



Welcome to

EFSPi's

5th Statistics Leaders Meeting

June 11, 2014
Roche facilities
Basel, Switzerland





Organization

5th EU Statistics Leaders Meeting

- Local Organization Thank You to Roche
 - for Hosting the Meeting
 - Sponsoring the Dinner

- Organizing Committee
 - Stefan Driessen
 - Uli Burger
 - Chrissie Fletcher
 - Byron Jones
 - Jonathan Alsup

Welcome to Basel!





Welcome to Basel

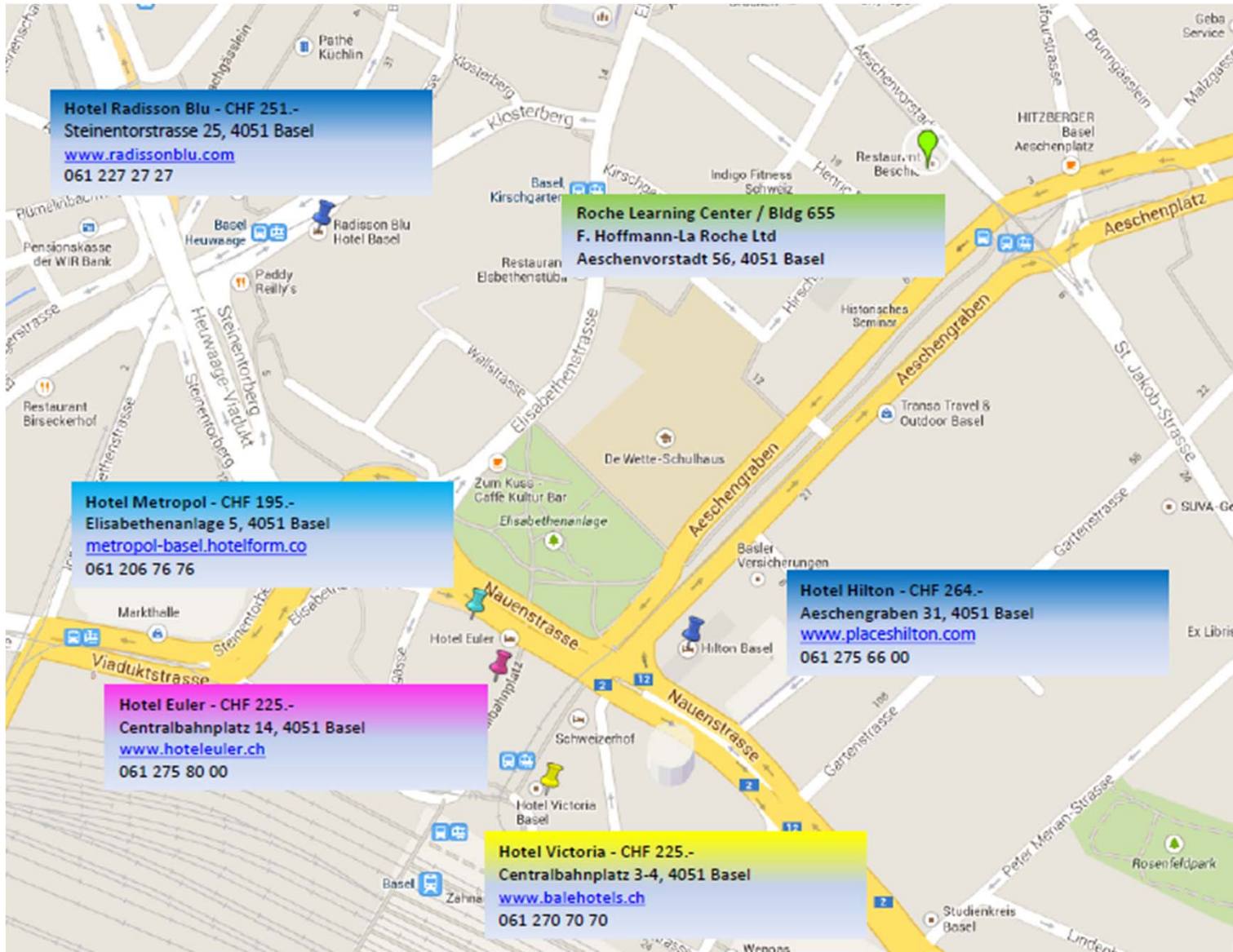
- Basel has a long history in Mathematics, statistics and pharma
 - 3 pharmaceutical companies headquartered here
 - Large biostatistics community (~250 statisticians), primarily in industry
 - No biostatistics department at University of Basel but mathematical institute with long lasting history

