scientific advice, use of real world data in HTA, evidence generation strategies for HTA and study design and analysis challenges in HTA.

All presentations can be found on our website ('EFSPI international events'):
<http://www.efspi.org/index.php?p=EFSPI+activities&fid=19>

Egbert Biesheuvel (the Netherlands)

Scientific Chair

Statistics Leaders Meeting



The fifth EFSPi Statistics Leaders Meeting was held on June 11, 2014, in Basel, Switzerland.

A record number of thirty (30) leaders from 8 different countries and 20 pharmaceutical companies attended. Topics at this meeting were: EFSPi strategic objectives 2013-2015, recap 2013 meeting, Data Sharing, SIG updates, new SIG IDA presentation, SIG Pharmaco-Epidemiology achievements and plans, and finally Recent Developments in Data Science. The Working Group (WG) on Data Sharing that was formed end of 2013 presented their objectives and informed the meeting about the current five work streams. Main goals of the group are to identify and prospectively prioritize statistical issues in data transparency, and co-ordinate and communicate on statistical contributions across Europe. The current landscape of implementation of Data Sharing in the companies was presented, including the practical issues encountered. The overview also showed that different technical solutions have been put in place by various companies.

After a short update on Special Interest Groups (SIGs) the new SIG Integrated Data Analysis presented their aims and objectives. This SIG has set up four working group dealing with Efficacy Data in phase 2 and 3, Safety Data, Network Meta-Analyses, and IDA relative to greater Data Transparency. The aspirations of the SIG were well received. The SIG was encouraged to closely collaborate with other SIGs on topics like NMA and Rare Events, and also to work on estimates for safety parameters for later use in HTA and Benefit-Risk assessments. The SIG in turn asked for case studies from the companies on certain specific areas. The Pharmaco-Epidemiology SIG were next to present their many achievements over the last 6 years of their existence with several papers, handbooks and contributions to workshops and meetings. Feedback noted benefit in revitalizing previous SIG deliverables, especially in the area of Rare Events, bias and confounding. The latter is increasingly important due to the trend of increasing observational research within Pharma.

In the afternoon in a plenary session recent developments in the industry and regulatory environment regarding data and data sciences were reviewed and discussed. All participants had received in advance pre-reads on the topics like IMI, EFPIAs New Clinical Trial Design Task Force, EMAs guidance on Parallel Scientific Advice Regulators and HTA, EMAs PAES Working Groups, and Big Data – Real World Data. Also a survey was sent out

in advance on the level of involvement of the Statistics Leaders in these developing areas. Clearly there is growing involvement and interest in these new areas of data and associated data science. Many statistical challenges were identified in performing HTA, observational studies and the use of secondary data bases and Big Data projects (even though the last item lacks a clear definition). Predominantly data quality and how to deal with bias and confounding were mentioned as challenging when working in this area. And a need for best statistical practices was voiced. Access to best statistical practices and methodologies developed by experts in the field (e.g., SIGs, working groups), will give statisticians opportunities to demonstrate their added value, by collaborating and partnering with other data scientists. In this era of electronic health records and access to large databases, big data will bring new opportunities for statisticians.

Statistics Leaders Forum

In 2014 the Statistics Leaders Forum consisted of about 45 active members from all EFSPi countries and from a wide range of EU pharmaceutical companies, or affiliates of ex-EU companies. Chair of the forum is Stefan Driessen (stefan.driessen@abbott.com).

All material from the EU Statistics Leaders Meetings held in 2010 through to 2014 are available on the EFSPi website:

http://www.efspi.org/EFSPi/Statistics_Leaders_Meetings/Previous_Meetings/5th_Meeting_-_June_2014/EFSPi/Statistics_Leaders_Meetings/5th_Meeting.aspx?hkey=60573615-f29f-4c2d-96cd-a140f3c68cee.

