

## Meeting report series

### Report of the 2nd Therapies Scientific Committee (TSC) Meeting

Dublin, Mespil Hotel

15 April 2013

#### Participants

Prof Josep Torrent I Farnell, Barcelona, Spain

Prof Gert-Jan Van Ommen, Leiden, the Netherlands

Dr Giles Campion, Leiden, the Netherlands

Dr Adam Heathfield, Sandwich, UK

Dr Maria Mavris, Paris, France

Dr Fulvio Mavilio, Evry, France

Dr Luigi Naldini, Milan, Italy

Dr Christine Colvis, Bethesda, USA (WG Repurposing + small molecules)

Dr Lucia Faccio, San Raffaele, Italy (WG Regulatory aspects and bottlenecks)

Dr Virginie Hivert, Scientific secretariat, France

#### Apologies

Dr John Mc Kew, Bethesda, USA

Dr Elizabeth Mc Neil, Bethesda, USA

Dr Glen Nuckolls, Bethesda, USA

Dr Melissa Parisi, Bethesda, USA

Dr Jack Welch, Bethesda, USA

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#### Agenda

1. Review of the IRDiRC Policies and Guidelines
2. Presentation of the IRDiRC Scientific Secretariat
3. Roadmap of the Committee
4. Finalisation of the Working Group composition and definition of their mandate

## EXECUTIVE SUMMARY

The second meeting of the IRDiRC Therapies Scientific Committee (TSC) took place in Dublin on 15 April 2013. This meeting was organized at Mespil Hotel, Dublin.

Among the participants were seven members of the TSC including the chair and two members of related Working Groups.

This meeting took place right after the Executive Committee Meeting that the chairs of the three Scientific Committees attended. It was reported that the Executive Committee reviewed and adopted the Policies and Guidelines document earlier this afternoon.

The organization, role and budget of the Scientific Secretariat were presented.

It was decided to invite representatives from the industry, FDA and EMA to further complete the Working groups. The relevance of the four working groups were discussed in length but it was finally agreed to keep the same groups as previously considered, but to rename two WG as following:

- ▶ 'Advanced therapies' to 'Cell & Gene based Therapies'.
- ▶ 'Small molecules + repurposing' to 'Innovative orphan medicines: small molecules and repurposing'.

## REPORT

### Introduction

The Chair of the TSC and the Chair of the Diagnostic Scientific Committee agreed that it would be useful that the two Committees be joined to discuss the three first points on the agenda.

All the participants were invited to present themselves.

### 1. Review of the IRDiRC Policies and Guidelines

The chair of the TSC presented briefly the IRDiRC governance structure and the role of the Scientific Secretariat.

He also gave feedback about the discussion held during the Executive Committee Meeting. The Policies and Guidelines have been extensively reviewed by the Executive Committee and were due to be adopted at the end of the Meeting previously mentioned. A clean and final copy of the Policies and Guidelines should be sent shortly to the participants by the Scientific Secretariat, taking into account the last revisions asked by the Executive Committee. For example, one issue has been raised regarding the use of the wording 'patients' that will be replaced by 'individuals affected by a disease'. On a global point of view, modifications have been realized in the way the Policies and Guidelines are presented and emphasis has been put on the mandates applicable to each Committees.

Part of the mandate of the TSC is coordination within TSC Working Groups and collaboration with Diagnostic SC and Interdisciplinary SC. How this connection will be practically managed has to be further refined by the Scientific Committees and by the IRDiRC Scientific Secretariat. It was considered convenient, if feasible, that TSC and DSC will try to schedule a face-to-face meeting in the near future.

Among the actions of the TSC is also to finalize the list of members of the four Working Groups and to revise their mandates. There should be an efficient connection between the Groups in order to put in place high-level collaboration and save utmost time.

### 2. Presentation of the Scientific Secretariat of IRDiRC (Support-IRDiRC)

The Scientific Secretariat was introduced by two of its members. The three Work Packages of the project Support-IRDiRC (FP7-funded project) were presented (Implementation, Cooperation and Dissemination), along with the budget, the role of the IRDiRC Committees and the IRDiRC Scientific Secretariat.

