

# 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,  
Hegenheimerweg 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](http://www.efspi.org)

## Or contact:

EFSPi Secretariat  
Tel: +44 (0)1625 664549  
[efspi@kingstonsmith.co.uk](mailto:efspi@kingstonsmith.co.uk)

## For information regarding the scientific content, contact:

Fred Sorenson  
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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*