Stefan Driessen (the Netherlands)

Statistics Leader Forum Chair

Special Interest Groups



In 2014 all SIGs have presented themselves in the EFSPI Newsletter (see scheme below). Almost each month a SIG showed their composition, their objectives and what they had achieved already and their plans for the future. See the EFSPI website to view each SIG summary.

		2014 EFSPI Newsletter Schedule with SIG input											
SIG	SIG Lead	January	February	March	April	May	June	July	August	September	October	November	December
Modelling & Simulation	Chris Campbell												✓
Health Technology Assessment	Chrissie Fletcher		✓										
Epidemiology & Safety	George Quartey						✓						
Benefit/Risk	Alexander Schacht										✓		
Biomarkers	Athula Herath			✓									
Medical Devices	Roland Marion-Gallois				✓								
Toxicology	Gareth Thomas					✓							
Integrated Data Analysis	Byron Jones									✓			

Stefan Driessen (the Netherlands)

Special Interest Group Chair

Communications



Monthly newsletters were distributed in 2014, and each of the Special Interest Groups (SIGs) had the opportunity to be “SIG of the month” summarising their activities to the EU statistics community. All newsletters are available on the EFSPi website (see address below).

The majority of the website redesign was completed in 2014 but the new website will be released in Q1 2015. The website redesign was achieved with the agreed budget with minimal overspend. The current website address is <http://www.efspi.org>.

Chrissie Fletcher (UK)
Communication Officer

François Aubin (France)
Website

Operations Board Summary

The Operations Board had monthly teleconference meetings over the year, during which all ongoing and future activities were reviewed and issues discussed.

The board is composed of: Birgitte Biilmann Rønn (Treasurer), François Aubin (Website), Egbert Biesheuvel (Vice-President and Scientific Affairs), Stefan Driessen (Statistical Leaders Meeting and SIGs), Chrissie Fletcher (President and Communications Officer), Christoph Gerlinger (Regulatory Affairs), and Julie Mellish (Executive Office).

Council Membership

In 2014, 10 countries national associations of pharmaceutical statisticians from 10 European countries were represented within EFSPi, totalising a combined membership of more than 2200.

During 2014, Maylis Coste replaced Françoise Tondu for France, Magnus Kjaer replaced John Alder for Sweden, and Mark Morris replaced Kevin Carroll for the UK.

Members of the EFPSI Council at the end of 2014 are listed in the Appendix.

Council Summary

Two Council meetings were held in 2014. The first took place in Basel, Switzerland, on 12th June. Seven countries were represented with 9 delegates. The second was held in Helsinki, Finland, on 3rd December. Twelve delegates, representing seven countries were present.

In addition to the two face to face meetings, two web conferences were held on 5th March and 8th October.

In December 2014 Marisa Bacchi replaced Egbert Biesheuvel as Vice-President.

Executive Office

The Executive Office facility continues to be provided by Kingston Smith (UK). Julie Mellish is the Secretariat for EFSPi.

Appendix: Council members at the end of 2014

Belgium

Emmanuel Quinnaux, IDDI
An Vandenbosch, Janssen

Denmark

Arne Haahr Andreasen, Andreasen Statistical Consulting
Birgitte Biilmann Rønn, Novo Nordisk

Finland

Tiina Hakonen, Oncos Therapeutics
Toni Sarapohja, Orion Pharma

France

Francois Aubin, Cardinal Systems
Maylis Coste, Servier

Germany

Frank Langer, Lilly
Christoph Gerlinger, Bayer

Italy

Paolo Morelli, Cros IT
Giampaolo Giacobelli, Rottapharm Madaus

Netherlands

Stefan Driessen, Abbott
Egbert Biesheuvel, MSD

Sweden

Marie Göthberg, Novo Nordisk
Magnus Kjaer, AstraZeneca

Switzerland

Hans Ulrich Burger, Hoffmann-La Roche
Marisa Bacchi, Actelion Pharmaceuticals

UK

Chrissie Fletcher, Amgen
Mark Morris, Conatus Pharma