Small populations and level of evidence

Wednesday June 27, 2018,

Allschwil (Basel)

Over the last years progress has been made in statistical methodology for the efficient assessment of safety and efficacy of treatments in small populations. This meeting brings together experts to discuss these methods and the level of evidence that is needed to apply them.

The Basel Biometric Society (BBS) and the European Federation of Statisticians in the Pharmaceutical Industry (EFSPI) are pleased to organize a European Scientific Meeting on this relevant topic. This meeting will provide a forum to hear about the latest development of methods in small populations from representatives from European regulatory bodies, and from practitioners in the pharmaceutical industry and academia.

9:00am to 5:00pm

Confirmed presenters:

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

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

Elina Asikanius

Roche

Using a non-interventional study to strengthen the evidence collected in Phase III program: a Hemophilia A case

study

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

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 diseases.





Venue

Idorsia Pharmaceuticals, Hegenheimermattweg 91, Allschwil, Switzerland

Registration Costs

On or before May 25th Industry rate: €150.00 Academic rate: €90.00

After May 25th Industry rate: €190.00 Academic rate: €120.00

Fee includes lunch & refreshments

TO REGISTER PLEASE GO TO:

www.efspi.org

Or contact:

EFSPI Secretariat Tel: +44 (0)1625 664549 efspi@kingstonsmith.co.uk

For information regarding the scientific content, contact:

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Note that there is only limited parking space. Public transport is recommended:

From the airport: Line 50; change to line 31 or 38 at *Kannenfeldplatz*; exit at *Im Brühl*