

Importance of discovery and clinical testing during orphan drug development

Scientific meeting *Stuurgroep Weesgeneesmiddelen* at Figon Dutch Medicines days in Lunteren on 7 October 2008

This year the *Stuurgroep Weesgeneesmiddelen* wants to bring to the attention the importance of drug discovery and clinical testing during the process of orphan drug development. The meeting will be chaired by **prof. dr. Herjan Coelingh Bennink, CEO of Pantarhei Bioscience.**

Morning session (11:00-12:30): Discovery

With over 500 designated products, the EU Regulation on Orphan Medicinal Products begins to show its potential to stimulate research, development and marketing of products for rare disorders. Interestingly, small molecules still represent the largest share, and include both novel as well as old molecules. **Dr. Remco de Vruh (Stuurgroep)**, will start off with an introductory overview on orphan drug development and will focus on the apparent lack of drug development for ultra rare disorders. He will touch upon the potential of high-throughput screening and repurposing of drugs to fill up the pipeline for ultra rare disorders. **Dr. Johan Tijhuis (Specs)** will discuss the potential of combining biological expertise with their chemical expertise and provide some examples of successful screening of large compound libraries and identification of several new classes of compounds with biological activity. The morning session will be concluded by **Dr. Cees van Veldhuizen (Orphan drugs NL)**, who will discuss in detail the innovative repurposing of levamisol, an existing drug, as potential therapy for nephrotic syndrome, a rare kidney disorder.

Afternoon session (14:30-17:30): Clinical testing

Successful clinical development of an orphan drug does not only require specific practical expertise, but also requires thorough knowledge of the rare disorder as well as involvement of the patient. **Drs. Harald Heemstra (University of Utrecht)** will start off and will share the latest results of his study to identify determinants of the clinical development program indicative for failure of an orphan drug. **Dr. Kim Wever (VSOP)** will discuss in great detail the importance and added-value of patient involvement in clinical trials. **Dr. Rolf Boot (AMC)** will share his vision on the importance and value of good biomarkers in patient management, but also their potential to provide new targets for therapeutic intervention. Next, **Dr. Eva Morava (UMC St. Radboud)** will focus on the natural course of disease in inborn errors of metabolism and how to evaluate the success of treatment in orphan diseases, using metabolic diseases as an example. The afternoon session will be concluded by **Dr. Jaap de Boer (Genzyme)** who will provide an industry perspective on the clinical development of orphan drugs, using the development of Myozyme[®] as an example.

More information:

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