

## Meeting report series

### Report of the 7th IRDiRC Patient Advocates Constituent Committee Meeting

Teleconference

June 13, 2018

#### Participants

##### Members

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

Maureen McArthur, Global Genes, USA

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

Prasanna Kumar Shirol, ORDI-Organization for Rare Diseases, India/USA

Lisa Phelps, National Organization for Rare Disorders (NORD)

Harsha Rajasimba, ORDI-Organization for Rare Diseases, India/USA

Durhane Wong-Rieger, Canadian Organization for Rare Disorders, Canada

Ritu Jain, Rare Disease International, Singapore

##### Guests

Nicole Boice, Global Genes, USA

Nicola Page, Rare Diseases South Africa

##### Secretariat

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

Kevin Huang, Chinese Organization for Rare Disorders (CORD)

Nicole Millis, Rare Voices, Australia

Ramaiah Muthyala, Indian Organization for Rare Diseases, India

Yukiko Nishimura, Advocacy Service for Rare and Intractable Diseases’ multi-stakeholders, Japan – Vice Chair

Eda Selebasto, Botswana Organization for Rare Disease, Botswana

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## Agenda

1. Recap of PACC Vienna breakout meeting
2. Discuss and finalize proposal for Activity B (*Identify barriers to patient participation in RD R&D and recommendations to remove them*) – Document 1
3. Finalize updated PACC membership criteria – Document 2
4. Any other business

## REPORT

The Chair of the Patient Advocates Constituent Committee (PACC) thanked and welcomed participating members to the teleconference, aimed at providing a debrief of the IRDiRC Vienna meeting, discussing the proposal for Activity B and validating the updated patient advocacy organization membership criteria.

### 1. Recap of PACC Vienna breakout meeting

The discussion at the Vienna PACC break-out meeting was mostly focused on reshaping Activity B (*Identify barriers to patient participation in RD R&D and recommendations to remove them*).

- ▶ The activity was adapted based on ideas and suggestions from all members present at the meeting. Based on the discussion, a proposal was generated that was presented later on during the Vienna meeting.
- ▶ Some time was also spent discussing Activity F (*Position statement with recommendations on model for applying Goal 2 internationally*) and how some questions focused around international therapy development and the inclusion of patients' perspectives in that therapy development could potentially be incorporated into the Activity B effort.

At the Consortium Assembly session after the breakout meetings, all Committees reported back from their respective breakout sessions:

- ▶ PACC Vice Chair presented the updated Activity B.
- ▶ FCC Chair and TSC Chair received approval on Activity A and Activity C, respectively
- ▶ PACC will also move Activity B forward for vote once finalize proposal

### 2. Discuss and finalize proposal for Activity B - Identify barriers to patient participation in RD R&D and recommendations to remove them

Objective of the activity:

- ▶ Leverage IRDiRC's stakeholder and geographic representation to conduct a complementary environmental scan of barriers and recommendations for patient participation in RD research.
- ▶ To accomplish this, recommendations will be developed to:
  - Inform future activities of IRDiRC (note that this was moved first to make explicit the need for this activity to serve IRDiRC)
  - Facilitate better patient engagement across geographic areas with shared resources
  - Determine strategic areas for new funding initiatives
  - Determine alignment of current efforts with needs
- ▶ This activity must serve IRDiRC and its goals at its core, and help other Committees achieve their goals in service of affected individuals and their families

The process and timeline of main steps:

- ▶ Develop proposal (within PACC) – by June 2018
  - Once the proposal is finalized, it will be send for approval to the CA over the summer of 2018
- ▶ Initiate PACC-led Task Force – by Sept 2018
- ▶ Start PACC-led Task Force work to define implementation details - via TC and finalize at F2F, Q3-4 2018
- ▶ Initiate PACC focus groups – Q1 2019
- ▶ Initiate Funders Constituent Committee (FCC) and Companies Constituent Committee (CCC) scan – Q1 2019
- ▶ Initiate Scientific Committees scan to represent academic researchers across jurisdictions – Q1 2019
- ▶ Intermediate review of results from three streams (PACC focus groups, FCC/CCC scan, SCs scan) – Q2 2019
- ▶ Analyze results and develop recommendations – Q3 2019
- ▶ Publish/disseminate recommendations – Q4 2019

