EFSPI

European Federation of Statisticians in the Pharmaceutical Industry

Promoting Professional Standards in Europe

Roche

Webinar 1: Data monitoring committees - evolving their role in a changing drug development landscape

5th EFSPI regulatory statistics workshop
12th October 2020
Kaspar Rufibach, on behalf of EFSPI and the organizing committee

We have put you on mute.

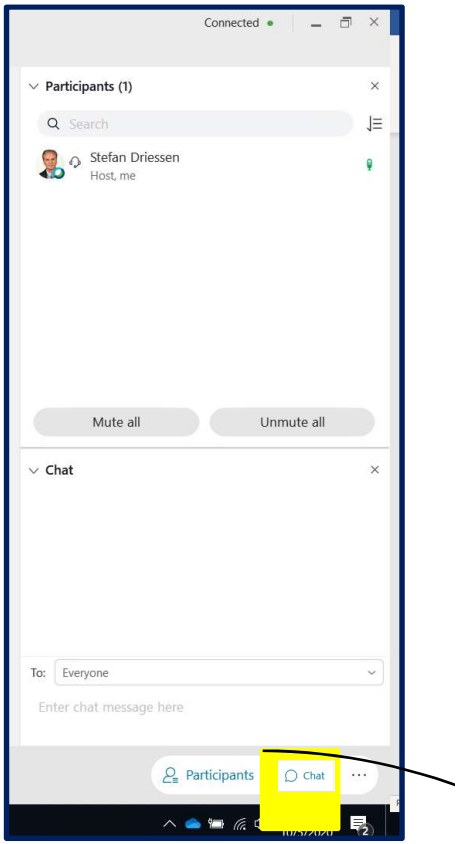
We are waiting for other attendees to join.

We will start soon

Logistics

- We have put all attendees (except for the speakers) on mute
- We will have about <791> participants
- You can send queries through the Chat function:
  - wrt presentation at hand (e.g., clarification), or
  - for panel discussion
- As follow-up all queries will be answered by the presenter if not already addressed during the meeting (Q&A doc)
- Hardly no time for questions between the presentations

- *<All speakers>* have agreed to sharing slides, these will be available at the EFSPI website in due time
- *<All speakers>* have agreed to live recording, also this will be available at the EFSPI website in due time



5th EFSPI regulatory statistics workshop 12th October 2020

- Organization: EFSPI and organizing committees THANKS!
- Host: Roche (in particular Kaspar Rufibach) THANKS!

- Tomorrow Tuesday 13th October 2020 - Webinar 2:
  Estimands – emerging questions now that we are using the framework
- Registration link:
  https://docs.google.com/forms/d/e/1FAIpQLSdlqa1_T9lqMboJwOO3vAHrhp9TbNKKLX4AO2beFgwVfBtdGA/viewform

Organizing committees

- Local organizing committee

Egbert Biesheuvel (Danone)

Hans Ulrich Burger (Roche)

Christoph Gerlinger (Bayer)

Kaspar Rufibach (Roche)

Emmanuel Zuber (Novartis)

- Scientific committee

Andreas Brandt (BfArM)

Randi Gron (Novo Nordisk)

Maria Gruenewald (Swedish Medical Products Agency)

Cecilia Hedlund (Swedish Medical Products Agency)

Lorenzo Hess (Swissmedic)

Benjamin Hofner (Paul-Ehrlich Institute)

Armin Koch (Medizinische Hochschule Hannover)

Khadija Rantell (MHRA)

Kit Roes (Radboud UMC)

Ina-Christine Rondak (EMA)

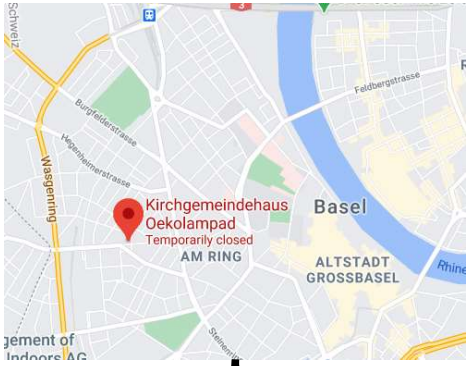
Aldana Rosso (Danish Medicines Agency)

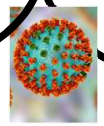
Anja Schiel (Norwegian Medicine Agency)

