

Meeting report series

Report of the 4th IRDiRC Patient Advocates Constituent Committee Meeting

Teleconference
January 26, 2018

Participants

Sharon Terry, Genetic Alliance, USA (Chair Patient Advocates Constituent Committee (PACC))
Virginie Bros-Facer, EURORDIS-Rare Diseases Europe, France
Ramaiah Muthyala, Indian Organization for Rare Diseases, India
Yukiko Nishimura, Advocacy Service for Rare and Intractable Diseases' multi-stakeholders, Japan
Durhane Wong-Rieger, Canadian Organization for Rare Disorders, Canada

Christine Cutillo, National Center for Advancing Translational Sciences, NCATS/NIH, USA
Marlène Jagut, Paris, France – Scientific Secretariat (Sci Sec), France
Anneliëne Jonker, Paris, France – Scientific Secretariat, France
Lilian Lau, Paris, France – Scientific Secretariat, France

Apologies

Kelly du Plessis, Rare Diseases South Africa
Kevin Huang, Chinese Organization for Rare Disorders (CORD)
Nicole Millis, Rare Voices Australia
Peter Saltonstall, National Organization for Rare Disorders (NORD)

Agenda

1. Introduction of PACC Representatives
2. Discussion on the IRDiRC Roadmap
 - 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
3. Discussion about the election for Vice-Chair Patient Advocates Constituent Committee

REPORT

The Chair of the Patient Advocates Constituent Committee (PACC) thanked and welcomed participating members to the teleconference, aimed at discussing the PACC actions that are part of the IRDiRC Roadmap 2018, and discussing the upcoming election for PACC Vice Chair.

1. Introduction of PACC Representatives

Representatives present on the call were asked to introduce themselves, and state a development or activity of their organization they were excited about.

Durhane Wong-Rieger

- ▶ President and CEO of the Canadian Organization for Rare Disorders
- ▶ Activity
 - Organization of Rare Disease Day
 - Major change in policy issues, in particular for drug pricing, which will hopefully lead to incentives for pharmaceutical companies to bring drugs to Canada in a timely fashion
 - Launch of rare disease framework

Ramaiah Muthyala

- ▶ Founder of I-ORD
- ▶ Activity
 - Attention at political level for rare diseases in India
 - Organization of Rare Disease Day, in which several high placed Indian politicians will participate, such as India's Prime Minister
 - Involvement of RDI

Virginie Bros-Facer

- ▶ Scientific Director at EURORDIS-Rare Diseases Europe, replacing Béatrice de Montleau as representative for EURORDIS-Rare Diseases Europe
- ▶ Activity
 - Preparation of European Joint Programme (EJP) on Rare Diseases, where EURORDIS will be in the lead of all activities for pillar 3, which is about training for patients, clinicians and scientists

Yukiko Nishimura

- ▶ President of NPO ASrid, representing rare and intractable diseases
- ▶ Activity
 - ASrid is the secretarial office of Japan's Rare Disease Day, managing over 40 events all over Japan

- Development of a workshop on ELSI issues, with participation of multiple stakeholders, which has led to an ELSI Roadmap
- Presentation scheduled at AMED, on collaboration between patients and researchers

Sharon Terry

- ▶ President of Genetic Alliance
- ▶ Activity
 - Moving PCORnet into a people-centered network, called the People-Centered Research Foundation, collaborating with NCATS

Nicole Millis (comment by email)

- ▶ Executive Officer for Rare Voices Australia
- ▶ Activity
 - The Australian Health Ministry has made a critical announcement, dedicating 69 M AUS dollar to rare diseases and rare cancer
 - The Australian government has made rare diseases an undoing priority, for the first time

2. Discussion on IRDiRC Roadmap

IRDiRC recently set out its Roadmap for the new year, in a collaborative and efficient process. Two actions of the PACC have been included in this Roadmap, Action B and Action F. Both actions address all the three IRDiRC goals.

2.1 Action B

- ▶ First action of the PACC, that is aimed at the identification of barriers to patient participation in RD R&D recommendations to remove them
 - This topic will be investigated by creating a multi-stakeholder survey, that will be send to patients and other stakeholders
 - The expected outcome is a set of recommendations on how to empower and enable patient participation in rare diseases research
 - The action will start in February, with the design of the survey, the translation of the survey, and the determination of the target audience, followed by a survey period, and a workshop in which the outcomes are discussed. Based on this discussion, recommendations are drafted and an article is expected to be written.

