

Meeting report series

Report of the 3rd Therapies Scientific Committee Meeting

Teleconference
29 October 2013

Organization

Organized by: Scientific Secretariat

Hosted by: GoToMeeting session

Participants

Prof Josep Torrent I Farnell, Barcelona, Spain

Dr Giles Champion, Leiden, the Netherlands

Dr Adam Heathfield, Sandwich, UK

Dr Maria Mavris, Paris, France

Dr Glen Nuckolls, Bethesda, USA

Dr Melissa Parissi, Bethesda, USA

Dr Asla Pitkänen, Kuopio, Finland

Dr Karin Rademaker, Utrecht, the Netherlands

Prof Gert-Jan Van Ommen, Leiden, the Netherlands

Dr Ellen Welch, South Plainfield, USA

Dr Barbara Cagniard, Scientific Secretariat

Dr Virginie Hivert, Scientific Secretariat

Dr Sophie Höhn, Scientific Secretariat

Apologies

Yann Le Cam, Paris, France

Dr Fulvio Mavilio, Evry, France

Dr Luigi Naldini, Milan, Italy

Dr John McKew, Bethesda, USA

Dr Elizabeth McNeil, Bethesda, USA

Dr Robert Schaub, Waltham, USA

Agenda

1. Election of the TSC chair
2. Revision of the TSC mandate and update on the composition: discussion and agreement on how to best deliver our priorities and recommendations to IRDiRC funder members.
3. Revision of the WGs composition, mandate and specific TSC recommendations to each WG for planning their own work plan throughout teleconference(s).
4. Working procedure and timelines for the TSC taking into account the feedback from TSC WG.
5. Next TSC meeting: location and date

REPORT

Introduction

After welcoming the participants to the teleconference, the agenda was presented. The information was circulated that the chair of the Therapies Scientific Committee (TSC) resigned as the chairman.

1. Election of the TSC chair

Only one member has expressed his interest in being a candidate to this election. The members present at the teleconference accepted to a large majority the candidature of Yann Le Cam as chairman of the TSC.

2. Revision of the TSC mandate and update on the composition: discussion and agreement on how to best deliver our priorities and recommendations to IRDiRC funder members.

Apart from the updates mentioned in the 'Introduction' section, this point was reported to the forthcoming meeting/teleconference.

3. Revision of the WGs composition, mandate and specific TSC recommendations to each WG for planning their own work plan throughout teleconference(s).

Revision of the WGs composition

The list of suggested participants for the four TSC working groups (WGs) was submitted to the members for discussion and approval. It was mentioned that some amendments were done following the recommendations of the Executive Committee. One member of the TSC was proposed in each WG, to be approved by the concerned parties. Some names are still missing in the list but WGs activities could nonetheless start right away.

WG on Biomarkers for disease progression and therapy response

- ▶ The TSC members agreed on the composition of this WG.

WG on Chemically-derived products including repurposing

- ▶ The TSC members agreed on the composition of this WG.

- ▶ Amendments were proposed regarding the contact details of some members.
- ▶ A member will be proposed by the FDA, USA.

WG on Biotechnology-derived products including cell- & gene-based therapies

- ▶ The TSC members agreed on the composition of this WG.
- ▶ Amendments were proposed regarding the contact details of some members.
- ▶ A member will be proposed by Shire.

WG on Orphan drug-development and regulatory processes

- ▶ The TSC members agreed on the composition of this WG.
- ▶ A member will be proposed by Shire.

It can be considered that the composition of the WGs is adopted by the TSC.

Review of the briefing documents prepared for each WGs

The briefing documents for each WGs contain common sections and section specific to each WGs.

General mandate of the WGs

There were no comments on the general mandate of the WGs.

Questions were raised on the flexibility of the topics that could be discussed by the WGs and on the way to deal with overlaps when topics are covered by several WGs. General thinking is that everything is open regarding the topics of interest for the rare disease community. Whenever needed, common teleconferences would be organized between two WGs, composition of the WGs may change with time, several WGs could be merged to create a new one, and others could be cancelled whether they achieved their goal or got to an end. Some amendments or adaptations could also be suggested by the WG.

Composition and meetings section

There were no comments on those sections, which are extracted from the IRDiRC policies & guidelines/Governance document.

'WG on Biomarkers for disease progression and therapy response' briefing document

It was raised that the above-mentioned issue on overlap of topics of interest is particularly crucial here, as the discussion would be mainly therapeutically-orientated in this WG which is quite different from

the diagnosis aspects linked to biomarkers. Collaboration has to be foreseen between the relevant WGs and will be facilitated by the full access of each WG members to the meeting reports from other WGs.

Additionally, the TSC members that will not participate to this WG offered their support to the TSC representative.

'WG on Chemically-derived products including repurposing' briefing document

No comments were made on this document.

'WG on Biotechnology-derived products including cell- & gene-based therapies' briefing document

No comments were made on this document.

'WG on Orphan drug-development and regulatory processes' briefing document

The discussion focused on how this WG might have the best added-value and on what could be the IRDiRC contribution with regard to the current changes of the regulatory process, as for example the gene therapies regulation. It was argued that there are certainly other opportunities to advise or suggest changes for the regulatory process than the ones proposed in the list and that there is a room to get the voice of the academic researchers, actively working in the R&D process and so, closely dealing with regulators. Proposals for new members to be included in this WG have to be sent to the Scientific Secretariat.

4. Working procedure and timelines for the TSC taking into account the feedback from TSC WG.

- ▶ The timeline proposed was adopted.
- ▶ The invitation to join the WGs will be sent before the end of the week by the Scientific Secretariat and members will be asked to confirm or refuse to participate within ten days. Teleconferences will then be organized in November 2013 and January 2014.
- ▶ The purpose of the next TSC teleconference planned in January 2014 will be to discuss the reports of the WGs teleconferences, to elaborate recommendations to be presented to the Executive Committee, and the prepare guidance for the WGs to help them to re-focus strategically for the next steps.
- ▶ Assistance was once again proposed by the TSC members to ease the task of those that are TSC representatives in the WGs in order to have proper material to discuss in January.
- ▶ A Doodle will be launched soon in order to find a date for the next TSC teleconference.

5. Next TSC meeting: location and date

The next TSC meeting is foreseen in March/April 2014. As the last two meetings were held in Europe, it is suggested that the next one could be in the USA in order to easier participation of the NIH members with international travelling restriction. Volunteers are needed to host this meeting.