

EU-funded projects on Methodology for Clinical Trials in Small Populations to organise Symposium

Aachen, 04.03.2014 –In the EU diseases that affect no more than 5 in 10,000 people are called rare. In the EU 6 to 8 % of the population - that is, from 27 to 36 million people – suffer from at least one of the 5,000 to 8,000 different rare diseases. According to information from the European Society of paediatric oncology, most of the rare diseases affect children, of whom 30% die before reaching their fifth birthday.

The ability of conventional statistical methods to evaluate new therapeutic approaches for any given rare diseases is limited due to the small number of patients concerned. This means that established statistical approaches to demonstrate the efficacy and safety of therapies may fail in this situation. Thus, there is an urgent need not only to develop new therapeutic approaches to treat diseases, but also to develop new statistical methods to establish which approaches work.

As a response to this need, the European Union have funded three projects to explore new methods for design and analysis of clinical studies in small population groups within the 7th Framework Programme. The three projects are the ASTERIX project led by Professor Kit Roes at UMC Utrecht, Netherlands, the IDEAL project led by Professor Ralf-Dieter Hilgers at the RWTH Aachen University, Germany and the INSPIRE project led by Professor Nigel Stallard at the University of Warwick, United Kingdom. All three projects bring together experts in clinical trial methodology and statistics in small populations and patient organisations from across Europe.

To mark the start of the three projects, the ASTERIX, IDEAL and INSPIRE projects are organising a joint Symposium on Small Populations to be held at the Medical University of Vienna, 1 July 2014. The aim of the Symposium is to bring together relevant researchers in the field, to provide a survey of the current state of the art, and to discuss directions for the future research of the three EU projects and by other experts in the field as well as representatives of rare disease groups.

Speaking about the workshop, Professor Roes said "This is a very exciting time for development of methods for clinical trials in small populations, with the real possibility of substantial progress in the next few years. This symposium will include more than 100 researchers who represent the best in Europe in this area and ensure that the ASTERIX, IDEAL and INSPIRE projects work closely together to address the serious challenges associated with conducting trials in this area." Professor Hilgers points out " We are all happy about the contribution of the European Union to support research in biostatistics. This shows the importance of the biostatistical methods for health research – in particular for new therapies in rare diseases. The ability to use mathematical and statistical techniques for the evaluation of new treatments where standard methods fail to be successful is a challenge and motivation for all of us. We all look forward to this exciting collaboration."

The announcement of the meeting today coincides with the international Rare Disease Day co-ordinated by EURORDIS to raise awareness of rare diseases. Professor Stallard added "Rare Disease Day serves as a reminder of the enormous need for continuing research in this area. The aim of the Symposium and the ASTERIX, IDEAL and INSPIRE projects is to develop methods to facilitate this

research by enabling clinical trials to be conducted more quickly whilst maintaining scientific rigour.”

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