

## Meeting report series

### Report of the 10th Patient Advocacy Constituent Committee Meeting

Teleconference  
November 5, 2018

#### Participants

Sharon Terry, Genetic Alliance, USA – Patient Advocacy Constituent Committee (PACC) Chair

Vanessa Boulanger, NORD, USA

Virginie Bros-Facer, EURORDIS-Rare Diseases Europe, France

Ritu Jain, Rare Diseases International, Singapore

Prasanna Kumar Shirol, ORDI, India

Maureen Mc Arthur, Global Genes, USA

Durhane Wong-Rieger, CORD, Canada

Christine Cutillo, NCATS, USA

Anneliene Jonker, Scientific Secretariat, France

Lilian Lau, Scientific Secretariat, France

Anne-Laure Pham Hung d’Alexandry d’Orengiani, Scientific Secretariat, France

#### Apologies

Yukiko Nishimura, ASrid, Japan – PACC Vice Chair

Kevin Huang, CORD, China

Nicole Millis, RVA, Australia

Ramaiah Muthyala, I-ORD, India

Kelly du Plessis, RDSA, South Africa

Eda Selebasto, BORDIS, Botswana

#### Agenda

1. Next steps Activity B – Identification of barriers to patient participation in RD research and recommendations to remove them
  - a. Goals Activity B
  - b. Translation focus groups questions
  - c. Budget focus groups
  - d. Format focus groups
  - e. Limitations focus groups
2. Agenda Brussels PACC face-to-face meeting

## REPORT

### 1. Next steps Activity B – Identification of barriers to patient participation in RD research and recommendations to remove them

#### 1.1 Goal Activity B

- ▶ Objective: Leverage IRDiRC's stakeholder and geographic representation to conduct a complementary environmental scan of barriers to and recommendations for patient participation in RD research. To accomplish this, recommendations will be developed to
  - (1) determine alignment of current efforts with needs
  - (2) facilitate better patient engagement across geographic areas with shared resources
  - (3) determine strategic areas for new funding initiatives
  - (4) inform future activities of IRDiRC.
- ▶ This project will allow PACC members to get beyond their own opinions, and go towards something that is larger, and allow to indicate where further research is needed.
- ▶ It will address a critical part of research that is currently not yet done, or at the very least start to tackle that part of research.
- ▶ It will allow the PACC to indicate where further funding is needed, thereby providing an opportunity to advise future activities and funding calls of IRDiRC / IRDiRC members, and effectively allowing this to influence the direction of IRDiRC.

#### 1.2 Translation focus group questions

In order to assist all PACC members in their preparation of the focus groups, the questions and educational slides will be translated. Currently, quotes for translation have been asked for the following languages:

- ▶ Chinese
- ▶ French
- ▶ Japanese
- ▶ Spanish

While this does not include all the different languages members work in, it is suggested to only get translation for the languages used in the place the focus group is organized in, in the language of the focus group facilitator and participants. After discussion, PACC members request also the following translation

- ▶ Hindi
- ▶ German (if funding becomes available for the travel of focus group participants)
- ▶ Italian (if funding becomes available for the travel of focus group participants)
- ▶ Portuguese (if funding becomes available for the travel of focus group participants)

Ideally, PACC members also request that budget will be made available for the return translation, from the focus group discussion into English, in order to have reliable and comparable resulting data.

[Post-meeting note: it appears that subcontracting will remain possible in 2019, therefore it might be possible to also pay for translation back into English]

### 1.3 Budget focus groups

At present, there is no budget for travel of the participants in the focus groups, therefore the different patient organizations are fully in charge of the focus group participants. Several PACC members indicate that this will be difficult in their region due to the cost that their organization cannot bear.

Budget is not available, as at the present there is only budget for one Task Force gathering, not 13 individual focus group gatherings. Budget from the next Sci Sec contract for any activity can only potentially be made available if included in the work plan of the following year, the deadline falling around 7 months prior to the start of the new year.

Several PACC members bring up that it will be very difficult for them to invite people to come to their offices, as they cannot cover the cost of travel. While it is not likely that funding could become available, PACC members are asked to submit their financial needs for this activity, to map out all needs.

→ Send in financial needs, in case funding could become available.

### 1.4 Format focus groups

The focus groups are ideally small, 8-12 people, so that there is space for an open discussion with every participant.

- ▶ Focus groups will last for approximately 2 hours, and will be led by a facilitator, the different PACC members.
- ▶ Focus group will unfortunately be limited by participants that are able to travel to the patient advocacy group's headquarters, which unfortunately will lead to selecting a "sample of convenience."
- ▶ Participants should not be "super-experienced" in patient advocacy, but rather "normal" participants.
  - Mix of participants that are and are not involved in research. Participants might not be involved, but might follow the development in research closely, and both categories of participants are therefore of great value.
  - Participants should not already be involved in policy groups
- ▶ Ideally, more than 8-12 participants should be invited, due to potential no-shows

→ Determine who to be invited to the focus groups (i.e. the characteristics of the people to invite)

### 1.5 Limitations focus groups

Due to the fact that focus groups only have a limited participation, and that they are held close to the facilitator, there are several limitations that should be considered:

- ▶ The facilitator is “per definition” biased, as all PACC members support research
- ▶ Focus group might reach only those that do have access to and knowledge about research, thereby not reaching the whole spectrum of potential participants and opinions (participants with no knowledge and not involved vs with knowledge but not involved vs with knowledge and involved
  - Question possibility: even if research is accessible, what are the barriers?
  - Benefit of research vs awareness towards research
- ▶ Focus group might be too broad, therefore not reaching “new” conclusions
  - Research outcomes might be too limited, therefore not reaching correct or misleading conclusions
- ▶ Possibility to pre-define the questions further, but this might lead to:
  - Too different answers depending on the focus group location
  - Further limiting the research, thereby the results becoming even more predictable

→ Send in what to ask the participants (“what do you think you get out of the focus group”).

## 2. Agenda Brussels PACC face-to-face meeting

The PACC breakout session during the IRDiRC Consortium Assembly meeting in Brussels, Belgium will take place on December 6, 2018, in the afternoon. The discussion will be led by the PACC Vice Chair, and the Chair will attend via teleconference.

Suggested topics for the agenda are:

- ▶ Next steps Activity B – Identification of barriers to patient participation in RD research and recommendations to remove them
  - Feedback Task Force discussion
  - Exchange on organization and format focus groups
  - Discussion on final questions focus groups
- ▶ Activity F: Draft Position Statement
  - Refinement position statement
- ▶ PACC work plan 2019

→ Send in comments on the agenda

### Next steps and actions

- ▶ Send in financial needs for the focus groups, in case funding could become available
- ▶ Determine who to be invited to the focus groups (i.e. the characteristics of the people to invite)
- ▶ Send in what to ask the participants (what do you think you get out of the focus group)
- ▶ Send in comments on the agenda for the Brussels PACC breakout session