



European Statistical Meeting on **Small populations and level of evidence**

Allschwil, 27 June 2018

Hosted by Idorsia



Welcome



- On behalf of the organizers: François Aubin, Egbert Biesheuvel, Fred Sorenson, Pierre Verweij
 - BBS: Basel Biometric Section
 - EFSPI: a non-profit federation of ten national groups, collectively representing 2800 members
- We thank Idorsia for hosting this meeting
- Guy Braunstein, Head of Global Clinical Development



Session 1



- Dan O'Connor, MHRA
 - Rare diseases and orphan drugs: A Regulator's Perspective
- Stephen Senn, Luxemburg Institute of Health
 - In search of the lost loss function: ethics, equity and rationality in rare disease research



Session 2



- Henriette Thole, Novartis
 - The potential and challenges of registry use when generating evidence in small populations
- Anja Schiel, Norwegian Medicines Agency
 - Rare diseases and orphan drugs: The HTA Perspective



Session 3

- Adele Morganti, Actelion
 - Borrowing historical control in a pediatric clinical trial for a rare disease

- Andreas Kaiser, Bayer
 - Bayesian analysis for small sample size trials using informative priors derived from historical data



Session 4

- Elina Asikanius, Roche
 - Using a non-interventional study to strengthen the evidence collected in Phase III program: a Hemophilia A case Study
- Hans Hockey, Biometrics Matters, New Zealand
 - Hockey sticks and broken sticks – a design for a single - treatment, placebo - controlled, double - blind, randomized clinical trial suitable for chronic disease
- Panel discussion