InSPiRe:
Innovative methodology for small populations research
An overview of research

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InSPiRe

Innovative methodology for small populations research

Project partners

- University of Warwick, United Kingdom

- Universitaetsmedizin Goettingen (UMG), Germany

- Medizinische Universitaet Wien (MUW), Austria

- INSERM, France

- BfArM, Germany

- Stockholms Universitet, Sweden

- Quintiles Ltd, United Kingdom

- Clinical Trials Consulting and Training Ltd (CTCT), UK
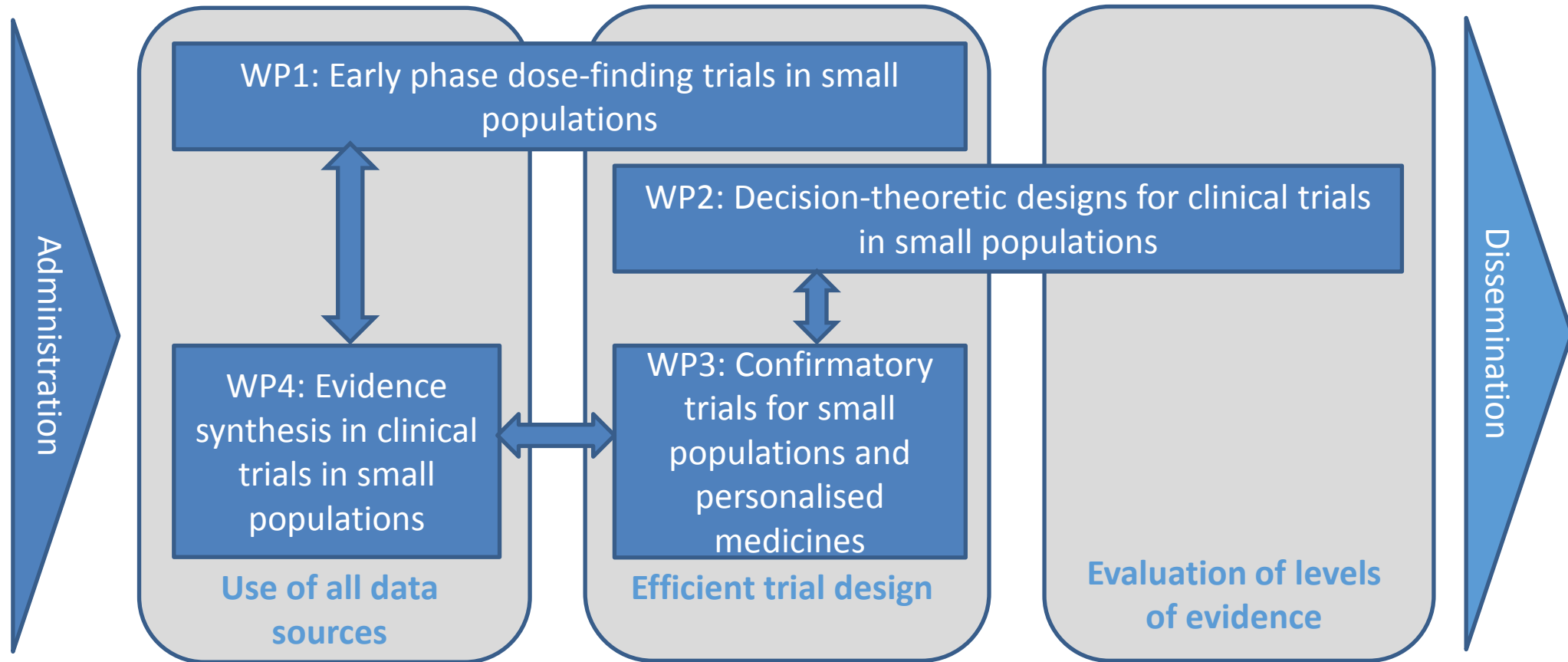
WP1: Research in early dose-finding trials in small populations

WP leader: Sarah Zohar (INSERM)

Development of efficient designs for early-phase dose-finding

     - use of PK data modelling to improve dose-finding

     - development of method using full PK/PD modelling

     - application in paediatrics

Extrapolation from adult data in paediatric studies

Prior elicitation for Bayesian extrapolation methods

Session 3: Sarah Zohar, Session 6: Moreno Ursino

WP2: Research in decision-theoretic designs for clinical trials in small populations

WP leader: Nigel Stallard (Warwick)

Review of designs for clinical trials in rare diseases

Decision-theoretic determination of sample size

- health-economic value-of-information method

- optimising gain function allowing for population size

- application in a series of case-studies

Optimal designs for sequential trials or series of trials

Session 4: Nigel Stallard

Hee *et al.* (2017) *Orphanet Journal of Rare Diseases*, 12, 44.

Stallard *et al. Biometrical Journal*. DOI: 10.1002/bimj.201500228

WP3: Research in confirmatory trials for small populations and personalized medicines

WP leader: Martin Posch (MUW)

Subgroup analysis with threshold selection

Decision-theoretic methods for trials of targeted therapies

      - optimal enrichment designs

      - optimal adaptive enrichment designs

Adaptive enrichment designs with survival endpoints

Session 4: Martin Posch

Graf *et al.* (2015) *Biometrical Journal*, 57, 76-89.

Ondra *et al.* (2016) *PLoS* One. 11:e0163726

WP4: Research in the use of evidence synthesis in planning and interpretation of clinical trials in small populations

WP leader: Tim Friede (Goettingen)

Evaluation of meta-analysis methods for few studies

   - heterogeneity estimation

   - improved confidence intervals

Robust extrapolation in meta-analysis using mixture priors

Development of novel methods for network meta-analysis

Session 2: Tim Friede

Friede *et al*. Biometrical Journal. DOI: 10.1002/bimj.201500236

Friede *et al*. Research Synthesis Methods. DOI: 10.1002/jrsm.1217

R packages `bayesmeta` and `nmainla`