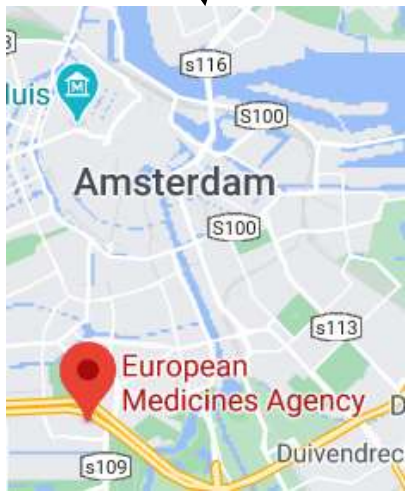
Steven Teerenstra (Radboud UMC)

5th EFSPI regulatory statistics workshop

- Jubilee
- After 4 successful years in Basel
- 5th to take place in Amsterdam
- New place of EMA
- However,
- COVID-19
- got in the way



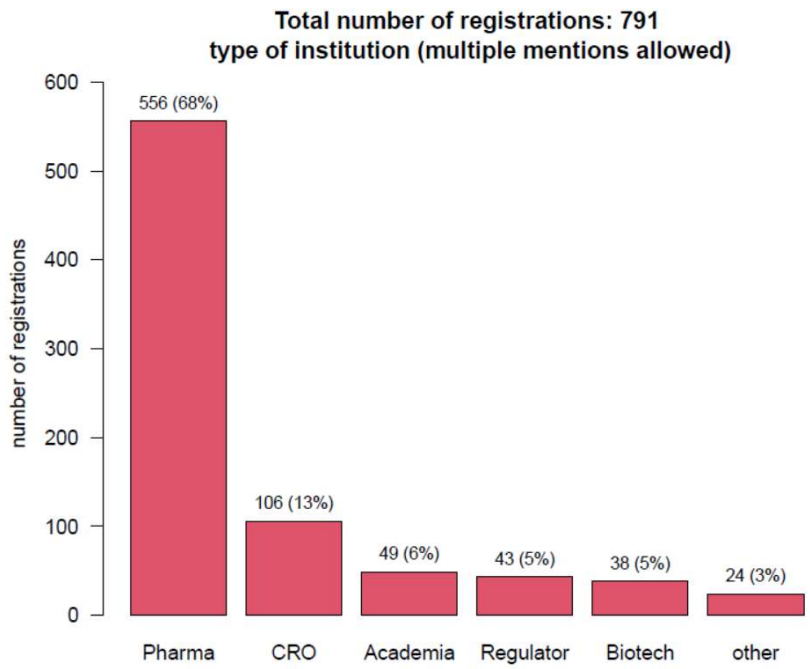


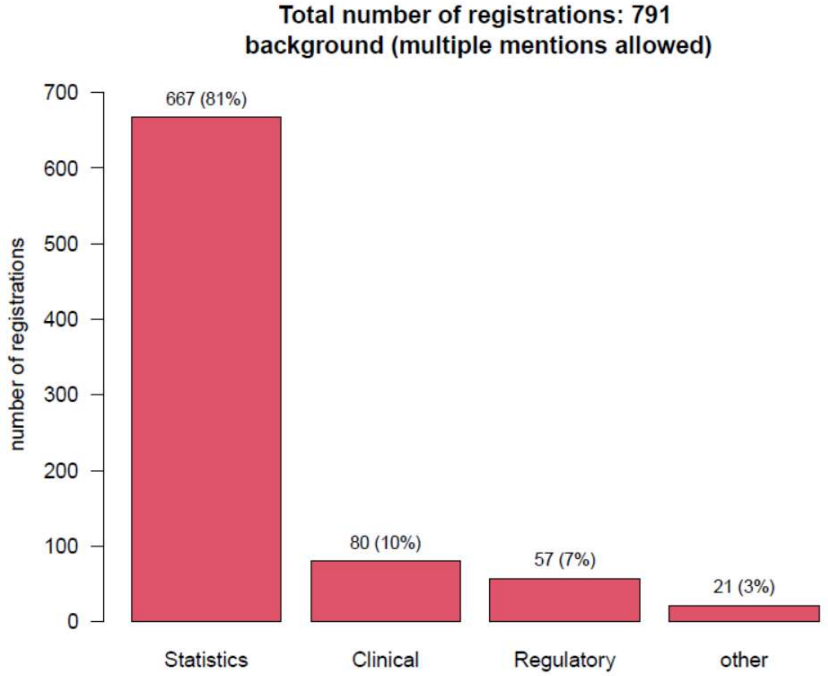