Welcome to Basel

- **Leonhard Euler**
(15.4.1707, Basel-18. 9.1783 St Petersburg)
- **Jakob Bernoulli**
(6.1.1655, Basel-16.8.1705, Basel)
Father of the Bernoulli distribution and of calculus
- **Johann Bernoulli** (brother of Jakob)
(6.8.1667, Basel-1.1.1748, Basel)
Followed his brother in Basel and friend of Leibnitz
- **Daniel Bernoulli** (son of Johann)
(8.2.1700, Groningen-17.3.1782, Basel)
Gamma function, Bernoulli effect, Friend of Leonhard Euler



Location of the meeting in the center of Basel city





Statistical Leaders Meeting - aims

- Network and share ideas
- Shape and influence our environment
 - Education & Continuing Professional Development
 - Methodology Development & Identification of Best Practice
 - Regulatory and Industry policies
 - Effective working with differing resourcing models
- **Help to shape the strategy for EFSPi**

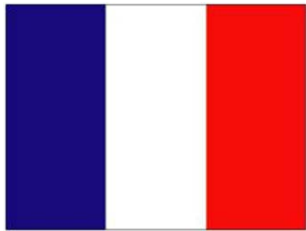


What is EFSPI?

- EFSPI = European Federation of Statisticians in the Pharmaceutical Industry
- Founded in 1992
- EFSPI is an “umbrella”, non-profit making organisation
- A federation of 10 National European Groups
- No individual members
- Our national organisations collectively represent ~ 2200 members



EFSPI - Our members



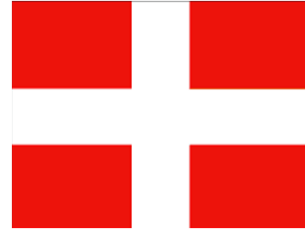
SFdS



SSL



APF



DSBS



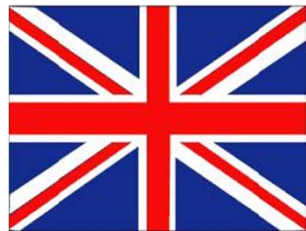
PSDM



BIAS



PSI



SBS/BVS



BBS



FMS



EFSPi Objectives

- To promote professional standards of statistics and the standing of the statistical profession in the pharmaceutical industry
- To offer a collective expert input on statistical matters to national and international authorities and organisations
- To exchange information on and harmonise attitudes to the practise of statistics in the European Pharma Industry and within member groups



EFSPI Strategic Objectives 2013-2015

1. Represent the association members of EFSPI and provide a united and respected voice on key scientific, regulatory and statistical issues in drug development

- Develop program of scientific meetings and partner with association members to provide opportunities to discuss, debate and align on key scientific, regulatory and statistical issues (responsible: Scientific Chair)
- Utilise EFSPI Statistics Leaders forum to discuss and align on emerging statistical areas and identify priorities and opportunities for EFSPI to lead/promote these areas to wider Industry bodies (responsible: Statistics Leader Chair)



EFSPI Strategic Objectives 2013-2015

2. Enhance the profile of EFSPI in Europe and strengthen alliances and collaborations with other professional bodies within Europe

Opportunities to engage with:

- the European Federation of Pharmaceutical Industry Associations (EFPIA) (responsible: President, Scientific Chair)
- the EMA Biostatistics Working Group and regulatory statisticians (responsible: Regulatory Chair)
- Associations important to the Special Interest Groups (SIGs), e.g. the International Society of Pharmacoeconomics and Outcomes Research (ISPOR) and the Health Technology Assessment (HTA) SIG (responsible: SIG Chair)

On the following key activities:

- ❖ Methodology/research and good research practice papers/reports
- ❖ Training opportunities
- ❖ Scientific meetings and discussing mutual areas of interest
- ❖ Development and review of regulatory and payer guidelines



EFSPI Strategic Objectives 2013-2015

3. Set and promote professional standards in Europe for the application, understanding and communication of statistics in drug development

- Write 1-2 professional position papers / best practice papers per year in collaboration with representatives from association members (responsible: Council representatives)
- Utilise SIGs to write best practice papers and/or manuscripts in their key topic areas (responsible: SIG Chair)



Key Goals for 2014

Strategic Objective 1 Represent the association members of EFSPi and provide a united and respected voice on key scientific, regulatory and statistical issues in drug development

- Scientific meetings – HTA and dose finding studies
- EFSPi Statistics Leaders meeting
- EMA transparency – working group
- Joint PSI/EFSPi regulatory committee – meeting with EMA Biostatistics working group
- Communications – newsletters, website
- Implement website upgrade



