

## Meeting report series

# Report of the 1st Teleconference of the WG on Biotechnology-derived products including cell & gene-based therapies

20 December 2013

## Organization

Organized by: Scientific Secretariat

Hosted by: Teleconference

## Participants

Dr Annemieke Aartsma-Rus, Leiden, Netherlands

Prof Katherine High, Philadelphia, USA

Prof Ian McDonald, Edmonton, Canada

Prof Gert-Jan van Ommen, Leiden, Netherlands

Prof Franz Schaefer, Heidelberg, Germany

Ms Elizabeth Vroom, Amsterdam, Netherlands

Dr Virginie Hivert, Scientific Secretariat

## Apologies

Dr Geraldine Honnet, Evry, France

Dr John Hyde, Silver Spring, USA

Dr Spiros Vamvakas, London, UK

Dr Daniel Scherman, Paris, France

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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

### 1) Overview of IRDiRC

The Secretariat gave a quick overview of the organisation of the IRDiRC Consortium, putting emphasis on the IRDiRC website ([www.irdirc.org](http://www.irdirc.org)) and especially the private section. The members of the WG have received login and password to enter this section where they can have access to the documents related to their work including the reports of the teleconference and meetings of the WG.

### 2) Role and support of IRDiRC secretariat

The IRDiRC Secretariat is located in Paris. The team is composed by 4 people, including a project manager, a communication officer, an information scientist and an assistant. Support in kind is also given 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 Committees and Working Groups (WGs), to take the minutes and propose a report, but also to prepare some documents upon request, including extraction of data from the Orphanet database or literature survey when needed.

### 3) Review of WG mandate

The participants have reviewed the content of the Briefing document, pointing the specific topics to be addressed by the WG:

- ▶ Scope of the WG
- ▶ Identification of clusters of compounds, that will request an overview of the already funded projects
- ▶ Establishment of priorities and criteria
- ▶ Standards to be proposed

The participants agreed that the gaps identified by the Exon skipping network are consistent with the field and that their objectives could serve as a starting point to propose elements to tackle these gaps, such as regulatory models or education of patients to prevent on unrealistic expectations.

The objectives of the exon skipping network are:

- ▶ Develop regulatory models for rare diseases
- ▶ Avoid fragmentation of work by good networking between people working on the same disease or the same therapeutic application (exon skipping for the exon skipping network, but for this WG of course this can also be gene therapy or cell therapy)
- ▶ Harmonize efforts for good outcome measures (ideally surrogate markers)
- ▶ Improve stakeholder communication

## 5) Election of the WG chair

Prof Gert-Jan van Ommen, who is the representative of the TSC, has been elected as the chair of the WG.

## 6) WG Deliverables and timing for next call

- ▶ Recommendations from the TSC have to be proposed to the Executive Committee in May, so the WGs will report to the Therapies Scientific Committee in between. To stay in line with the agenda of the TSC, the WG plans to organize the next conference call the last week of January 2014 (Doodle to be sent by the Secretariat).
- ▶ The Secretariat will provide early January the inventory of projects that are relevant for the WG, including a list of relevant preclinical and clinical studies. The full list of projects collected within the IRDiRC Consortium and funded since 2010 are available here: [http://www.irdirc.org/?page\\_id=39](http://www.irdirc.org/?page_id=39)
- ▶ The Secretariat will circulate the document 'Analysis of current issues justifying specific policies for RD research' and the participants will prepare comments for the next conference call.
- ▶ Members will also review the Memorandum of Understanding of the Exon skipping COST action which is accessible on the main page of the website <http://exonskipping.eu/wp-content/uploads/2013/09/BM1207-MoU.pdf>
- ▶ Members will provide the Secretariat with a short bio.