Welcome to

EFSPI's

4th Statistical Leaders Meeting

June 18, 2013
Novo Nordisk facilities
Copenhagen, Denmark

EFSPI's

4th Statistical Leaders Meeting

Copenhagen June 18 2013

VP Niels Kamp

Novo Nordisk at a glance

Novo Nordisk is a global healthcare company with 90 years of innovation and leadership within:

- diabetes care
- insulin
- injection devices

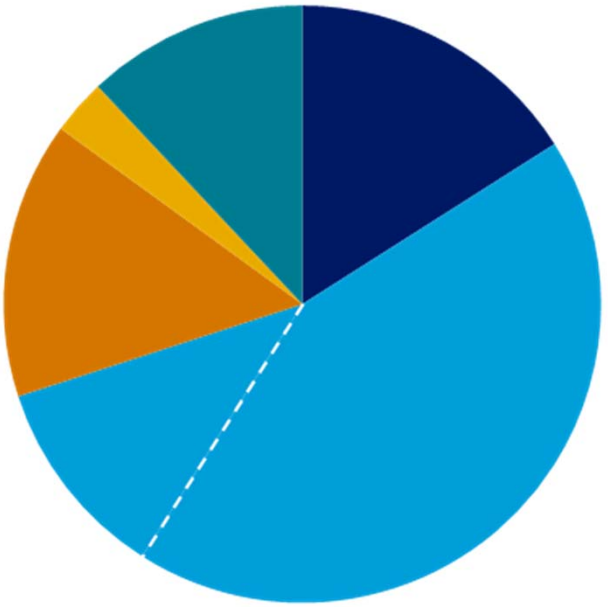
Thanks to dedicated research into proteins, Novo Nordisk also holds leading positions within:

- haemostasis management
- growth hormone therapy
- hormone replacement therapy

More than 35,000 employees around the world

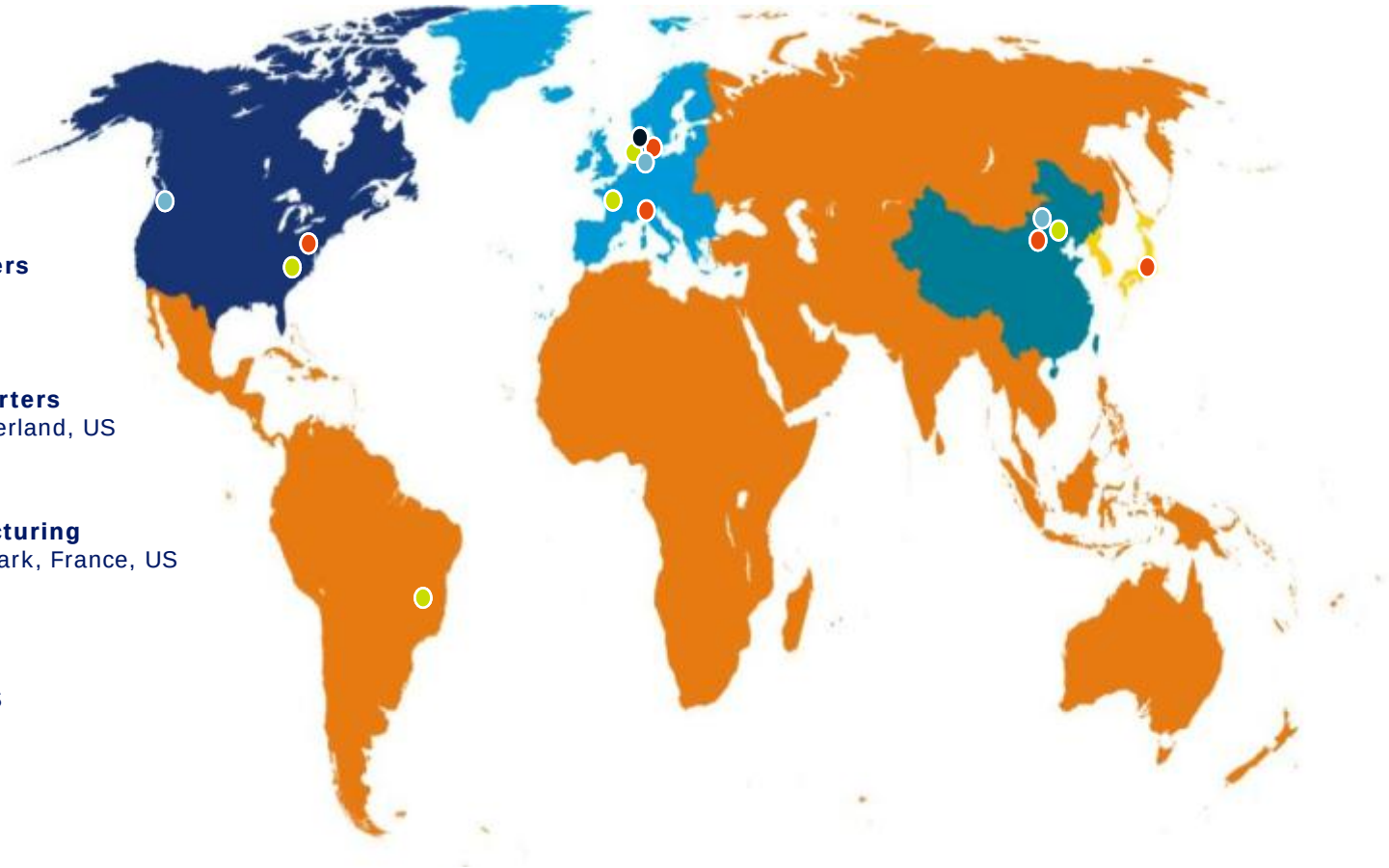
Employees by region

- **North America 16%**
- **Europe 54%**
  of which 43% are employed in Denmark[1]
- **International Operations 15%**
- **Japan & Korea 3%**
- **Region China 12%**