Data transparency working group

- Regulatory policy/guidance
- Analysis best practices
- Future impact to Biostatistics
- Minimal requirements for data sharing
- Ensuring patient confidentiality

Key Goals for 2014

Strategic Objective 2 Enhance the profile of EFSPI in Europe and strengthen alliances and collaborations with other professional bodies within Europe

- EFPIA – Clinical Development Committee & Clinical Trial Design Taskforce
- SIGs – support and develop partnerships, e.g. HTA and ISPOR

Strategic Objective 3 Set and promote professional standards in Europe for the application, understanding and communication of statistics in drug development

- Position papers – e.g. EMA transparency
- SIGs – support new areas & promote SIG publications

Recent SIG publications







- HTA SIG
 - *Adjusting overall survival for treatment switches: Commonly used methods and practical application*, [Watkins et al](#)
 - Utility values in health technology assessments: a statistician's perspective, [Whately-Smith et al](#)
 - Subgroup Analyses in Cost-Effectiveness Analyses to Support Health Technology Assessments, [Fletcher et al](#)



www.efspi.org



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

HOME	EFSPI is supporting the 2013 International Year of Statistics initiative - http://statistics2013.org - See below for more information .	
NEWS	European Statistical Meetings 2013: HTA Joint BBS/EFSPI Seminar June 4th 2013 Click here for Flyer - Click here to Register	
INFORMATION ON EF SPI	EFSPI/PSI Joint One Day Meeting on Structured Benefit-Risk Assessment September 17th 2013 Click here to register	
ADVERTISEMENT S	*** NEW *** EFSPI Position on access to clinical trial data – click here to view *** NEW ***	
PUBLICATION S	*** NEW *** EFSPI 3 Year Strategy – click here to view *** NEW ***	
CALENDAR OF EVENT S		
EF SPI ACTIVITIES		
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The World of Statistics



What is Statistics?

When many people hear the word "statistics," they think of either sports-related numbers or the college class they took and barely passed. While statistics can be thought about in these terms, there is more to the relationship between you and statistics than you probably imagine.

[Read More →](#)



The World of Statistics Activities

JUNE 2, 2014



Why Big Data is in Trouble: They Forgot About Applied Statistics

MAY 28, 2014
Stats Life »

This year the idea that statistics is important for big data has exploded into the popular media. First came the Lazer et. al paper, ["The parable of Google Flu: traps in big data analysis"](#), in *Science* that got the ball rolling on this idea. Followed by the *Financial Times* article, ["Big data are we making a big mistake?"](#). Then the *New York Times* published ["Google Flu Trends: the limits of big data"](#) and ["Eight \(No, Nine!\) Problems with Big Data"](#).

[Read More →](#)

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Why Statistics is Important to You



<http://www.worldofstatistics.org/>



Organization

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Statistics Leaders Forum

- At present:
 - 55 on mailing list
 - 43 Active members
 - 10 (all) EFSPi countries
 - 22 Pharma companies
 - 7 CROs
- All material from the EU Statistics Leaders Meetings held in 2010, 2011, 2012, and 2013 are available on the EFSPi website:

<http://www.efspi.org/index.php?p=EFSPi%20activities&fid=399>



2013 Annual Report

- **Statistics Leaders Forum call – February 2013**
- **EFSPi Position on Clinical Trial Data Transparency – March, April 2013**
- **EFSPi Statistics Leaders Meeting – June 2013**
 - Survey on Meeting – July 2013
- Regular updates to EFSPi Council and Operations Board



5th Statistics Leaders Meeting

Year	Venue	Host	# attendees
2014	Basel	<i>Roche</i>	30
2013	Copenhagen	<i>Novo Nordisk</i>	23
2012	Amsterdam	<i>Abbott</i>	26
2011	London	<i>Amgen</i>	22
2010	Berlin	<i>Bayer</i>	26



Recap 4th Stats Leaders Mtg

Main agenda items:

- SIG Medical Devices
- SIG Benefit/Risk
- Clinical Trial Data Transparency workshop

- Minutes & Presentations Meeting 2013 on our website:
 - [http://www.efspi.org/index.php?p=EFSPI activities&fid=432](http://www.efspi.org/index.php?p=EFSPI%20activities&fid=432)



SIG Medical Devices

Stats Leaders Mtg 2013 – request to EFSPi:

- Contribute to comments on upcoming regulatory guidelines changes
- Backing up SIG in promoting good scientific practice as in pharma

Follow-up:

- EFSPi supported SIG in sending a letter to EU Parliament asking for attention to introduce more profound statistical principles in upcoming regulatory framework changes than presently proposed
 - no answer yet from EU deputies
 - Some of SIGs concerns taken into account
 - Yet current regulatory thinking also different from some expectations
 - Role of EMA is not as direct than expected
 - Post-market device surveillance is re-inforced
 - Notified bodies (which are delivering the CE mark) will be less and better controlled.



