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
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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
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

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