Several questions were raised about the internal dissemination and the secured access to data for preliminary results, and also to the availability of the list of research projects to be considered to fit with the IRDiRC requirements and funded by the Research Agencies, members of IRDiRC.

The Scientific Secretariat will provide the TSC and the other Committees with the preparatory documents and preliminary analysis necessary to the discussions. Effective analysis of the state-of-the-

art and the trends in the field of rare diseases research should be possible as all the research projects gathered by the Scientific Secretariat will also be registered within the Orphanet database where they will be tagged specifically and linked to the relevant information, including the classification of rare diseases and the type of research project.

### **3. Road map of the Committee**

The road map of the Committees, defining the deliverables and milestones, should have been discussed during the Executive Committee Meeting. The main objectives are to pave the road for discussions, actions and relationship between the different entities from the Working groups to the Executive Committee, and also ensuring collaboration with the two other Scientific Committees. It will be further discussed at the next TSC meeting once it has been circulated by the Scientific Secretariat.

### **4. Finalisation of the Working Group composition and definition of their mandate**

The first issue was to go through the mandate of the Working Groups. The work plan has to be established for the next 2-3 years as the TSC should guide the Working Groups onto the discussions to be held and the goals to be reached. There is no budget for face-to-face meeting for the Working Groups, only web-casting, teleconference or video conferences are foreseen.

Several general issues were discussed regarding the name, scope and composition of the Working Groups.

- ▶ A TSC member has to be appointed in each Working Groups to ensure consistency and dissemination.
- ▶ There is a need of someone from the USA in the WG 'Advanced therapies'. A letter will be sent to the Executive Committee to ask for the appointment of someone from the FDA specialized into biologics.
- ▶ The wording 'Advanced Therapies' is not convenient as it is use only in the EU. Therefore, at the end of the discussion, it was agreed to replace the label of 'Advanced therapies' by 'Cell & Gene based Therapies'.
- ▶ The Working Group 'Repurposing' could have additional people from the industry sector. The name of the WG was proposed to be modified to 'Innovative orphan medicines: small molecules and repurposing'. It was noted that the advances in oncology, which include many substances that interact with specific therapeutic target, are also included in this WG.
- ▶ It could be interesting to invite people from the two FP7-funded projects, NeurOmics and EuRenOmics.
- ▶ The interaction of the WG 'Regulatory aspects and bottlenecks' with the other TSC WG are highly encouraged.

The TSC discussed in length the relevance of the four Working Groups as on the one hand, the field of chemical and biotech innovation is not covered by any of the Groups. The possibility to merge or re-allocate some of the topics has been explored ('Repurposing' and 'Advanced Therapies' together; two

separate Groups dealing with ‘Molecules’ aspects, ie ‘New Therapies’ and ‘Re-purposing’; ‘New Therapies/Re-purposing’ instead of ‘Re-purposing’ to take into account the innovation...). It has been finally agreed to keep the same groups as previously considered, but to rename two WG as following:

- ▶ ‘Advanced therapies’ to ‘Cell & Gene based Therapies’.
- ▶ ‘Small molecules + repurposing’ to ‘Innovative orphan medicines: small molecules and repurposing’.

The proposed lists of members of the Working Groups were reviewed by the TSC members and many changes were considered.

An update version of the WG composition will be circulated to the TSC members for approval.

To ease the work of the Groups and the follow-up by the TSC, it will be necessary to consider also face-to-face meetings and it should be investigated whether the hosting institution could sometimes pay for the travel & accommodation of the participants to the Working Groups.

### **Main deliverables**

- ▶ A final and clean version of the Policies and Guidelines will be circulated soon.
- ▶ The roadmap has to be issued.
- ▶ A conference call is foreseen in September, a Doodle has to be sent to the participants.
- ▶ A face-to-face meeting has to be organized in October or November; a Doodle has to be sent to the participants. The venue has to be determined according the proposals and to the budget.
- ▶ The report of the TSC meeting will be drafted within one week and circulated to the Chair of the Committee before the dissemination to the members of the TSC.