EFSPI

European Federation of Statisticians in the Pharmaceutical Industry

Promoting Professional Standards in Europe

WELCOME

to the 6th EFSPI Regulatory Statistics Workshop 2021

Webinar 3:
Real-world data and short topics session

We have put you on mute
We are waiting for other attendees to join
We will start soon

6th EFSPI Regulatory Statistics Workshop 13th – 15th September 2021

- Three webinar sessions devoted to:

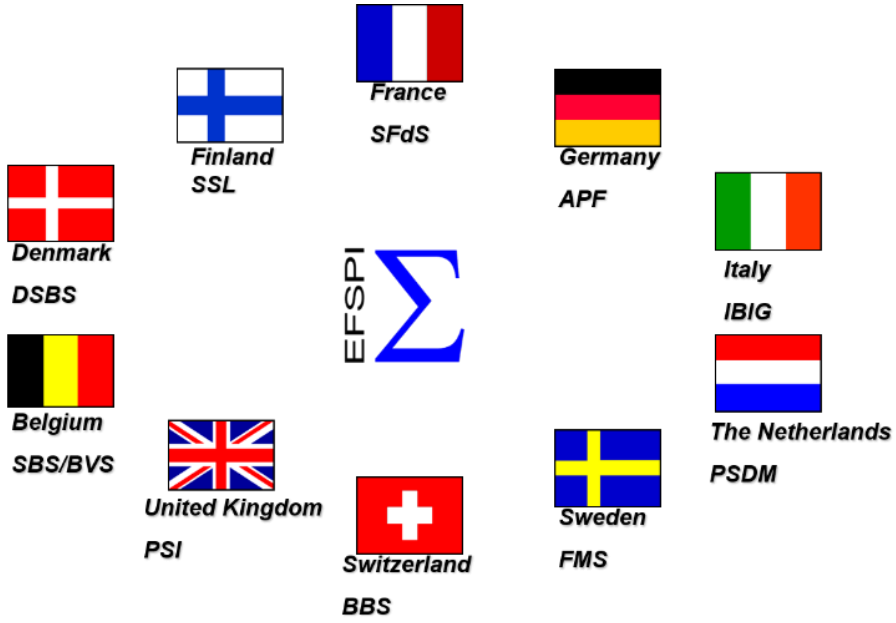
Monday	1. Decentralized trials: What is the impact on evidence generation?
Yesterday	2. Complex innovative designs: Where is their place in drug development?
Today	**3. Real-world data and short topics session**

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

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

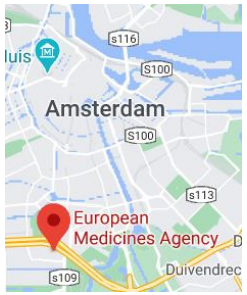
- Website: www.efspi.org

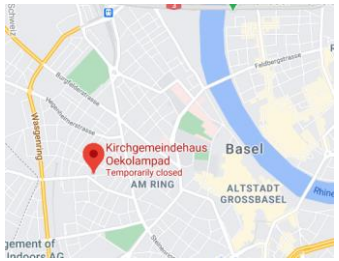


6th Regulatory Statistical Workshop!