Prior to setting up this action, it is time to reflect on several key elements:

- ▶ Overall
 - Who is the audience for the results? Funders? Policy makers? Researchers? All?

- If asked the question what barriers patient encounter to participate in rare diseases R&D, not even all people will understand the question. For some people the idea that they can participate in R&D is still not clear, due to different circumstances.
- Current resources are mainly focused in the western world, which means a great deal of the world is neglected. In fact, greater numbers of people do not have access to research than do.
- At present, there are several problems:
 - There is no rare disease research going on in many parts of the world;
 - If there is research, people do not necessarily know how they can be involved, so emphasis should be on the question how much people know they can be involved;
 - Clinicians do not always know how to involve patients in their research
- ▶ **Timeline**
 - Time required to set up the survey might need to be extended, dependent on the number of surveys that will be created.
- ▶ **Survey**
 - Should be translated in “lay” language, making the survey as accessible as possible
 - PACC members should be highly involved in the design of the survey, to make it accessible
 - If the final desired outcome is to remove the barriers, there are two steps:
 - What opportunities are out there to participate in R&D?
 - What are the barriers to overcome the participation?
- ▶ **Outcome**
 - What is envisaged by the recommendations; should these be delineated by target audience (developed vs developing nations..), or should it be a global set of recommendations, thus being less dedicated to specific stakeholder groups, populations or region. Recommendations could be set out in primary and secondary target audiences.
 - If separate set of recommendations, this will further emphasize the separation in society, geography, economy, etc creating further disparities, not empowering everyone equally
 - If joint, this might set the bar too low in order to fit everyone, thereby not sufficiently contributing to the development of the field. A joint set of recommendations might however lead to a better integration of different populations, in which they can learn from each other
 - Final goal needs to be determined in order to set out a vision (no barrier for R&D participation), then outcomes can be split by diseases group, geography, profession, economy
 - Funders should certainly be a target audience for the recommendations, in order to further stimulate patient participation

2.2 Action F

- ▶ Second action of the PACC, to create a position statement including specific recommendations on
 - Model for applying Goal 2 (therapy development) internationally, thereby aimed at stimulating drug development equally worldwide
 - Model for inclusion of patients' perspectives in that therapy development
 - The expected outcome is a recommendations or position paper to nations re: multi-stakeholder collaboration on regulatory and therapy development pathways
 - This action is scheduled to take place after Action B has finished

Prior to setting up this action, it is time to reflect on several key elements:

- ▶ Overall
 - IRDiRC as a whole is not a policy or lobbying organization, so therefore it cannot, in its entirety set out a position statement
 - Outcome can therefore either be a position paper on behalf of the PACC or a recommendations paper on behalf of IRDiRC
- ▶ Timeline
 - This action will take place after completion of Action B, but preliminary work can get started prior to the official start of this action, perhaps simple discussion to prepare, and maybe a preliminary statement
- ▶ Outcome
 - First a statement from PACC to rest of IRDiRC, then as next step a full position paper
 - Major question: where is drug development happening, outside the of EMA and FDA?

A next call will be organized to start brainstorming on this action.

3. Discussion about the election for Vice-Chair Patient Advocates Constituent Committee

With the departure of Béatrice de Montleau – EURORDIS-Rare Diseases Europe, the PACC has no longer a Vice Chair. This therefore represents an opportunity for all PACC members to elect their new Vice Chair. As the PACC has a diverse membership and is currently still growing, it would be great if the PACC Chair and Vice Chair represent the PACC's global membership.

- ▶ Procedure
 - Host a call for nominations, in which we ask PACC members that are interested in the position of Vice-Chair to send their CV and a short paragraph stating their motivation (total 1A4 text) (1-2 weeks)
 - Sci Sec will compile incoming nominations (1 week)
 - Compiled nominations will be send it to all PACC members for an anonymous vote (1-2 weeks)
 - The Sci Sec will announce the selected Vice Chair shortly after the closing of the vote

Main deliverables

- ▶ Organize the next PACC TC to brainstorm further on action F
- ▶ (Self-)Nominate members that are interested in the Vice Chair position of PACC