END SYMPOSIUM

Program (preliminary)

Monday September 18th, 2017

13.00h – 13.30h **Opening & Welcome**
By representative of the Dutch Ministry of Health (VWS)

13.30h – 15.00h **Session 1: Disease clustering to guide trial design and analysis**

Clustering rare medical conditions has guided innovative development of trial design and analysis and will improve efficiency in the development program of orphan drugs. The Asterix clustering is outlined and used to evaluate applicability of novel methods using existing EPARs. Recommendations, specific for different clusters are considered.

15.00h – 15.30h **Coffee and Tea Break**

15.30h – 16.45h **Session 2: Challenge of fundamentals in small populations**
Ethical Framework and patient involvement – wide perspective

Rare diseases with their small populations provide unique opportunities for patients to be involved in research to accelerate availability of novel treatments. This session highlights ethical aspects, the role of patient engagement in clinical research and an application of the POWER model, a tool for patient engagement in clinical trials.

16.45h – 18.00h **Session 3: Challenge of fundamentals in small populations**
Correct evidence: the role of randomization and observational data

Rare diseases with their small populations provides challenges on how to gather correct information of evidence. In this interactive session, the role of randomization and the use observational data in registries is explored. A forum of clinical, statistical and field experts can be challenged by the public on these issues.

18.00h – 19.00h **Reception**

19.00h – 22.00h **Dinner**

Tuesday September 19th, 2017

8.30h – 9.30h **Session 4: Improving clinical trial design: sequential, adaptive and multiple treatment comparions**

An overview of new methods developed within the Asterix project leading to improved clinical trial design for small populations is presented. Two recent examples of implementation of new methods in clinical trials in rare diseases are illustrated: ALS and Cystic Fibrosis.

9.30h – 10.15h **Session 5: Potential and pitfalls of meta-analysis in small populations**

At the start of the Asterix project, we were optimistic about the use of meta-analysis in small populations. After four years, we have gained a much better knowledge and understanding of the applicability of meta-analysis in drug development for rare diseases. The problem of heterogeneity as well as practical advice in sparse settings is discussed.

10.15h – 10.45h **Coffee and Tea Break**

10.45h – 12.15h **Session 6: Improving relevance and efficiency of endpoints in trials**

This session starts with a presentation of a regulator's view of the use of alternative endpoints in clinical trials of small populations. Next, it is focused on Goal Attainment Scaling (GAS) by showing a demo, and presenting a clinician's and patient's view on the use of GAS. The session concludes with the plans to submitted GAS to EMA for scientific advice to shape regulatory landscape.

12.15h – 13.15h **Lunch**

13.15h – 14.30h **Session 7: How to justify different evidentiary standards for decision making in rare disease?**

A framework based on prior beliefs to relax the evidence in the target population is presented. Considerations on the total number of patients to be treated as well as using utility functions to support decision making is illustrated. A regulatory view on decision making completes this session.

14.30h – 15.00h **Coffee**

15.00h – 17.00h **Session 8: Implementation and Continued Development**

This final session reflects the perspectives of various relevant stakeholders, like Asterix Advisory Board, young statistical researchers, Patient Think Tanks, similar FP7 projects and regulators.
consortia on small populations

17.00h **Closure**

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Confirmed Speakers:

Prof. Fernando de Andrés-Trelles (Universidad Complutense of Madrid, PDCO of the EMA)

Prof. Kors Van der Ent (University Medical Center of Utrecht)

Prof. Leonard Van den Berg (University Medical Center of Utrecht)

Charlotte Gaasterland (Academic Medical Center Amsterdam)

Dr. Vincent Gulmans (Dutch Cystic Fibrosis Foundation)

Dr. David Haerry (European AIDS Treatment Group)

Prof. Jörg Hasford (Ludwig-Maximilians-University of Munich)

Prof. Ralf-Dieter Hilgers (University of Aachen),

Dr. Martine Jansen-van der Weide (Academic Medical Center Amsterdam)

Prof. Armin Koch (Hannover Medical School)

Florian Lasch (Hannover Medical School)

Prof. Bert Leufkens (Medicines Evaluation Board)

Marian Mitroiu (University Medical Center of Utrecht)

Stavros Nikolakopoulos (University Medical Center of Utrecht)

Dr. Katrien Oude Rengerink (University Medical Center of Utrecht)

Dr. Caridad Pontes, MD (Universitat Autònoma de Barcelona)

Prof. Martin Posch (Medical University Vienna)

Dr. Kaczmarek Radoslaw (European Hemophilia Consortium)

Prof. Kit Roes (University Medical Center of Utrecht)

Arantxa Sancho, MD (Hospital Universitario Puerta de Hierro)

Dr. Mark Sheehan (University of Oxford, and member of Asterix Ethics Advisory Board)

Dr. Edwin Spaans (Khondrion)

Prof. Nigel Stallard (University of Warwick)

Prof. Josep Torrent-Farnell (Universitat Autònoma de Barcelona)

Kerry Leeson-Beevers (Alström Syndrome UK): is she confirmed??