

Meeting report series

Report of the 11th Patient Advocacy Constituent Committee Meeting

Brussels F2F
December 6, 2018

Participants

Yukiko Nishimura, ASrid, Japan – Vice Chair
Dixie Baker, Martin, Blanck and Associates, USA
Virginie Bros-Facer, EURORDIS-Rare Diseases Europe, France
Natalie Douglas & Maureen Mc Arthur, Global Genes, USA
Virginie Hivert, EURORDIS, France
Ritu Jain, Rare Diseases International, Singapore
Katherine Lambertson, Genetic Alliance, USA
Ramaiah Muthyala, I-ORD, India
Durhane Wong-Rieger, CORD, Canada

Christine Cutillo, NCATS, USA

Apologies

Sharon Terry, Genetic Alliance, USA – Chair
Vanessa Boulanger, NORD, USA
Kelly du Plessis, RDSA, South Africa
Kevin Huang, CORD, China
Prasanna Kumar Shirol, ORDI, India
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Agenda

1. Next steps for: **Activity B** – *Identification of barriers to patient participation in RD research and recommendations to remove them*
2. **Activity F** – Refinement of draft position statement of the PACC
3. Areas of need/potential focus for Consortium in the future

REPORT

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

- Recap of Task Force (TF) discussions, to date
 - Background:
 - Proposal approved in early July
 - Nominations for TF members were collected via CA/PACC/SC members and the public via the IRDiRC website and Twitter account in July-end of September
 - Advancing aspect of IRDiRC Goal 2 (serving therapies to patients with underserved RDs or those without available treatment) – main question: how do we empower and enable patient participation in rare diseases research?
 - To accomplish this, the TF aims to develop recommendations on:
 - Determining alignment of current efforts with needs
 - Facilitating better patient engagement across geographic areas with shared resources
 - Determining strategic areas for new funding initiatives
 - Informing future activities of IRDiRC
 - TF membership:

Type	Name	Affiliation	Expertise	Proposer
External	Anna Ambrosini	Fondazione Telethon, Milan, Italy	Methodology, surveys, qualitative data analysis	Lucia Monaco
	Rebecca Dimond	School of Social Sciences in Cardiff, UK	Expertise in patient, family and professional perspectives as well as qualitative research methods	Virginie Bros-Facer
	Pauline McCormack	Newcastle University	Expertise and interest in social policy and participatory methods specifically patient experiences in translational research and clinical trials, patient participation and bioethics in rare diseases	
	Thomas Morel	University of Leuven	Patient-centered outcomes research and policy, rare disease policy and advocacy lead, regulatory interactions	Self-Nomination
	Gloria Pino-Ramírez	Alianza Iberoamericana de Enfermedades Raras - ALIBER	Psychology, research, patient with Marfan Syndrome	
	Antoine Regnault	Modus Outcomes Ltd	Methodology, quantitative research, qualitative methods, patient-centered outcome research	

Internal Reps	Sharon Terry (PACC, Chair)	Genetic Alliance	
	Katherine Beaverson (CCC)	Pfizer	
	Virginie Bros-Facer (PACC)	EURORDIS	
	Prasanna Kumar Shirol (PACC)	ORDI	
	Ritu Jain (PACC)	RDI	

- TF has already held two calls:
 - Initial call (October 24) summary of discussion:
 - Characterizing patient focus groups
 - Should be cognizant of tremendous diversity in umbrella PAGs
 - Leverage knowledge and expertise of TF members
 - Care needed to not introduce biases
 - Focus group format
 - 8-12 invited participants
 - Standard set of slides as introduction and disclosure of activity
 - Need to sample from two categories of participants:
 - Those who have not been involved in RD research
 - Those who have
 - Should define “participation” beforehand within focus groups
 - Draft v1 questions:
 - Is there a question you would like answered by rare disease research?
 - Have you ever participated in this kind of research?
 - What do you feel about rare disease research? (“If yes” and “If no” follow-up questions)
 - How would you like to find the answer to the question you posed at the beginning?
 - Anything else you would like to share?
 - Suggested reframed questions:
 - Have you ever wondered about research opportunities?
 - Has your physician talked to you about participating in research?
 - Are you aware of opportunities for participating in research?
 - What do you know about research?
 - Need for reframing and finalization
 - Need to refocus questions on barriers perceived within patient involvement (rather than their perceived needs)
 - Due to limited time and resources, exclude questions around basic/animal model research and policies/regulations/legal
 - Broaden scope of questions rather than focus on clinical trials
 - After the call questions were sent to the PACC for their feedback on ideas for focus group implementation since they were slated to run them in initial plan.
 - Second call (November 20) summary of discussion:

- Need to clarify the target participants and the knowledge that we want to gain
 - We don't want to leave people out, but also don't gain much when asking questions such as this in areas without health systems altogether
 - Striking the appropriate balance of targeted participants (and determining how to do so) given the diversity of communities is complex and important.
 - → Feedback from TF/PACC: any knowledge that we create and put in the public domain is going to be useful
- Need resources to reach “hard to reach” groups and participants
 - The budget for next year has already been finalized (due to the EJP RD planning cycle).
- Phased plan approach proposed and agreed upon, so that we can get at the low hanging fruit from the IRDiRC advocates/ reps and their responses can collectively steer the TF in the right direction for collection of data directly from the patients.
 - Phase 1 (2019):
 - Gather feedback on questions of perceived barriers
 - Ask leadership of IRDiRC PAGs and other IRDiRC constituency representatives
 - Create budget for Phase 2 in Q1-2 for submission in Q203
 - Phase 2 (2020):
 - Deeper dive into the tissues
 - Develop protocol and questionnaire/survey
 - Gather feedback directly from the patients and the stakeholders themselves
 - Questions could be tailored according to culture/lexicon but themes could be universal
- TF Chair will need to be determined (from external experts)
 - Next steps
 - TF will be meeting in January to determine next steps for implementing the phased plan
- Feedback from PACC members to provide foundation for evolving TF plan
 - Overall multipronged approach
 - Aiming to perform a scan of PAGS (what works/doesn't), scan of SCs/CCC/FCC (methodologies used for engaging; what works/doesn't; how do they align/not)
 - Then, will be able to create recommendations for how funders (and others) should push research in the “right” direction
 - Suggestion for updated phased approach
 - Phase 1 (2019)
 - Prepare for Phase 2:
 - Finalize co-chairs – 1 from external experts; 1 from PACC
 - Determine how we define “participation” and “research” to set up for Phase 2
 - Determine exact methodology and create budget for Phase 2 in Q1-2 for submission to EJP in Q2-3 (go hand-in-hand)

- Might need to include funds for ethics review board
 - Benchmarking and literature review of existing resources and surveys in this area
 - E.g., Rare Barometer Voices (not published yet, so would have to get internal results); Paradigm (also not published yet, so internal results needed); EUPATI
 - *Timeline – finalize methodology and budget for Phase 2 with proposal by end of April (so that can be sent to the EJP RD in May ahead of June Steering Committee meeting)*
 - Gather initial feedback:
 - From leadership of IRDiRC PAGs (i.e., PACC representatives) and other constituent representatives (i.e., SC, FCC, and CCC representatives)
 - Aim: to gather viewpoints of PAG, academic researcher, and funder perspectives within IRDiRC
 - Ask same set of questions on perceived barriers
 - Publication based on initial results referencing that more inclusive data will be generated/published in 2020
- Phase 2 (2020)
 - Based on initial feedback from Phase 1:
 - Finalize questions to be included in questionnaire/survey (or other methodology determined during Phase 1 prep work)
 - Questions potentially could be tailored according to culture/lexicon (if appropriate based on the determined methodology)
 - Conduct questionnaire/survey:
 - Directly from the patients and stakeholders themselves
 - Aim: to gather representative PAG, academic researcher, and funder perspectives, more writ large
 - Analyze results
 - Publication based on full results
 - Recommendations to:
 - Better align efforts and needs
 - Create list of the suite of methodologies/ strategies successfully used in patient engagement/participation (particularly with developing countries in mind)
 - Facilitate better patient engagement/participation across geographic areas with shared resources
 - Determine strategic areas for new funding initiatives
 - Inform future activities of IRDiRC and the broader rare disease research community
- Phased plan suggestions/recommendations for the TF:
 - Due to updated phasing approach and the need to determine questions/answers first from IRDiRC PAGs and constituency representatives, PACC recommends (if there are volunteers) to add 1-2 more PACC representatives to the TF and having co-chairs.