- 2nd time virtual workshop

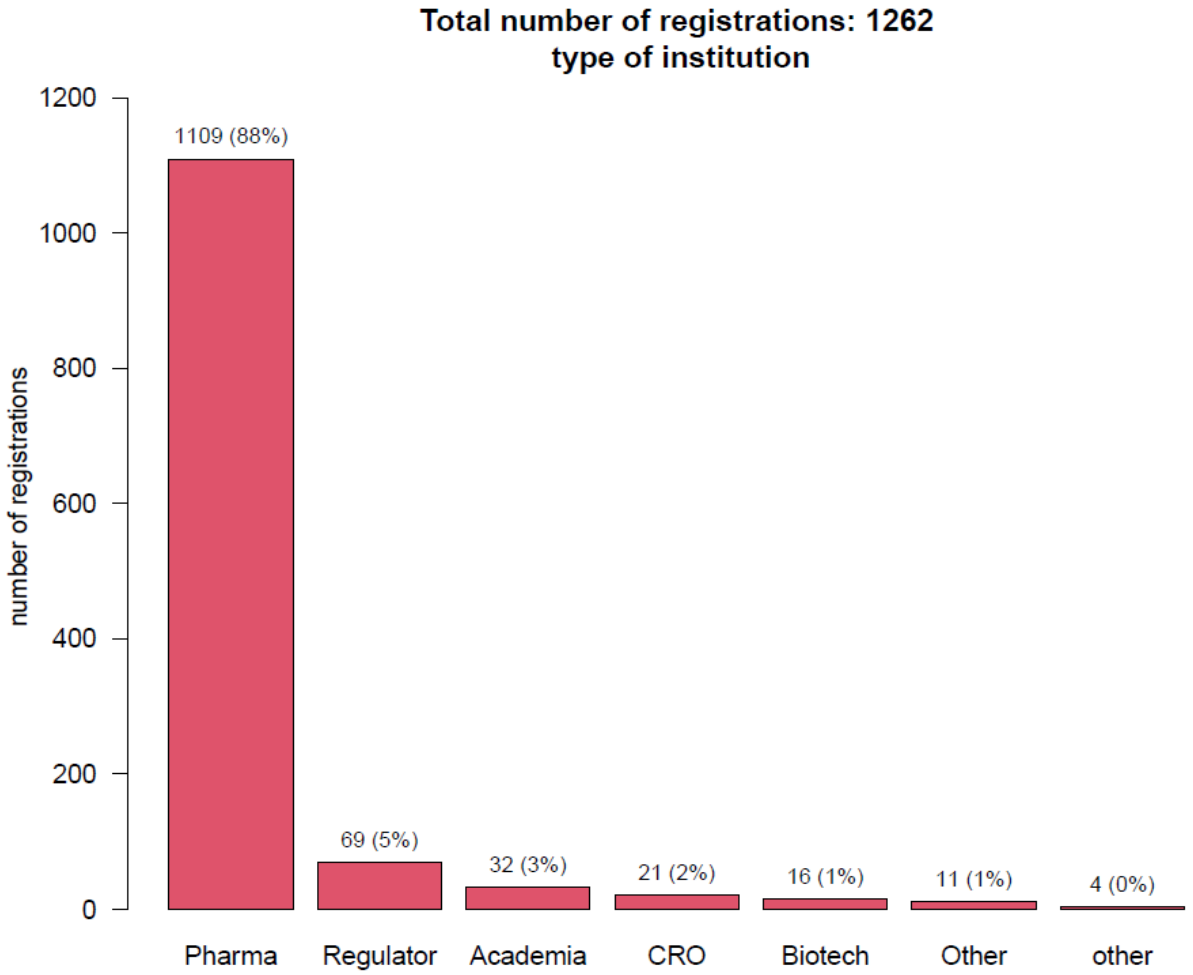
- 2022 live meeting???