- *However*

Increased number participants

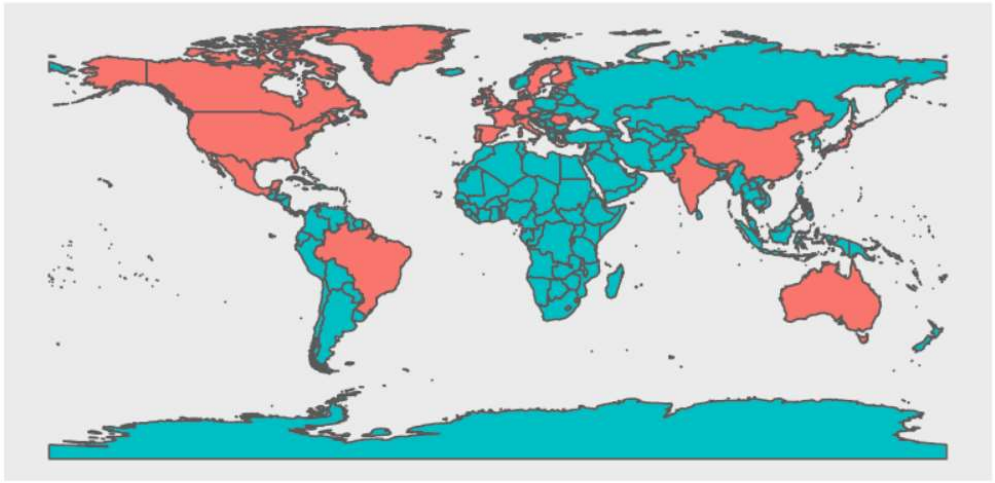
from 200+ live workshop to ≈ 800 people in webinar

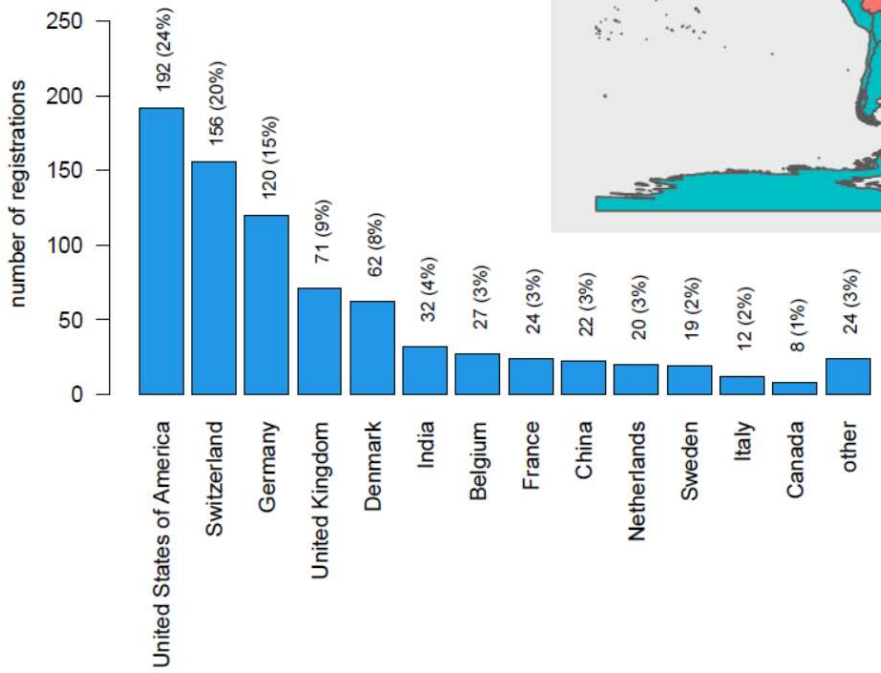




Increased worldwide coverage

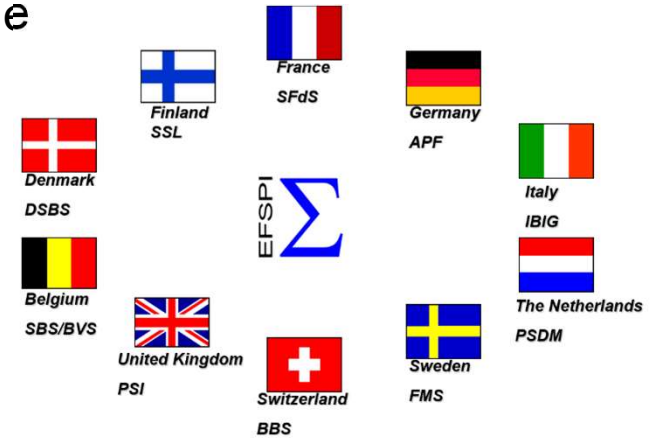
from mainly EU live workshop to “across the globe” webinar





What is EFSPI?