- Up to the discretion of PACC/SC/FCC/CCC representatives to determine how they would like to answer Phase 1 questions; perhaps give them 1 month to answer the questions (as some wanted to get feedback from their organization)
 - Feedback/initial thoughts about the draft questions:
 - What do we mean by “participation in RD research”? What should the parameters for the TF be? What part should they focus on?
 - In Phase 1, TF should determine how they define both terms for the effort as a whole
 - What do you think the barriers are (to patient participation in RD research)?
 - Seems broad
 - Determine how the literature and others have previously noted as perceived barriers.
 - Is a drop down a good option for such a question?
 - For both questions #1 & 2, be sure to clarify whether the question is asking about barriers to participation in research or barriers to engagement
 - Phase 1 methodology
 - See suggestions above.
 - Phase 2, for focus groups or running a survey in your region, what might it truly entail?
 - Q&A collected from the PACC should be a good basis.
 - Perhaps virtual focus groups might be a cheaper possibility? Others thought that this might bias the information collected.
 - See suggestions above

→ Present/provide recommendations to Act B TF re: thoughts on updated phased approach

→ When scheduling next Act B TF call, remind to send nominations for co-Chairs of Act B TF and develop draft timeline

2. Activity F – Refinement of draft position statement of the PACC

- Background
 - Position statement with recommendations on:
 - Model for applying IRDiRC Goal 2 (therapy development) internationally
 - Model for inclusion of patients’ perspectives in that therapy development
 - The expected outcome is recommendations or a position paper re: multi-stakeholder collaboration on regulatory and therapy development pathways
 - This action was scheduled to take place after Activity B has finished
 - ...but there seemed to be an interest in developing a short, simplified statement without specific recommendations on the application of Goal 2, while the other IRDiRC Committees are working on its implementation strategies
 - Language discussed on September-November PACC calls
- Discussion
 - Even though it is short, make the formatting more dynamic and easier to read (for easy sharing and viewing online)
 - Phenotype might be too scientific and what we actually mean is in parentheses

- Suggest consolidating into single bit: “what is of greatest concern to the affected individual” or “lived experience of the disease”
 - First stated goal (model for inclusion of patients’ perspectives in that therapy development) seems to be lacking from the statement
 - Primarily for sharing via
 - Website
 - Twitter
 - Newsletter
- Potential updated language
 - The Patient Advocates Constituent Committee strongly supports the approval of 1000 new therapies. We recommend that IRDiRC make sure these therapies are available internationally, and that these approvals increase access worldwide, to allow all rare disease patients in all countries around the globe, including developing and emerging nations*, to benefit from these approvals.
 - We also recommend that patients, caregivers, and family members are included in all phases of therapy development. This includes, but is not limited to, input into: *what matters with regard to what is of greatest concern to the affected individual*; the protocol and structure of clinical trial designs, where patients’ perspective in particular is important for trial feasibility; including data sharing policies; lay communication of the patients’ benefit of the trial; recruitment and retention; data analysis; dissemination of results; and implementation into new practice guidelines.
 - * To be defined: developing and emerging nations

→ Send updated language out to PACC for final review (inclusive of suggested change)

3. Areas of need/potential focus for Consortium in the future

- ▶ Data protection solutions/technologies to enable data sharing
 - Interest as potential future PACC activity
- ▶ Data protection regulation
 - How do we ensure that GDPR doesn’t inhibit aspects of RD research? Is there a role for IRDiRC?
- ▶ Encourage and enable research funding in LICs
- ▶ Access
 - Innovative instruments/technologies
 - For data collection (re cost and access)
 - Language translation and/or pictorial representation
- ▶ Other items
 - Ensure to coordinate and collaborate with Pillar 3 of EJP RD (capacity building re: patient involvement in research)
 - Goal 3 – we *need* to improve access/impact
 - Prevention research (of interest to LICs) – referring to premarital screening (i.e., prior to birth) to minimize more distal access/infrastructure issues

4. Any other items

→ Schedule PACC and Activity B TF telecons

Next steps and actions

- ▶ Schedule PACC and Activity B TF telecons
- ▶ When scheduling next Act B TF call, remind to send nominations for co-Chairs of Act B TF and develop draft timeline
- ▶ Present/provide recommendations to Act B TF re: thoughts on updated phased approach
- ▶ Send updated language out to PACC for final review (inclusive of suggested change)