

Meeting report series

Report of the 12th Interdisciplinary Scientific Committee Meeting

Teleconference
8 February 2016

Participants

Prof Hanns Lochmüller, Newcastle, UK (Chair)
Ms Gema Chicano, Murcia, Spain
Prof Jack Goldblatt, Perth, Australia
Prof Bartha Maria Knoppers, Montreal, Canada
Dr Petra Kaufmann, Bethesda, USA
Dr Jeffrey Krischer, Tampa, USA
Ms Samantha Parker, Paris, France
Dr Domenica Taruscio, Roma, Italy

Dr Lilian Lau, Scientific Secretariat, Paris, France
Dr Anneliene Jonker, Scientific Secretariat, Paris, France

Apologies

Dr Angel Carracedo, Santiago de Compostela, Spain
Dr Stephen Groft, Bethesda, USA
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Agenda

1. Welcome and introduction of new member
2. Paragraph on additional expertise needs in the ISC
3. Identify priorities for the ISC and IRDiRC for the next year
4. Agendas of the Lyon meetings

REPORT

1. Welcome and introduction of new member

The Chair of the Interdisciplinary Scientific Committee (ISC) welcomed its members and introduced Ms Gema Chicano who was voted to be a member of the ISC by the Executive Committee (Exec Comm).

Gema is mother of a patient with ectodermal dysplasia (DE), and the founder and president of the National Asociación de Afectados por Displasia Ectodérmica (AADE) in Spain. She is a practicing lawyer with over twenty years of experience, specialising in administrative, labour law and human rights, and a lecturer at the University of Murcia. She fights for the rights of patients at the administrative and governmental levels and often offers free legal advice to patients, families and other associations in Spain. She also works with a sponsor of a clinical trial for the treatment of ED in collaboration with other patient organisations through an international network for ED. She believes in the importance of research to improve the quality of life of rare diseases patients. She is very pleased to be part of the ISC.

2. Paragraph on health technology assessment (HTA)-related expertise

A document on HTA-related expertise has been prepared and will be edited to a paragraph with just the key requirements. Once the paragraph is agreed upon, the document will be circulated to all members of the ISC to further work on by email before submission to the Exec Comm for approval.

3. Identify priorities for the ISC and IRDiRC for the next year

The topics identified as topic of priority for the ISC/IRDiRC which will be up for further discussion in Lyon:

- ▶ Implementation of Participant Unique Identifier (PUID) Task Force (TF)
- ▶ Best practices in patient group/stakeholder engagement
- ▶ Development of guidelines: from drug licensing to affordability to patients

The Chair of the ISC also asked members who presented TF proposals in Glasgow in June 2015 to revisit and update their ideas if they are still interested in advancing these projects, and put them forward as agenda item for further discussion in Lyon.

3.1 Participant Unique Identifier (PUID) TF

The Exec Comm has approved the implementation of PUID TF, which will be set up as a joint IRDiRC-GA4GH TF. Many of the actors to invite to this TF have already been named in the proposal. A preliminary call is being planned by GA4GH to discuss, among others:

- ▶ Composition of the TF
- ▶ How to take some models forward for use at international level
- ▶ Learning from existing initiatives: success stories and obstacles/difficulties faced

3.2 Best practices in patient group/stakeholder engagement

A proposal of TF to work on best practices in patient group/stakeholder engagement with industry and translational process in drug development was raised in Glasgow, and a discussion with the Chair of the Therapies Sci Comm (TSC) had indicated interest in this topic.

The Chair of the TSC had put through a related proposal to the Exec Comm in Montreal in September 2015, which was also recently reviewed by the members of the TSC for additional feedback. Both draft proposal and feedback documents will be forwarded to the members of the ISC for information.

Moving forward, both the ISC and TSC should work synergistically to support this TF. ISC will engage in direct discussion with TSC to identify if there is any preparatory work required and to agree on content and work plan. This topic will be discussed in both the ISC meeting as well as the joint Sci and Exec Comms meeting in Lyon.

3.3 Development of guidelines: from drug licensing to affordability to patients

One problem/gap that is becoming more and more prominent lies in translation from drug licensing approval to getting agreement to fund the treatments. IRDiRC Sci Comm members need to put its collective mind together and brainstorm for ways to facilitate and push for affordable care to patients, e.g. through adaptive licensing, creation of precompetitive database, cost sharing, etc. IRDiRC should not only push for development of 200 new therapies by 2020 but also availability to patients who need them.

Two proposed ways to move this forward:

- ▶ Discussion/brainstorming in Lyon
- ▶ Proposal of specific actions, e.g. via creation of a TF on this topic

A short document to kickstart and feed discussion in Lyon will be prepared.

4. Agendas of the Lyon meetings

The ISC will meet on the morning of 14 March, and jointly with the other Scientific Committees (Sci Comms) as well as Exec Comm in the afternoon.

Topics for the agenda of the ISC (the agenda will be finalised via email):

- ▶ The ISC
 - The way the committee runs
 - The way the committee interacts with TFs
 - The way “IRDiRC Recommended” is implemented
 - Call of interest to co-chair the ISC
- ▶ Priorities for the ISC
 - Implementation of Participant Unique Identifier (PUID) Task Force (TF)
 - Best practices in patient group/stakeholder engagement

- Development of guidelines: from drug licensing to affordability to patients
- ▶ RARE-Bestpractices
- ▶ The Toolkit Project (this will also be a topic for the joint meeting)

4.1 RARE-Bestpractices (RBP)

In the framework of the EU project RBP (<http://www.rarebestpractices.eu/>), the European Commission specifically asked for the inclusion of a work package (WP) devoted to the collaboration and links between RBP and IRDiRC, i.e. WP7. Moreover, among RBP advisory board members are some who are also involved in IRDiRC. The RBP project is scheduled to conclude at the end of 2016, and its investigators intend to ask for an extension. The goals and objectives of RBP can be found on http://www.rarebestpractices.eu/pagine-1-project_description.

Two public databases have been built through the RBP project, which are available for use and input:

- ▶ RareGUIDELINES (<http://www.rbpguidelines.eu/>), a database of rare disease guidelines on 43 disease topics of which their quality was appraised using AGREE II instrument
- ▶ RareGAP (<http://www.rbpresearch.eu/>), a database of validated research recommendations for diagnosis and treatment of rare diseases identified from systematic reviews; this is a tool which institutions and funding agencies can use to identify gaps in research and structure their calls

A document listing specific suggestions on how IRDiRC can interact with the RBP will be prepared and sent to the Sci Sec for subsequent circulation as part of the set of preparatory documents of the ISC Lyon meeting.

4.2 The Toolkit Project

The Toolkit Project was recently launched in the US which takes stock of tools developed by various organisations that would aid patient engagement in drug and therapeutic development process. Information about this project will be shared and feedback will be sought on inclusion of rare diseases-related resources that should be aware of and included.

This project will be presented to the ISC during its morning meeting in Lyon, and to the larger IRDiRC committees in the joint meeting in the afternoon.

5. Main action points

- ▶ Edit and review short paragraph on HTA-related expertise
- ▶ Discuss with the TSC on best practices in patient engagement
- ▶ Develop the idea on guidelines to improve drug affordability to patients
- ▶ Identify how IRDiRC can interact with RBP and vice versa