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