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

June 11, 2014, Basel  5th EU Statistics Leaders Meeting
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
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)
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
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
2014+ World of Statistics

http://www.worldofstatistics.org/

June 11, 2014, Basel

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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:
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
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<th>Year</th>
<th>Venue</th>
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<th># attendees</th>
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<td>2014</td>
<td>Basel</td>
<td><em>Roche</em></td>
<td>30</td>
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<tr>
<td>2013</td>
<td>Copenhagen</td>
<td><em>Novo Nordisk</em></td>
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<td>2012</td>
<td>Amsterdam</td>
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<td>2011</td>
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<td>2010</td>
<td>Berlin</td>
<td><em>Bayer</em></td>
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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:
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 EFSPAN/PSI one day meeting in September 2013

• 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:

• 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

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<th>Time</th>
<th>Topic</th>
<th>Presenter / Facilitator</th>
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<tr>
<td>9:00 - 9:30</td>
<td>• Welcome address + Intro</td>
<td>Stefan Driessen + Hans Ulrich Burger</td>
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<tr>
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<td>o Org. Ctee</td>
<td>Chrissie Fletcher</td>
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<td>o EFSPPI President</td>
<td>Stefan Driessen</td>
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<td></td>
<td>o Recap Stats Leaders meeting 2013</td>
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<tr>
<td>9:30 - 11:00</td>
<td>• Clinical Trial Data Transparency</td>
<td>Hans Ulrich Burger</td>
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<td>o Update by Working Group</td>
<td>Christoph Gerlinger</td>
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<td>o EMA - Latest Developments</td>
<td>Rebecca Sudlow</td>
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<td>o Practicalities of Data Sharing</td>
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<td>11:00-11:30</td>
<td><strong>Break</strong></td>
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<td>11:30 – 12:15</td>
<td>• SIGs – Update</td>
<td>Stefan Driessen</td>
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<td>• SIG Integrated Data Analysis</td>
<td>Byron Jones</td>
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<td>o Introduction + Discussion</td>
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<td>12:15 - 13:00</td>
<td>• SIG Pharmaco-Epidemiology</td>
<td>Jonathan Alsop</td>
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<td>o Introduction + Discussion</td>
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<td>13:00-14:00</td>
<td><strong>Lunch break</strong></td>
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## Agenda - afternoon

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| 14:00 - 15:00 | **Recent Data & Design Developments:**  
  - 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 | *Break*                                                             |                                             |
| 15:15 – 16.15 | **Recent Developments - continued**                                 |                                             |
| 16:15 – 16.30 | **Meeting summary**                                                | Stefan Driessen                             |
| 16:30       | *Meeting adjourn*                                                   |                                             |
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 EFPSI
  – level of involvement of EFSPi
  – focus of EFSPi