

Meeting report series

Report of the 5th IRDiRC Patient Advocates Constituent Committee Meeting

Teleconference

May 7, 2018

Participants

Sharon Terry, Genetic Alliance, USA (Chair Patient Advocates Constituent Committee (PACC))
Paloma Tejada, Rare Disease International, Spain
Maureen McArthur, Global Genes, USA
Virginie Bros-Facer, EURORDIS-Rare Diseases Europe, France
Nicole Millis, Rare Voices, Australia
Harsha Rajasimba, ORDI-Organization for Rare Diseases, India/USA
Durhane Wong-Rieger, Canadian Organization for Rare Disorders, Canada
Yukiko Nishimura, Advocacy Service for Rare and Intractable Diseases' multi-stakeholders, Japan
Nicole Boyce, Global Genes, USA
Ritu Jain, Rare Disease International, Singapore
Eda Selebasto, Botswana Organisation for Rare Disease, Botswana

Christine Cutillo, National Center for Advancing Translational Sciences, NCATS/NIH, USA
Marlène Jagut, Paris, France – Scientific Secretariat, France
Anneliene Jonker, Paris, France – Scientific Secretariat, France
Lilian Lau, Paris, France – Scientific Secretariat, France
Anne-Laure Pham Hung d'Alexandry d'Orengiani, Paris, France – Scientific Secretariat, France

Apologies

Ramaiah Muthyala, Indian Organization for Rare Diseases, India
Kelly du Plessis, Rare Diseases South Africa
Kevin Huang, Chinese Organization for Rare Disorders (CORD)
Peter Saltonstall, National Organization for Rare Disorders (NORD)

Agenda

1. Introductions

2. Discussion of patient advocacy organization membership criteria
3. Preparation for discussion on the IRDiRC Roadmap at the F2F meeting
- Action B: Identify barriers to patient participation in RD R&D recommendations to remove them
- Action F: Issue position statement including specific recommendations on:
 - Model for applying Goal 2 (therapy development) internationally
 - Model for inclusion of patients' perspectives in that therapy development

REPORT

The Chair of the Patient Advocates Constituent Committee (PACC) thanked and welcomed participating members to the teleconference, aimed at discussing the patient advocacy organization membership criteria and to prepare the discussion of the PACC activities from the IRDiRC roadmap in Vienna. An agenda with discussion points will be sent ahead of the meeting to allow reflection and a proactive meeting in Vienna.

1. Discussion of patient advocacy organization membership criteria

The main discussion for the past several months was how to accurately define the criteria for umbrella patient advocacy groups to join IRDiRC and therefore the PACC, given regards to the diversity of organizations worldwide, both in their scope, goals, and activities and the elements they can contribute to IRDiRC's mission.

Elements to keep in mind:

- ▶ Present governance indicates that *“To be considered as an IRDiRC Patient Advocacy Group member, the umbrella organization must be a patient organization representing broad patients’ interests for all rare diseases in at least one country or larger area”*
- ▶ Also, to focus on the main goal: being useful to IRDiRC and not only promote own organizations or get more visibility

Suggested additions to Governance verbiage were discussed.

2. Preparation for discussing the IRDiRC Roadmap at the F2F meeting

2.1 Action B: Identify barrier to patient participation in RD R&D and recommendations to remove them

Action B has been discussed previously, but it is expected that the Vienna meeting will be a good opportunity to really move forward. So far, PACC members have indicated a large difference in patient participation in rare diseases research and development worldwide.

- ▶ A detailed agenda, with discussion points, will be sent to all, prior to Vienna's meeting
 - To address action B, a survey will be sent to the global community in rare diseases to figure out challenges ahead. What needs to be reflected upon:
 - Who is the target audience? Who should it reach?
 - How can we address the survey to make sure to get developing nations/regions opinions, ideas and concerns? (Where there is sometimes no access to research or no research at all)

- What are simple actions we can take to alleviate those barriers, for each concerned region?
- How can this survey meet the need of developing countries/regions?
 - What can this survey do for my region?
- Those questions will be raised during the meeting in Vienna and participants are welcomed to come with ideas that concern their respective regions. If TSC members cannot attend the meeting, they are most welcome to send in their comments.

→ PACC members are asked to come to Vienna with ideas and reflections on action B, regarding their specific region or country.

2.2 Action F: Issue position statement including specific recommendations on a model for applying Goal 1 (therapy development internationally and a model for inclusion of patients' perspectives in that therapy development

- ▶ In order to make a statement about action F, action B need to be complete.
 - Nonetheless, there may be simple statements that could be discussed, for the inclusion of patients' perspectives in therapy development that are already known and that could be brought on the table during the Vienna meeting.

→ PACC members are asked to come to Vienna with ideas and reflections on action F

Main action points

- ▶ Ideas and reflection on action B for each PACC member's region/country
- ▶ Brainstorm on action F