



EF SPI/PSI SIG



Small Populations - Rare Diseases

Egbert Biesheuvel

Stats Leaders Meeting

5 July 2016

- ASTERIX was funded as part of FP7 Call end 2013
New methodologies for clinical trials for small population groups



- EFSPI/PSI is research partner in Asterix

Advances in Small Trials dEsign for Regulatory Innovation and eXcellence

- Provide professional platform through its connections to organize targeted dissemination activities as well as joint workshops across all stakeholders
- Contribute additional case studies and will assist in the *Validation of new methods within clinical as well as regulatory settings (WP5)*

The main aims of this SIG:

- *To exchange information, share case studies and discuss strategies and methodology being applied in this area of research,*
- *To form a working expert group to interact with the external community, like the FP7 programs (IDEAL, INSPIRE and ASTERIX), and in particular to be in reach for the ASTERIX project in which EFSPI is a partner*
- *Organise and/or participate in workshops and create visibility on biostatistics activities for small populations*

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- Started in January 2016
 - Monthly webinars/telecons
 - Core steering committee:
 - Francois Aubin Venn Life Sciences
 - Karola Beckmann Bayer
 - Egbert Biesheuvel Danone
 - Olivier Imbert Servier
 - Sharing information via the members area on the EFSPI website

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- Current members:
 - Tal Otiker (GSK)
 - Natacha Gallot (Veramed)
 - Lilla DI Scala (Actelion)
 - Tim Friede (University Goettingen)
 - Julie Jones (Novartis)
 - Topics so far:
 - presentations on new techniques, feedback of conferences and initiatives (IRDiRC workshop),
 - limited discussion of real cases so far

- *Who in your company/department has interest?*
- *How to promote this SIG?*
- *How to discuss real case studies &
How to interact with the three consortia (until 2017)*
- *Any other suggestion?*
- *Related: legal status of EFSPI & BREXIT*





Backup



- include patient level info & perspectives in design and decision making throughout the clinical trial process
- statistical design innovations in individual and series of trials
- re-consider the scientific basis for levels of evidence to support decision making at the regulatory level
- framework for rare diseases wrt rational trial design choices
- validation of new methods against real life data and regulatory decisions to improve regulatory decision making



Academisch Medisch Centrum

Universiteit van Amsterdam



Medizinische Hochschule
Hannover



Universitat Autònoma
de Barcelona



- assessment of randomization
- extrapolation of dose-response information
- adaptive trial designs
- optimal experimental designs in mixed models
- pharmacokinetic and individualized designs
- simulation of clinical studies
- involvement and identification of genetic factors
- decision-theoretic considerations
- evaluation of biomarkers and surrogate endpoints



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- early dose-finding trials
- decision-theoretic designs
- confirmatory trials in small trials and personalized medicines
- evidence synthesis in planning and interpretation of clinical trials in small populations

