
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



- **IDeAI**

Integrated Design and AnaLysis of small population group trials



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



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- 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

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

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- 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?*



