

Meeting report series

Report of the 6th Companies Constituent Committee Meeting

Teleconference

August 1, 2018

Participants

Dr Mathew Pletcher, Roche, Switzerland – Chair

Dr Madhu Natarajan, Shire, USA – Vice Chair

Dr Katherine Beaverson, Pfizer, USA

Dr Tim Considine, Recursion Pharmaceuticals, USA

Dr James McArthur, Cydan II, USA

Dr Anneliene Jonker, Scientific Secretariat (Sci Sec), France

Dr Lilian Lau, Sci Sec, France

Dr Anne-Laure Pham Hung d’Alexandry d’Orengiani, Sci Sec, France

Apologies

Ms Karen Aiach, Lysogene, France

Dr Andrea Chiesi, Chiesi Farmaceutici, Italy

Dr Carlo Incerti, Genzyme, USA

Dr Ning Li, BGI, China

Dr Robert Mashal, NKT Therapeutics, USA

Dr Brett Monia, Ionis Pharmaceuticals, USA

Dr Tom Pulles, Ultragenyx, Switzerland

Dr Ellen Welch, PTC Therapeutics, USA

Dr Jame Wu, WuXi AppTec, China

Agenda

1. Review of Activity H worksheet and next steps
2. Update on new member invites

REPORT

1. Review of Activity H worksheet and next steps

- ▶ The goal of Activity H is to fill the knowledge gap on rare diseases and motivate companies to shift their interest from better-known rare diseases to others with little or no research programs
- ▶ As discussed in the previous teleconference, the first step will be to generate a spreadsheet containing a list of data type of interest/importance
 - Members to help identify data categories that are of interest to companies
 - E.g. in vitro assays, animal models, monogenic or multigenic disorders, gene implicated, disease progression, availability of biomarkers
 - In addition, members are asked to indicate potential sources to provide the requested data
- ▶ Together with the list of diseases generated based on Shire's work, potential to come up with a short list of RD beyond the usual "core" diseases that have high commercial interest value
 - Plan to organize and classify by gene
 - Classic designations often predate understanding by genetics
- ▶ A proposal will be written up to formally create and approve the Activity
 - To consider how best to assemble missing data/gaps, e.g. where data can be mined and captured
 - To potentially gather these information

→ CCC members to update the worksheet with additional categories believed to be of importance for companies and the potential source that could provide the data

2. Update on new member invites

- ▶ CCC members have received a template letter of invitation from the Sci Sec
- ▶ The introductory paragraph targets stakeholders in RD research and should be updated to mention:
 - The unifying passion and focus on advancing rare disease research
 - The patient groups' involvement and support of research
 - The fact that IRDiRC is a research-based organization
 - Emphasize that the main goal is to make research more effective, efficient and collaborative

→ The CCC Chair to edit the letter of invitation and disseminate among CCC members to personalize accordingly and initiate the outreach

Main deliverables

- ▶ Update the action spreadsheet with categories
- ▶ Incorporate suggestions in the letter of invitation and forward to CCC members