





# New

# **Special Interest Group**

## **Small Populations - Rare Diseases**

Egbert Biesheuvel

Stats Leaders Meeting

1 July 2015

FP7 Call – HEALTH.2013.4.2-3 New methodologies for clinical trials for small population groups

Three projects are funded:

**Background** 

#### ASTERIX

Advances in Small Trials dEsign for Regulatory Innovation and eXcellence

### IDeAl

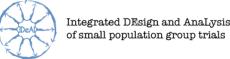
Integrated Design and AnaLysis of small population group trials

#### InSPiRe

Innovative methodology for small population research











- 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



de Barcelona



Integrated DEsign and AnaLysis of small population group trials

- 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



**RU**B

universiteit



Wrocław University of Technology UNIVERSITET



CHALMERS UNIVERSITY OF TECHNOLOGY





- 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



UNIVERSITY OF





## PSI/EFSPI will:

 Take the lead in the Dissemination & Stakeholder involvement (WP6): including a project website, bi-annual leaflets/progress letters, scientific publications, stakeholder meetings and a final ASTERIX conference → Egbert Biesheuvel

• 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)

SIG could play important role here





- Exchange information & share case studies of statistical/methodological challenges in this area
- Collaborate and discuss strategies and methodology
- Create visibility on biostatistics activities, and promote and highlight opportunities for statisticians
- Form a working expert group within industry identified by, and interact with the external community
- Support in the validation, appreciation and promotion of new methodology (in particular for the ASTERIX project)
- Organise and/or participate in workshops in this field





The SIG Small Populations will be organised by:

- A core organising committee of a few dedicated individuals who actively develop SIG activities (starting as Chair: Egbert Biesheuvel)
- A free membership and a SIG mailing list to keep people up to date with our plans and progress
- A series of monthly telecoms/webinars
- Sharing information mainly via the dedicated SIG section on the EFSPI website





- To get started and install a core organising committee, arrange telecoms with minutes and create a mailing list
- Announce our existence and increase our visibility within EFSPI, the statistical community and the expertise field of rare diseases
- Exchange existing knowledge of the individuals involved, and discuss current landscape of regulatory guidance wrt statistical methodology for small population
- Create a relationship with FP7 projects and others





- Name SIG: Small Populations ↔ Rare Diseases
- Who in your company/department has interest?
- How to promote this SIG?
- Scope of activities for remainder of 2015?
- Any other suggestion?