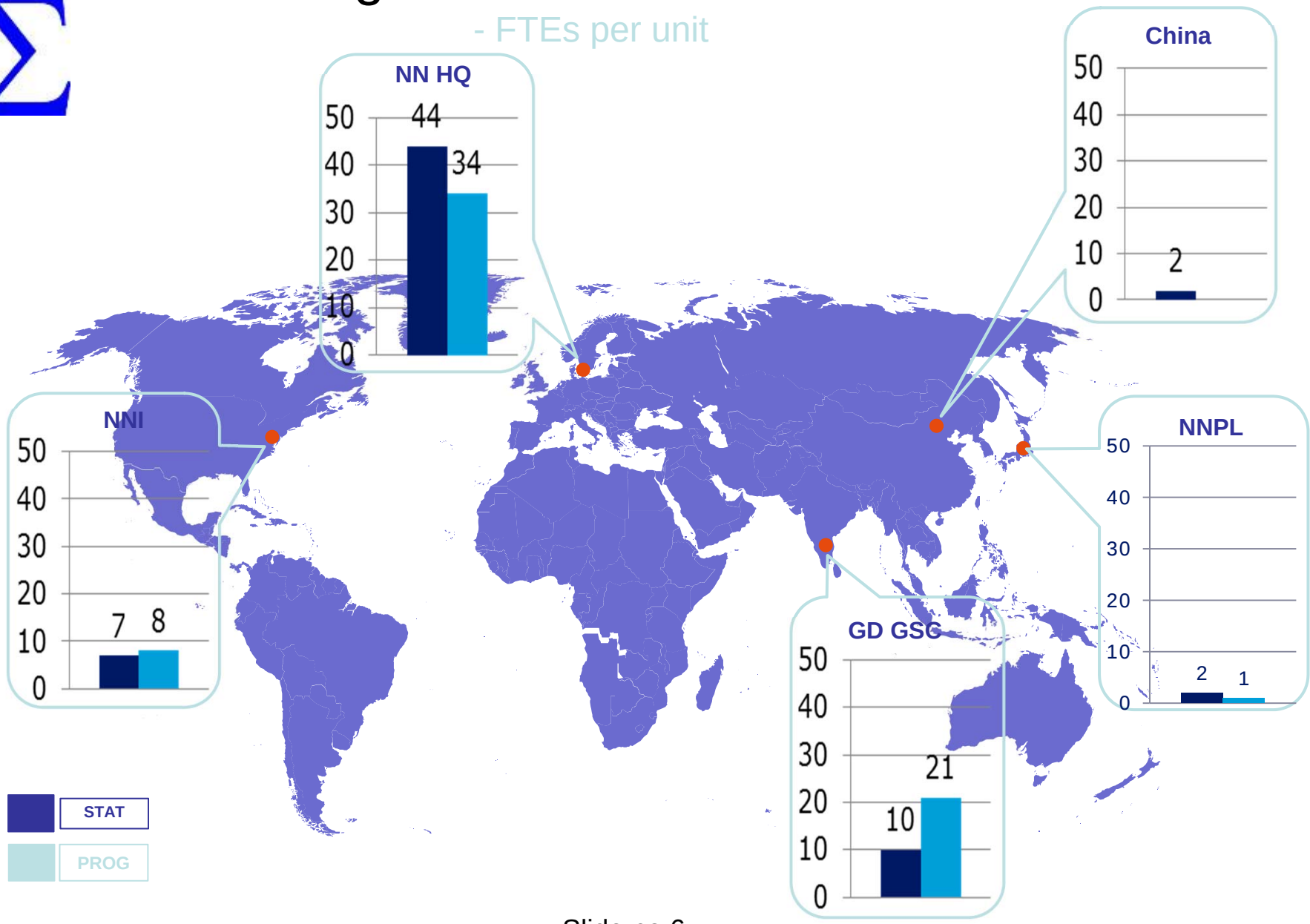
Our global presence

- **Global headquarters**
  Denmark
- **Regional headquarters**
  China, Japan, Switzerland, US
- **Strategic manufacturing**
  Brazil, China, Denmark, France, US
- **R&D facilities**
  China, Denmark, US



Global organisation Biostatistics 2013

- FTEs per unit



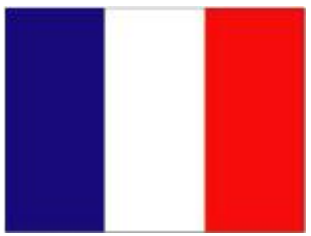
Statistical Leaders Meeting

- Initiative from EFSPI
- Develop a forum for Statistical Leaders to
  - 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?

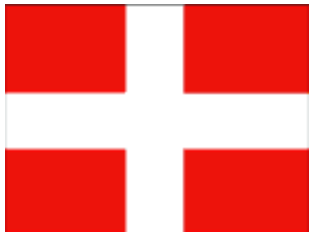
- EFSPI = European Federation of Statisticians in the Pharmaceutical Industry
- Founded in 1992
- EFSPI is an “umbrella”, non-profit making organisation
- A federation of National European Groups
- Now have 10 national groups
- No individual members
- Our national organisations collectively represent ~ 2800 members

EFSPI - Our members

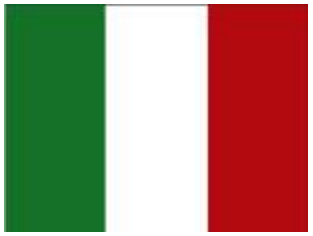

SFdS


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 2013

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, benefit-risk, survival + webinar
- EFSPI Statistics Leaders meeting
- EMA transparency – advisory groups, review draft policy, discussion forum(s)
- Communications – newsletters, website
- Develop website strategy

Key Goals for 2013

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
- EMA Biostatistics Working Group – engage in development of regulatory guidance
- 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. career development, EMA transparency
- SIGs – support new areas, e.g. meta-analysis, promote SIG publications

What we can achieve?

- Successfully supported PSI in lobbying EU Regulators to increase number of statisticians employed by the EU Regulatory Agencies

- Heads of Agencies listened and then commissioned a Strategy Paper to investigate the matter further

OPEN LETTER

European regulatory agencies should employ full time statisticians

Sara Hughes, chair of PSI (professional UK body of statisticians in the pharmaceutical industry) and director of statistics, GlaxoSmithKline chair@psiweb.org
Nigel Howitt, EFSPI president (European Federation of Statisticians in the Pharmaceutical Industry) and director of analysis and reporting, PRA International;
Kit Roes, EFSPI vice president and vice president, Global Clinical Information, Organon Scherin Plough;

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VIEWPOINT

Pharmaceutical Statistics

Received 1 December 2009, Accepted 19 December 2009 Published online 8 February 2010 in Online Library (wileyonlinelibrary.com) DOI: 10.1002/pst.416

Statistical resource needs to be increased in the European regulatory agencies

Oliver N. Keene,[a] Sara H. Hughes,[a]* Nigel Howitt,[b] on behalf of the PSI and EFSPI Boards of Directors

The following viewpoint from PSI and EFSPI regarding the current level of statistical resource in the European regulatory agencies was first presented as a position paper to a meeting of the EU Heads of Agencies in July 2009, and was endorsed by EFPIA. Copyright © 2010 John Wiley & Sons, Ltd.

PHARMACEUTICAL STATISTICS
Pharmaceut. Statist. (2009)
Published online in Wiley InterScience
(www.interscience.wiley.com) DOI: 10.1002/pst.367

Statisticians in European regulatory agencies

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What we can achieve?

