INVITATION

Workshop 'Beyond the current clinical endpoints'

Date: July 6th 2017
Time: 14:00-21:00
Location: Muntgebouw, Leidseweg 90, Utrecht, The Netherlands

How can we improve the selection and use of challenging endpoints such as the 'established' 6-minute walk test (6MWT) or the less well-known ones such as the Goal Attainment Scaling (GAS)? This is the key question of the next RSNN meeting, and a continuation of the theme of our RSNN meeting at the 2016 FIGON Dutch Medicines Day and the 2016 annual RSNN Workshop.

Today, the majority of clinical endpoints inform us about the efficacy and clinical relevance of new drugs. However, some endpoints are difficult to translate into a clinically meaningful benefit. For example, the 6MWT is widely used as an endpoint to estimate the effectiveness of interventions in numerous diseases, such as osteoarthritis, pulmonary fibrosis, Duchenne Muscular Dystrophy, and Fabry Disease. Reasons for its use vary: it is a cost-effective test, easy to perform, and reproducible; it is seen as the standard and a good surrogate for clinical outcome; and sometimes it is just "the best we have". However, the 6MWT has sparked a lot of discussion in the field; it could be queried, for instance, whether distance can be translated into the most relevant, often complex, functional outcomes. These types of questions are important, since in the end the choice of 'established' or less well-known endpoints in the most relevant patient population is paramount in the benefit/risk discussion in the context of licensing of and reimbursement for drugs.

During the meeting, we aim to get some of the answers to these questions, which will not be easy! Therefore, we ask participants to come to the meeting prepared: a quantitative survey will go to all attendees so that the discussion can be focussed.

The day of the meeting will start with 4 lectures that will address the topic from the perspective of different stakeholders: e.g., the scientist, the patient, the investigator, and the regulator. Thereafter, the participants will have an active role in the workshops. The aim of this part of the day is to formulate common viewpoints. These then will be presented during a plenary session. And there will be ample time to continue discussions over dinner.

We look forward to meeting you and to receiving your input.

The full agenda of the meeting is on the next page.

For more information please contact Peter van Meer | p.v.meer@cbg-meb.nl.

PROGRAMME

13:30 Registration and coffee

14:00 Opening
Bert Leufkens, Chairman of RSNN, MEB

14:10 Setting the scene: aspects of endpoint selection in clinical trials
Kit Roes, Julius Center UMCU, MEB

14:25 Key perspectives and examples from regulatory perspective
Violeta Stoyanova, COMP-EMA/MEB

14:40 Key perspectives and examples from the industry perspective
Henk Kamsteeg, Janssen Biologics

14:55 Key perspectives and examples from the reimbursement
Jacoline Bouvy, NICE

15:15 Workshop assignment/explanation

15:30 Workshop (with coffee and refreshments)

17:00 Feedback from the workshop

17:30 Plenary discussion

18:15 Close and drinks

18:30 Dinner
Dinner speeches - Marc Kaptein, Pfizer / Yvonne Schuler, UvA

21:00 End


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Regulatory Science Netwerk Nederland