

## Meeting report series

### Report of the 1st Companies Committee Meeting

Teleconference

August 30, 2016

#### Participants

Dr Christopher Austin, National Center for Advancing Translational Sciences, NCATS/NIH, USA  
Dr Carlo Incerti, Genzyme, USA  
Dr Sangeeta Jethwa, Roche, Switzerland  
Ms Karian Aiach, Lysogene, France

Ms Christine Cutillo, National Center for Advancing Translational Sciences, NCATS/NIH, USA  
Dr Anneliene Jonker, Scientific Secretariat (Sci Sec), France

#### Apologies

Dr Katherine Beaverson, Pfizer, USA  
Dr Andrea Chiesi, Chiesi Farmaceutici S.p.A., Italy  
Dr Omar Francone, Shire, Ireland  
Dr Robert Mashal, NKT Therapeutics, USA  
Dr Brett Monia, Ionis Pharmaceuticals, USA  
Dr Ellen Welch, PTC Therapeutics, USA  
Dr James Wu, WuXi AppTec Co., Ltd., China

Dr Lilian Lau, Scientific Secretariat, France  
Dr Ana Rath, Scientific Secretariat, France

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#### Agenda

1. Introduction of Companies Committee
2. Purpose of Committee
3. Analysis and discussion of impact of IRDiRC Policies and Guidelines
4. Discussion of Companies Committee

## REPORT

### Actions and deliverables

- ▶ All: Send additional ideas/thoughts to Sci Sec re:
  - Overlap and gaps in company priorities
  - Common roadblocks in company space that IRDiRC should address
  - Opportunities and challenges that IRDiRC should address
  - People from other companies who would benefit the committee due to their vision and relevance
- ▶ All: Review the impact of IRDiRC policies and guidelines on your constituency/company's work
- ▶ Sci Sec: Schedule another call with this group prior to Catania to catch those who weren't able to attend

### Conference notes

- ▶ Introduction of Companies Committee
  - With the growth of IRDiRC, committees are not only asked to comment, but also to be involved in setting the goals and actions, and contributing to those goals
  - Growth of IRDiRC requires structure that is more manageable
  - Once governance issues are resolved, a Companies Committee representative will participate in OpComm
- ▶ Purpose of Committee
  - Constituency groups will be analogous to the scientific committees in terms of their structure and level in the organization
  - Provide common forum specific to constituency areas and discuss problems that can only be solved by that constituency
  - The main initial goals of this group are to identify:
    - Overlap and gaps in company priorities; find common ways to address problems
    - Common roadblocks to efficient execution of research in company space that IRDiRC should address
  - Would like to use that information to:
    - Determine appropriate goals for IRDiRC in the rare diseases company space for the next 5 years
      - These goals will also be discussed and finalized at the IRDiRC Conference in February 2017
    - Identify how companies groups will contribute to that new set of goals
  - The committee would like to ruminate on these common issues/goals and send around thoughts to the group via email
  - Constituency committee meetings will be held during the Catania CA meeting
- ▶ Analysis and discussion of impact of IRDiRC Policies and Guidelines
  - Paper on IRDiRC Policies and Guidelines currently being written up
    - Members asked to think about impact on their organization and ask to send to Sci Sec
- ▶ Discussion

- How to stimulate and promote cross-border RD research?
  - Emphasize scientific approaches to researching access to therapeutics (as a part of the overall research strategy)
  - How to transform science (trial endpoints, post-approval trials, outcomes for patients) while using real clinical experience and data?
    - For example, need to rethink trial endpoints and identify those that will satisfy scientific requirements, regulatory authorities, and allow the therapeutics to reach patients
- How to translate innovation into sustainable access to medicines?
  - The new set of challenges in the clinical world for therapeutic development is varied
  - Need to focus on commonalities – e.g., methodologies – and build consensus between companies, regulators, and payers
    - Demonstrating efficacy
    - Patient-reported outcomes
    - Reimbursement (a bit local, but generally-speaking)
- Leverage information from different constituency groups around the same topics, to assure that no knowledge is lost
  - Bring together different constituents to focus on the same topics, so that all different points of view are addressed

## Document history

Version 1. Report drafted by Anneliëne Jonker and Christine Cutillo on August 30, 2016  
Circulated to members on August 31, 2016