

## Meeting report series

# Report of the 1<sup>st</sup> WG on Orphan Drug-development and Regulatory Processes teleconference

20 December 2013

## Organisation

Organized by: Scientific Secretariat  
Teleconference

## Participants

Dr Didier Caizergues, Evry, France  
Dr Anthony Hall, Hoofddorp, Netherlands  
Dr Maria Mavris, Paris, France  
Dr Anne Pariser, Silver Spring, USA

Dr Barbara Cagniard, Scientific Secretariat

## Apologies

Dr Lucia Faccio, Naples, Italy  
Mr Yann Le Cam, Brussels, Belgium  
Mr David Lee, Ottawa, Canada  
Dr Debra Lewis, Silver Spring, USA  
Dr Jordi Llinares, London, UK  
Prof Devidas Menon, Edmonton, Canada  
Dr Bruno Sepodes, Lisbon, Portugal

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## Agenda

1. Overview of IRDiRC
2. Role and support of IRDiRC secretariat
3. Review of WG mandate
4. Election of the WG chair
5. WG deliverables and timing for next call

## REPORT

### Overview of IRDiRC

IRDiRC is a consortium of funders investing at least 10 million USD over 5 years in research projects contributing towards IRDiRC objectives and invited patient advocacy group. It is governed by an Executive group of funders (now 37) that will make decisions about priorities for funding in rare diseases. The two main goals of IRDiRC are:

- ▶ Produce diagnostic tools for a majority of rare diseases by 2020
- ▶ Develop 200 new therapies for rare diseases by 2020

IRDiRC has three Scientific Committees:

- ▶ Diagnostics Scientific Committee (DSC)
- ▶ Interdisciplinary Scientific Committee (ISC)
- ▶ Therapies Scientific Committee (TSC)

The purpose of the TSC is to help IRDiRC to reach the goal of developing 200 new therapies for rare diseases by 2020. The TSC oversees four Working Groups:

- ▶ WG on Biomarkers for Disease Progression and Therapy Response
- ▶ WG on Chemically-derived products including Repurposing
- ▶ WG on Biotechnology-derived products including Cell- & Gene-based Therapies
- ▶ WG on Orphan Drug-development and regulatory processes

Each WG will bring new insights and strategies to be considered by the TSC. The Chair of the TSC will report the priorities identified by the WG to the Executive Committee.

### Role and support of IRDiRC secretariat

The project manager of the IRDiRC secretariat introduced briefly the team that is located in Paris. The team is composed by 4 people, including a project manager, a communication manager, an information scientist and an assistant. Support in kind is also provided by people from the French Rare Diseases Foundation and from Orphanet. The role of the IRDiRC secretariat is to help organizing the meeting and teleconference of the IRDiRC Committees and Working Groups (WGs), to take the minutes and propose a report, but also to prepare documents upon request, including extraction of data from the Orphanet database or literature survey when needed.

### Review of WG mandate

The discussion mostly focused on clarifying the role of the WG, i.e., identifying priorities and developing recommendations for IRDiRC members. The recommendations made by the experts recruited to the working groups will assist the Scientific Committee to provide clear criteria to the Executive Committee

for the future funding and strategy of the IRDiRC funders. A representative of the TSC is also member of the WG to facilitate the communication between the WG and the TSC.

Topics that can be addressed by this WG in addition to the ones listed in the briefing document:

- ▶ Early dialogue
- ▶ convergent therapeutic guidelines,
- ▶ adaptive clinical trial design,
- ▶ alternatives to animal models,
- ▶ patient focused outcomes.

### **Election of the WG chair**

The TSC decided that a US-Europe co-chair system rather than a single chair would be pertinent for the TSC WGs, especially for the WG on regulatory processes. Anne Pariser (FDA) and Jordi Llinares (EMA) were approached and they both agreed to co-chair this WG. Participants of the teleconference are fine with this system.

### **WG deliverables and timing for next call**

The collective objective of this WG is to provide a list of 4-5 key recommendations in the area of interest.

- ▶ **Jan 10th:** Send comment to the Scientific Secretariat on specific topics to be addressed by this WG.
- ▶ **2nd teleconference** to be held before Jan 24 to discuss these topics/key priorities and prepare a short list for the TSC. Scientific Secretariat will send a Doodle.
- ▶ **Early March:** review the key topics based on TSC feedback and prepare a few recommendations for the TSC to be reviewed prior to submission to the Executive Committee in April.