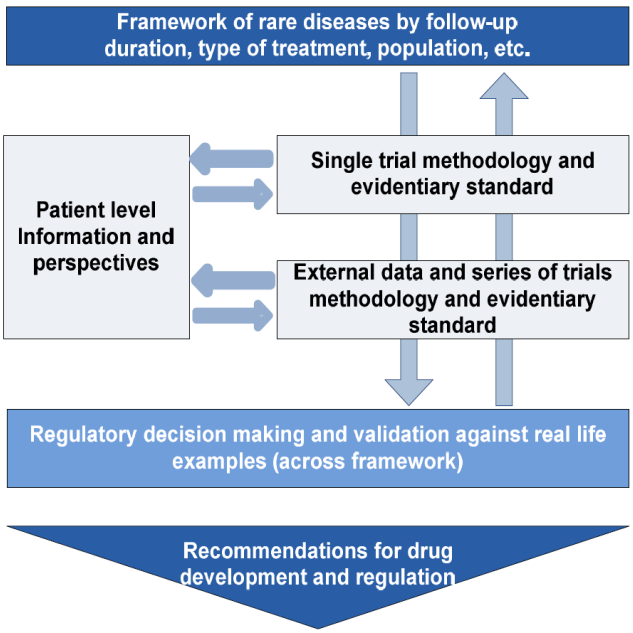
The Asterix consortium: towards better evidence for new treatments for orphan diseases

Johanna H. van der Lee[1], Armin Koch[2], Egbert H.E. Biesheuvel[3], Cor Oosterwijk[4], Caridad Pontes[5], Martin Posch[6], Ferran Torres[7], Rieke van der Graaf[8], G. Caroline M. van Baal[8], Kit C.B. Roes[8]

[1]Pediatric clinical Research Office, Academic Medical Center Amsterdam, the Netherlands; [2]Institute of Biostatistics, Medizinische Hochschule Hannover, Germany; [3]Biometrics Advance Medical Nutrition, Nutricia Research Utrecht, the Netherlands; [4]VSOP Soest, the Netherlands; [5]Clinical Pharmacology Unit, Universitat Autònoma de Barcelona, Spain; [6]Center for Medical Statistics, Informatics and Intelligent Systems, Medizinische Universitaet Wien, Austria; [7]Biostatistics Unit at the Faculty of Medicine, Universitat Autònoma de Barcelona, Spain; [8]Julius Center for Health Sciences and Primary Care, University Medical Center Utrecht, the Netherlands

Background. The challenge of generating evidence in small populations is one of the main factors hampering the market authorization of orphan drugs. To enhance the clinical phase of orphan drug development, the European Union funded three consortia, including the Asterix project: Advances in Small Trials dEsign for Regulatory Innovation and eXcellence. This poster summarizes the research programme of this consortium.

Methods and Results.



The program includes a framework of condition clusters in which conditions with similar aspects relevant for trial design are clustered to enable methodological recommendations to be developed at a higher level without the need for case-by case decisions.

New statistical methodologies will both aim at individual trial designs and at series of trials and optimal use of observational data in (series of) trials.

Unique within the consortium is the important role for patients and their representatives to integrate their perspective and advice about small trial aspects for which the acceptability for patients is crucial, such as recruitment and outcome measurement.

Conclusion. The new methodologies will be evaluated from a statistical, patient, clinical and regulatory perspective with the aim to have impact for researchers and regulatory decision making in the field of rare diseases.

www.asterix-fp7.eu j.h.vanderlee@amc.nl