#### Discussion on timeline:

- ▶ Total process will take around 1.5 year
  - Timeline is short but manageable
  - There is a bit of flexibility built into the timeline. This is a bit of a strawman proposal since the TF (with appropriate expertise) will define the implementation details in fall 2018 so that it can be appropriately responsive throughout the process.
  - If the PACC wants to influence and support other activities of other Committees, the outcomes of this activity should not come too late
  - Even though the timeline is fairly short, it should still be feasible to get a sufficient number of responses
- ▶ Focus groups
  - Ideally face-to-face whenever feasible, but can be done by teleconference (to be determined by the TF)
  - Important to have standardization in the procedure whenever, even though there will be diversity in the question asked
  - Try to nail down the protocol as soon as possible, through the work of the multidisciplinary TF
  - It is the multidisciplinary TF who will be responsible for defining the explicit implementation details (including protocols, procedures, questions, etc) and detailed budget necessary
- ▶ Overlap
  - Important to recognize overlap with existing other efforts, to best focus this endeavor
  - Make clear that this, in and of itself, is a collaborative effort

#### People involved throughout the Task Force

- ▶ PACC-led TF

- Similar to traditional TF
- Ideally maximum 15 people, in order to allow most efficient interactions
  - Include internal representatives from PACC, FCC, CCC, and SCs
  - Include external experts in methodology, survey, qualitative data analysis, and data protection
  - Members that would like to be on the TF are asked to self-nominate, stating which qualifications they can bring to the table.
- Balance between who is best, and who is dedicated/ willing
- Qualitative data, data protection and/ or survey experts
  - Suggestions welcome. Members are asked to send suggestions to Sharon and Sci Sec, stating names, contact information, and the specifications they bring.
  - Suggestions from Virginie Bros-Facer: expert from Cardiff and expert from Newcastle
- ▶ PACC members
  - Increased effort from each PACC member to hold their umbrella organizations' member focus group and gather input
- ▶ FCC, CCC, and SCs members
  - Each member would need to allocate 30 minutes of interview by Sci Sec + TF member
- ▶ Sci Sec
  - For interviews, increased workload for scheduling and holding interviews with each FCC/CCC/SCs member

Regarding the experts, it is important to realize that they will be engaged on Sci Sec budget, using a sub-contracting mechanism, which is only possible by the end of this current year. Therefore, the experts should deliver the work, and therefore provide the Sci Sec with an invoice, before the end of November 2018.

- PACC members interested are asked to self-nominate for the Activity B TF, stating their qualifications
- PACC members are asked to send in suggestions for methodology, qualitative data, data protection or survey experts to the Chair and Sci Sec, stating their qualifications
- PACC Chair will present the proposal for vote to the IRDiRC CA via email, as it is too long to wait to do it on the next scheduled quarterly call

### **3. Finalize updated PACC membership criteria**

After the previous PACC TC, in which the membership criteria for patient advocacy groups were discussed, these criteria were updated with suggestions on the call.

No additional suggestions were made, and PACC members agreed that this version can be presented for vote to the CA.

→ PACC Chair will present the updated membership criteria for patient advocacy groups for vote to the IRDiRC CA

#### **4. Any other business**

Virginie Bros-Facer is involved in an Innovative Medicines Initiative (IMI) study entitled PARADIGM. PARADIGM's mission is to provide a unique framework that enables structured, effective, meaningful, ethical, innovative, and sustainable patient engagement (PE) and demonstrates the 'return on the engagement' for all players. In the framework of this study, there is currently a survey open for comments, that she asks all PACC members to fill out.

Rare Diseases South Africa shared the progress in the organization of the RareX conference. The Department of Health from the government of South Africa is on board for this initiative, and will participate to the conference, emphasizing the multi-stakeholder involvement.

Several members find the use of abbreviations difficult, and asked the Sci Sec for a list of abbreviations and their acronyms to be send out. Wherever possible, in the work of the PACC, we will spell the acronym out the first time and then resort to its use

→ Prepare list of abbreviations and acronyms

#### **Main action points**

- ▶ Self-nominate to be involved in the Activity B Task Force, stating your specifications for the Task Force
- ▶ Sent in suggestions for qualitative data, data protection or survey experts to the Chair and Sci Sec, stating their qualifications
- ▶ Present Activity Proposal B for vote to the IRDiRC CA
- ▶ Prepare list of abbreviations and acronyms