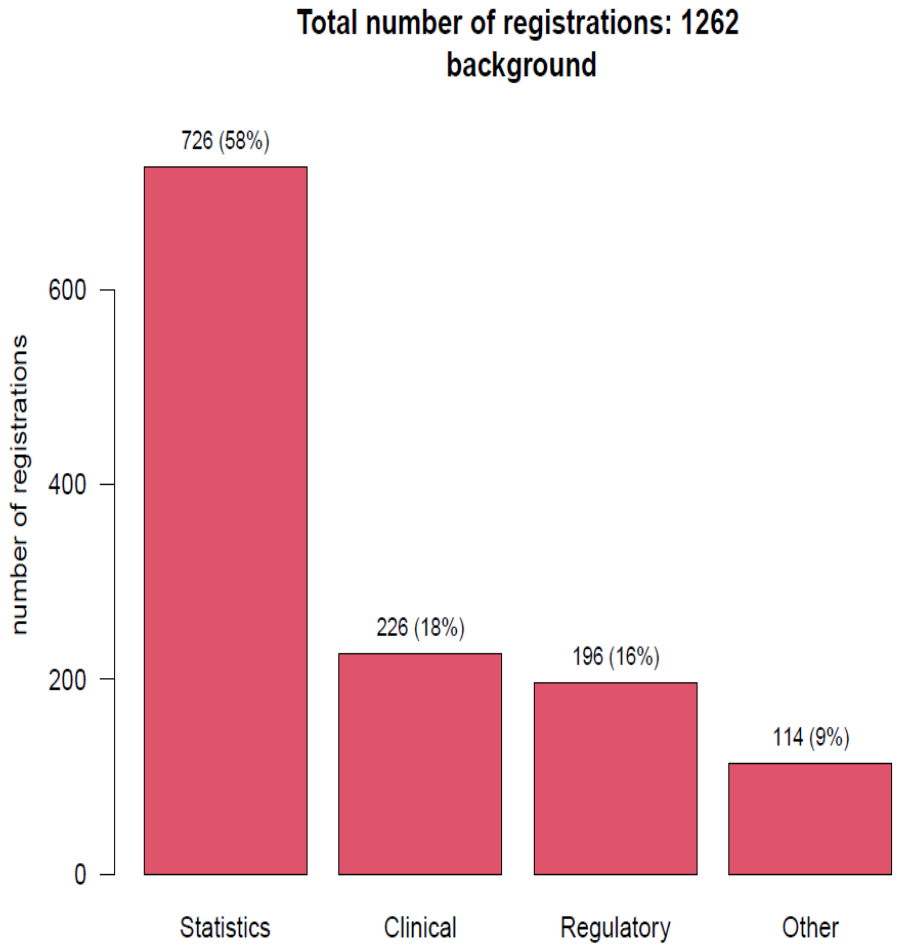




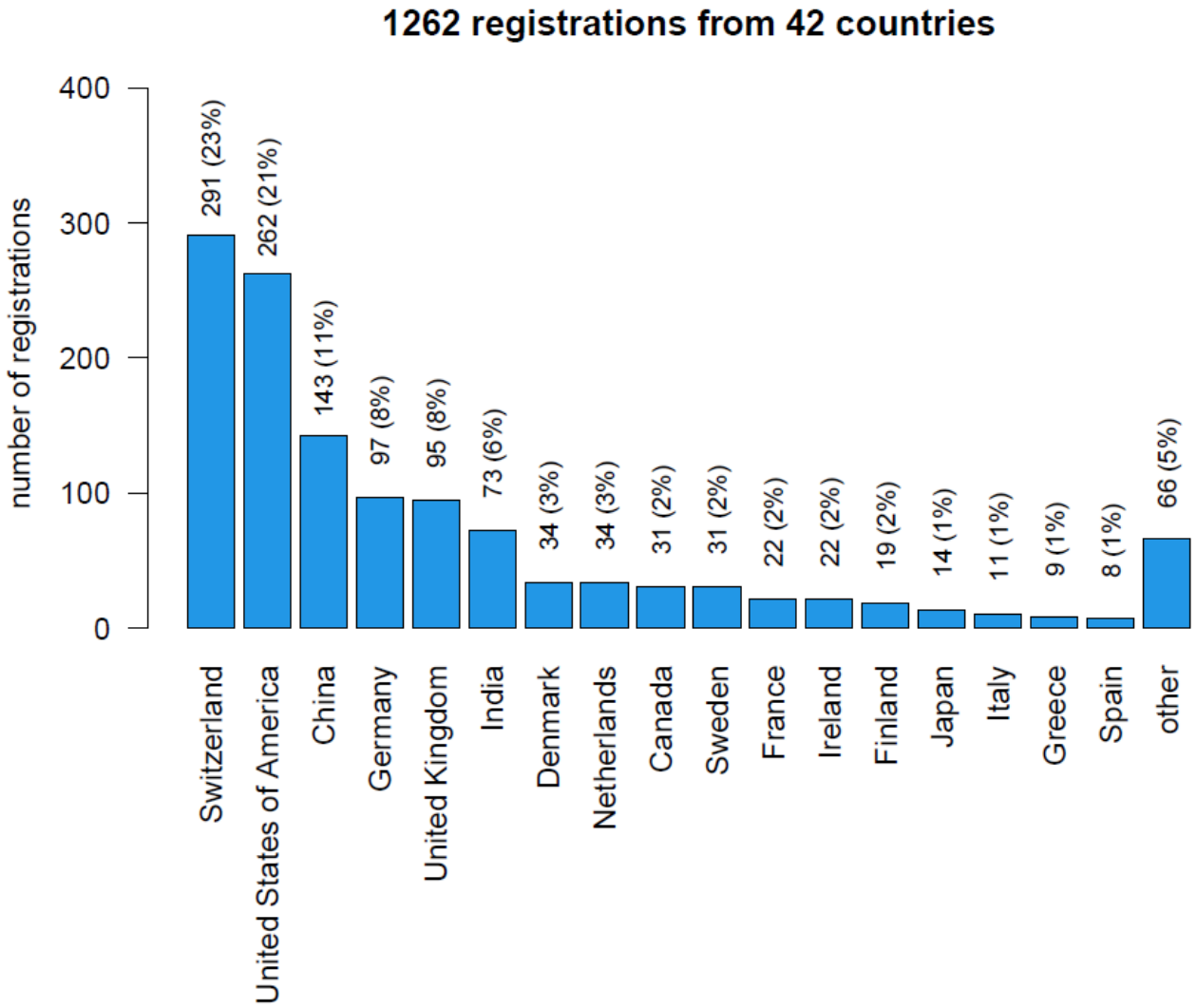
High number of participants

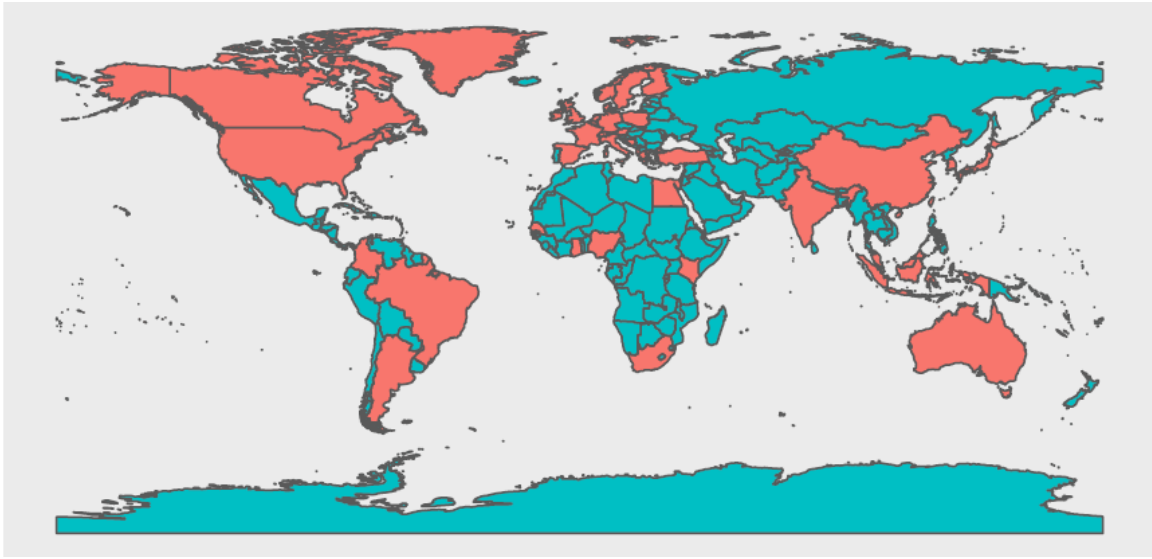
from 200+ live workshop (2019), ≈ 700 people in webinar (2020)
More than 1200 participants now!!





Worldwide representation





Big thank you to the organizers!

Local organizing committee

Egbert Biesheuvel (Danone)

Hans Ulrich Burger (Roche)

Christoph Gerlinger (Bayer)

Kaspar Rufibach (Roche)

Emmanuel Zuber (Novartis)

Scientific committee

Elina Asikanius (Finland)

Andreas Brandt (BfArM)

Maria Grünewald (Swedish Medical Products Agency)

Randi Gron (Novo Nordisk)

Cecilia Hedlund (Swedish Medical Products Agency)

Lorenzo Hess (Swissmedic)

Benjamin Hofner (Paul-Ehrlich Institute)

Armin Koch (Medizinische Hochschule Hannover)

Eftychia-Eirini Psarelli (EMA)

Khadija Rantell (MHRA)

Kit Roes (Radboud UMC)

Aldana Rosso (Danish Medicines Agency)

Anja Schiel (Norwegian Medicine Agency)

Steven Teerenstra (Radboud UMC)

Upcoming events with EFSPI involvement

28.09.2021 DIA webinar: Estimands How and Why - A Real Life Case Study in Oncology (co-organized by EFSPI / PSI WG estimands in oncology) link

21.10.2021 Joint PSI, EFSPI and ASA BIOP Webinar: Complex Innovative Designs link

Housekeeping for today

- If time allows clarification questions between the presentations will be discussed.