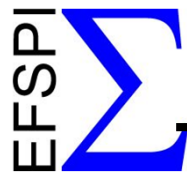
SIG Benefit Risk

- Stats Leaders Meeting 2013 :
 - SIG **can use EFSPi/PSI website** for posting of material to be kept under SIG's control such as Position Papers, Presentations, etc.
 - SIG free to investigate more wiki-like environments for triggering more feedback (but without control)
 - Encouraged to collaborate with **QSPI** (US analogy to EFSPi), but not let that hold up development blue print



SIG B/R follow up

- SIG had EFSPi/PSI one day meeting in September 2013
 - <http://www.efspi.org/index.php?p=EFSPi%20ACTIVITIES&fid=431>
- Submission publication:
 - *“Structured Benefit-risk assessment: A review of key publications and initiatives on frameworks and methodologies”*
- Regular meetings in subgroups
- Involvement in PROTECT
- Collaboration with QSPI:
 - intent to publish a newsletter together with QSPI related to B-R topics
- More information on website:
 - <http://www.psiweb.org/index.php?p=resources&fid=1517#>
- **Planned 2-days Training in 2015**
 - SIG is looking for venue (1 large room, 2 break outs)
 - please let Alexander Schacht know: schacht_alexander@lilly.com



Messages on Clinical Trial Data Transparency – Stats Meeting 2013

- Statisticians (we) should focus more on quality and statistical principles than trying to increase trust
- Statisticians should take independent, balanced view on publication/re-analysis remit, and we should **focus on key statistical elements and highlight good statistical practice**
- We should be open for collaboration and share our views with other stakeholders
- **One industry “solution”** would be best for Industry and EFSPI
 - Same principles on access and data anonymisation
 - Development of best practices on secondary (re-)analyses
- No matter which solution (unlimited access or restricted access) the workload will increase for statisticians
- This area is new to our community and will bring challenges and opportunities

Agenda - morning

Time	Topic	Presenter / Facilitator
9:00 - 9:30	<ul style="list-style-type: none"> • Welcome address + Intro <ul style="list-style-type: none"> ○ Org. Ctee ○ EFSPi President ○ Recap Stats Leaders meeting 2013 	Stefan Driessen + Hans Ulrich Burger Chrissie Fletcher Stefan Driessen
9:30 - 11:00	<ul style="list-style-type: none"> • Clinical Trial Data Transparency <ul style="list-style-type: none"> ○ Update by Working Group ○ EMA - Latest Developments ○ Practicalities of Data Sharing 	Hans Ulrich Burger Christoph Gerlinger Rebecca Sudlow
11:00-11:30	<i>Break</i>	
11:30 – 12:15	<ul style="list-style-type: none"> • SIGs – Update • SIG Integrated Data Analysis <ul style="list-style-type: none"> ○ Introduction + Discussion 	Stefan Driessen Byron Jones
12:15 - 13:00	<ul style="list-style-type: none"> • SIG Pharmaco-Epidemiology <ul style="list-style-type: none"> ○ Introduction + Discussion 	Jonathan Alsop
13:00-14:00	<i>Lunch break</i>	

Agenda - afternoon

Time	Topic	Presenter / Facilitator
14:00 - 15:00	<ul style="list-style-type: none"> • Recent Data & Design Developments: <ul style="list-style-type: none"> ○ IMI: GetReal, IMI2 ○ EFPIA: New Clinical Trial Design Task Force ○ EMAs Parallel Scientific Advice Regulators + HTA ○ EMA PAES Working Groups ○ Big Data – Real World Data 	Round Table Discussion: Chrissie Fletcher Stefan Driessen Uli Burger
15:00-15:15	<i>Break</i>	
15:15 – 16.15	<ul style="list-style-type: none"> • Recent Developments - continued 	
16:15 – 16.30	<ul style="list-style-type: none"> • Meeting summary 	Stefan Driessen
16:30	<i>Meeting adjourn</i>	



Objectives 2014 meeting

- Be informed and Give feedback on
 - Clinical Trial Data Transparency Working Group
 - SIG Integrated Data Analysis
 - SIG Pharmaco-Epidemiology
 - Recent Data & Design Developments
- Help EFSPi define strategy on these recent developments
 - preferred priorities for EFSPi
 - level of involvement of EFSPi
 - focus of EFSPi