- EFSPI = European Federation of Statisticians in the Pharmaceutical Industry
- Founded in 1992
- A federation of National European Groups
- Now have 10 national groups
- No individual members
- EFSPI is an “umbrella”, non-profit making organisation
- Our national organisations collectively represent 2200 members
- Each organisation has 2 members on the EFSPI Council



- Website: www.efspi.org

What is EFSPI not?

- EFSPI is not EFPIA, or part of EFPIA

- EFPIA = European Federation of Pharmaceutical Industries and Associations

- EFSPI works together with EFPIA
  - Commenting of guidelines such as the Points-to-Consider on implications of Coronavirus disease (COVID-19) on methodological aspects of ongoing clinical trials (EMA/158330/2020)

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 Activities

- Regulatory Affairs (joint committee with PSI)
- Organise annual EFSPI Regulatory Statistics workshop
  - Co-ordinates review of regulatory guidance within EU Statistical Community
  - Meet with EU Biostatistics Working Party (BSWG) annually to discuss hot topics
  - Meet with other statistical agencies, e.g. MHRA
  - Hold workshops to discuss draft guidance where appropriate
  - Works with Scientific to identify topics for scientific debate
  - EFSPI is recognized official body in EMA database

Data Monitoring Committee (DMC) Guidelines

- EMA (Sep, 2020): Q&A on DMC issues ***NEW!***
  - https://www.ema.europa.eu/en/documents/scientific-guideline/questions-answers-data-monitoring-committees-issues_en.pdf

- China NMPA (Sep, 2020): Guidelines for Drug Clinical Trial DMC (Interim) ***NEW!***

- EMA (2006): Guideline on DMCs
  - https://www.ema.europa.eu/en/documents/scientific-guideline/guideline-data-monitoring-committees_en.pdf
- USFDA (2006): Establishment and Operation of Clinical Trial DMCs
  - https://www.fda.gov/regulatory-information/search-fda-guidance-documents/establishment-and-operation-clinical-trial-data-monitoring-committees
- Japan PMDA (2013): Guideline on DMC
  - https://www.pmda.go.jp/files/000232300.pdf

Agenda – Webinar 1

12th October 2020 - Webinar 1: Data monitoring committees – evolving their role in a changing drug development landscape

Registration link: https://docs.google.com/forms/d/e/1FAIpQLScH2TNYpB6Z0IgXzZ_386oT-igMtUNZ1Qgfta3mBLa6MspZDg/viewform

14:00 – 14:10 **Stefan Driessen (EFSPI president)**
Welcome and scene setting

14:10 – 14:30 **Steven Teerenstra (Radboudumc & EMA Biostatistics Working Party)**
The new EMA Q&A document on data monitoring committees

14:30 – 14:45 **Paul Gallo (Novartis)**
An example of a difficult interim decision – what should be the role of the iDMC in ensuring trial integrity?

14:45 – 15:00 **Lisa Squassante (Roche)**
Use of futility interim boundaries to inform production or portfolio decisions

15:00 – 15:15 **Break**

15:15 – 15:30 **Thomas Jaki (MRC Cambridge Biostatistics Unit)**
Role of iDMC for complex clinical trials, incorporating portfolio decisions

15:30 – 15:45 **Janet Wittes (Statistics Collaborative Inc.)**
Navigating between sponsor, iDMC, and regulators

15:45 – 16:00 **Hans Ulrich Burger (Roche)**
iDMC in the context of adaptive designs

16:00 – 16:30 **Panel discussion (all speakers + Tom Fleming, University of Washington)**
All speakers

16:30 – 16:35 **Hans Ulrich Burger (EFSPI council member and "local" organizing committee)**
Closure

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BREAK!

We will start soon again.

Back Up

Potential session: data monitoring committees

- How does industry need to re-organize internal processes as a response?
- iDMC members:
  - What do they expect from authorities?
  - What do they expect from sponsors?
  - How much involved in portfolio decisions does an iDMC want to / can be?
- Connection of (i)DMCs / interim analyses to complex innovative designs.
- How to handle updates to trials based on «external» or «internal» information?
- When SAP is finalized, often info has leaked from trial (complex designs, interims, dry runs, etc.). How to prevent that?