- Panelists please stay on mute and turn off video when not speaking.
- Participants please ask questions using the chat or in Q&A. Questions will be picked up for the panel discussion or responded to in writing after the event.
- If applicable please indicate to whom you want to ask question.
- Slides and recording will be made available on EFSPI webpage **pending approval of all speakers**.

Real-world data - using their potential

- **Chantal Quinten (EMA)** Opportunities and Challenges of RWE to Support Regulatory Decision-Making
- **Khadija Rantell (MHRA)** Meeting the expectation of patients with rare diseases: examples of high-quality medicines and timely approval
- **Tim Williams (MHRA) and Dipak Kotecha (University of Birmingham)** Pragmatic trials in the real world: DaRe2THINK – a novel approach to healthcare-embedded clinical trials
- **Qing Wang (Roche)** Use of RWD to contextualize post-hoc analysis to support regulatory submission: an example from the ORATORIO Trial and the Long-Term MSBase Registry
- **Christoph Gerlinger (Bayer)** The use of external controls: To what extent can it currently be recommended?

break

- **Marianne Cunnington (GSK)** Evolving strategies in generating medication safety data in pregnancy – an industry perspective
- **Yuki Ando (PMDA)** RWD - PMDA's view on it
- **Panel discussion with all speakers + Anny Stari (PSI / EFSPI RWD SIG chair)**