Main Paper

Pharmaceutical Statistics

(wileyonlinelibrary.com) DOI: 10.1002/pst.508

Published online in Wiley Online Library

The draft FDA guideline on non-inferiority clinical trials: a critical review from European pharmaceutical industry statisticians

Bernhard Huitfeldt,[a]* and Jürgen Hummel,[b] on behalf of European Federation of Statisticians in the Pharmaceutical Industry (EFSPI)

The European Federation of Statisticians in the Pharmaceutical Industry (EFSPI) engages more than 2000 statisticians through its ten national organizations. Amongst other things, EFSPI is involved in reviewing regulatory guidelines under development, including the draft FDA guideline on non-inferiority clinical trials. This review resulted in several critical comments relating to as follows: (i) the lack of one single standard for proving efficacy of new drugs implied by the guideline; (ii) the problems with the suggested 'fraction of effect to be preserved'; (iii) the formulation of the primary hypothesis in a non-inferiority trial aiming at indirectly demonstrating a new drug is superior to placebo; and (iv) the preference in the guideline for the fixed-margin method over the synthesis method in the analysis. The presumed implications of this guideline, if implemented as is, are (i) increased confusion of how efficacy could be demonstrated when placebo control is not available, (ii) more complicated communication between pharmaceutical industry and FDA because of the apparent disagreements on fundamental statistical matters, and (iii) illogical consequences in the approval process because of which order drugs are approved rather than how they fulfill the regulatory requirements. We believe that the area is not yet ready for such a prescriptive regulatory guidance and that further research and experience are required until the methodology can be finally agreed. A strategy needs to be developed by regulatory agencies together with drug industry and academia for a long term solution for this topic. **Copyright © 2011 John Wiley & Sons, Ltd.**

Keywords: FDA guideline; non-inferiority; synthesis method; fixed-margin method; EFSPI

September 2011

What we can achieve?

European Federation of Statisticians in the Pharmaceutical Industry

Date: April 25, 2013

European Federation of Statisticians in the Pharmaceutical Industry (EFSPI) Position on European Medicines Agency (EMA) Access to Clinical Trial Data Initiative

Executive Summary

EFSPI supports the EMA policy for transparency and is committed to contribute to EMA's access to clinical trial data initiative. EFSPI was instrumental in the

www.efspi.org

EFSPI

!!! 2013 !!!

2013 Year of Statistics

http://www.statistics2013.org/

2013 Year of Statistics

- EFSPI is participating

Participants

Professional societies

- (Australia) Statistical Society of Australia
- (Austria) Austrian Statistical Society
- (Belgium) Belgian Statistical Society
- (Botswana) Botswana Statistical Association
- (Brazil) Brazilian Statistical Association
- (Canada) Southern Ontario Regional Association of the Statistical Society of Canada
- (Canada) Statistical Society of Canada
- (Congo) Association des Statisticiens du Congo (DRC), ASTACO
- (Croatia) Teachers Association "Suradnici u ucenju"
- (Czech Republic) Czech Statistical Society
- (Denmark) Danish Society of Theoretical Statistics
- (Estonia) Estonian Statistical Society
- (Estonia) Statistics Estonia
- (Ethiopia) Ethiopian Statistical Association
- (Europe) European Federation of Statisticians in the Pharmaceutical Industry (EFSPI)

- Updates in monthly newsletters

Again Welcome

- 4th European Statistical Leaders Meeting
- Thank you to organising team
  - Stefan Driessen
  - Egbert Biesheuvel
  - Uli Burger
  - Ian Hirsch
  - Marie Gøthberg
- Thank you to Novo Nordisk
  - for Hosting the Meeting
  - Sponsoring the Dinner

4th Statistical Leaders Meeting

Year	Venue	Host	# attendees
2013	Copenhagen	Novo Nordisk	23
2012	Amsterdam	Abbott	26
2011	London	Amgen	22
2010	Berlin	Bayer	26

Composition
Stats Leaders Forum

At present:

- Members
  - 54 listed
    - 11 on list did not attend any of the three mtgs
  - 43 on list that at least attended one meeting
    - all ten (10) EFSPI countries represented
    - 33 from 21 pharma companies
    - 8 members from 6 CROs
    - 2 from Academia

Statistical Leaders Meeting

Annual Report 2012:

