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:

- **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
• 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
• 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
Role within ASTERIX

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*
Aims SIG

- 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 EFSPRI website
Objectives and Activities 2015

- 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 EFSP, 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
Discussion Points

• 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?