ICORD 2015 - Abstract submission form

Please read the instructions in the pdf-file "ICORD 2015 abstract instructions"

ICORD welcomes submissions of abstracts representing all topics in the field of rare diseases and orphan drugs. To be selected for oral presentations during this year's annual meeting the abstract should cover the one of the following topics: Prevention & Screening; International registries and biobanks and International rare disease collaborations.

Do you agree to have the abstract published in the Rare Diseases and Orphan Drugs Journal? (Please indicate with an X below. There will be no proofreading before publication)

- Yes
- ~~No~~

Please indicate with an X the category that best fits your abstract:

- Academic study X
- Clinical study
- Ethics
- Orphan drug development and accessibility
- Policy and regulation
- Quality of life/ Psychosocial aspects
- Other

Please write the abstract in the box below according to the instructions (delete the instructions before submission)

METHODOLOGY COLLABORATIONS IN THE EUROPEAN UNION

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Three related projects have been funded under the European Union's Seventh Framework Programme for Research, Technological Development and Demonstration (FP-7) call for "New methodologies for clinical trials in small population groups." Each of these is an independent collaboration amongst several academic and industry-based methodologists. Furthermore, all three projects are sharing experiences and regularly interact with each other.

The IDeAl project ("Integrated Design and Analysis of small population group trials" http://www.ideal.rwth-aachen.de/) aims to utilize and connect all possible sources of information in order to optimize the complete process of a clinical trial. Topics for research include assessment of randomization, the extrapolation of dose-response information, the study of adaptive trial designs, the development of optimal experimental designs in mixed

models, as well as pharmacokinetic and individualized designs, simulation of clinical studies, the involvement and identification of genetic factors, decision-theoretic considerations, and the evaluation of biomarkers.

The Asterix project ("Advances in Small Trials dEsign for Regulatory Innovation and eXcellence" http://www.asterix-fp7.eu/) includes how to consider (quantitative) methods to include patient level information and patient perspectives in design and decision making throughout the clinical trial process; statistical design innovations for rare diseases in individual trials and in series of trials; reconsideration of the scientific basis for levels of evidence to support decision making at the regulatory level; developing a framework for rare diseases that allows rational trial design choices; and validation of new methods using real life examples to see how they may aid regulatory decisions making.

The InSPiRe project ("Innovative Methodology for Small Populations Research" http://www.warwick.ac.uk/inspire/) is looking at early stage dose-finding trials and dose-finding trials particularly in paediatrics; decision-theoretic designs; design of confirmatory trials and personalized medicines; and evidence synthesis in the planning of clinical trials in small populations.

Each of the separate projects has an external Independent Scientific Advisory Committee and the Principal Investigators from each project sit on each of the other Advisory Committees.

Several papers have already been published stemming from these projects, others are under review and still more in preparation. This talk will summarise the key methodological contributions that are being made.

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