3rd Statistical Leaders Meeting, Weesp, The Netherlands

- 26 attendees
- Agenda:
  - Overview SIGs
  - SIG Benefit/Risk
  - Career Path
  - Adaptive Statisticians
  - Strategic Workshop: Do's and Don'ts stats community EU Pharma
- Minutes & Presentations:
  - http://www.efspi.org/index.php?p=EFSPI activities&fid=410

Actions/progress from 3rd Stats Leaders Mtg 2012

Career Path:

- Major revision to be (re-)submitted to Pharmaceutical Statistics
- Survey was taken out and will be made available on website

SIGs:

- Add SIG Medical Devices to Stats Leaders Forum
  - Roland Marion-Gaulois added to member list
  - SIG Medical Devices on agenda Stats Leaders Mtg 2013
- No formal criteria for setting up a SIG
  - Initiative to set up new SIG on Meta-Analyses
  - Will be discussed in this meeting and EFSSPI Council mtg tomorrow
- Statistical Leaders and EFSPI to promote and support SIG Benefit/Risk
  - Addition of members to SIG
  - Help define charter
  - Support set up website
  - Determined as area of success (outcome strategic workshop)

Strategy Workshop

NXT generation statistician

- *Goal: new graduates have better understanding of the whole drug development process and areas of statistical influence*

Actions:

- EFSPI to contribute to or organize training program
- EFSPI to contact Universities to get this into their curriculum

Strategy Workshop

What differentiates EU from the rest

- *Goal: maintain collective expertise and strong link between regulators, industry and cooperative group and promote advantages of EU*

Actions:

- EFSPI to further links to other EU organizations
  - regulators, payer organizations, academia
- EFSPI to further organize meetings with these stakeholders in EU

Strategy Workshop

Change Management

- *Goal: increase awareness and need for change, thereby identifying the skill set needed for statisticians and leaders*

Actions:

- Statistical Leaders/EFSPI to set up Working Group to
  - define vision, perform benchmarking and gap analysis
  - develop, pilot, and share “support package” that will enable statistical leaders to drive change in companies
- Develop and communicate best practices, within and across companies

Strategy Workshop

Areas of Success now

Goal:

- *Educate statisticians in area of Benefit/Risk where there is a need for quantitative valid approaches to summarize, present and communicate results across various domains to come to an overall evaluation.*

- *To enable statisticians to add value beyond being a "number cruncher" and take a more strategic role while still utilizing their quantitative background*

Strategy Workshop

Areas of Success now

Actions Statistical Leaders:

- Get (more) acquainted with regulatory background and framework of Benefit/Risk (not only the techniques)
- Start projects/pilots on Benefit/Risk within your company, and encourage your statisticians to also concentrate on graphical presentation and sensitivity analyses
- Share EMA pilots on this area and best practices with other companies
- Develop experts within your company

Actions EFSPI:

- To request from and support SIG with developing a B/R toolkit and a plan/process to support companies to take this on.
- Organize more meetings/workshops to get statisticians exposed to topic.

Statistical Leaders Meeting

Report 2013:

- Telecon: February 13, 2013
  - 20 attendees
  - Recap Stats Leaders Mtg 2012
  - Request for items for Stats Leaders Mtg 2013
  - Discussion about possible registration fee
    - denied
  - EMA Clinical Trial Transparency
    - Status update by EFSPI representatives to Forum
    - Lively discussion
    - Defined as major topic for Stats Leaders mtg

- 4th Statistical Leaders Meeting

Agenda

Time	Topic	Presenter / Facilitator
9:00 - 9:15	• Welcome • EFSPI President Welcome address	Stefan Driessen / Niels Kamp Chrissie Fletcher
9:15 - 9:45	• EFSPI Strategic Objectives 2013-2015	Chrissie Fletcher
9:45 – 10:00	• SIGs - Update	Stefan Driessen
10:00 – 10:30	• SIG Medical Devices - Introduction	Roland Marion-Gallois
10:30-11:00	Break	
11:00 - 12:00	• SIG Benefit Risk – Progress on Roadmap	Ian Hirsch
12:00-13:00	Lunch break	
13:00 - 16:15	• EMA Clinical Trial Transparency	All
16:15 – 16:30	• Meeting summary	Stefan Driessen
16:30	Meeting adjourn	

Objectives 2013 meeting

- Be informed and give feedback on
  - EFSPI Strategy for coming 2-3 years
  - SIGs
    - Introduce “new” one: Medical devices
  - SIG Benefit Risk
    - Progress on development blue print as requested by Stats Leaders Mtg 2012
- Help EFSPI define strategy on EMA Transparency

EMA Clinical Trial Data Transparency

- EMA timelines