

Meeting report series

Report of the 9th Patient Advocacy Constituent Committee Meeting

Teleconference
October 10, 2018

Participants

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Apologies

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Agenda

1. Next steps Activity B – Identification of barriers to patient participation in RD research and recommendations to remove them
 - a. Updated timeline
 - b. Draft survey question discussion
 - c. Format focus groups
2. Next steps Activity F – Review of strawman’s position statement

REPORT

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

1.1 Updated timeline

- ▶ A timeline was included in the proposal sent to / approved by the Consortium Assembly (CA), which was recently updated and refined, in the context of approaching experts and planning the work for the coming months
 - Some aspects of the proposal need to be finished by the end of 2018, therefore some elements of the proposal were sped up slightly.
 - The PACC Chair will not be able to attend the CA meeting in Brussels, therefore, we do not plan a face-to-face meeting of the Task Force in conjunction to this meeting. A face-to-face meeting will be organized at a later stage.
 - All invitations to Task Force experts were sent out, and most experts have accepted
 - The definition of the design, the questions and implementation details of the survey will be done in the last quarter of 2018

1.2 Draft survey question discussion

In order to set the stage for the focus groups, we will use 2-3 informative slides prior to the start

- ▶ This will allow participants to understand the boundaries of the focus groups
- ▶ 3 educational slides
 - Why we have gathered you here?
 - What is rare disease research?
 - What the International Rare Disease Research Consortium is and who is funding this activity, including the facilitator disclosing whether they are volunteering or paid for facilitating this – and by whom?
- ▶ These slides will be prepared by the Task Force members, but will require review and input of PACC members
 - This will allow participants to better understand the process and to be informed upfront who sponsors this focus group, and what they can expect to get out of the experience

Following the educational slides, the focus group discussion will start. The PACC Chair drafted a number of questions:

- ▶ Is there a question you would like answered by rare disease research?
- ▶ What do you feel about rare disease research?
- ▶ Have you ever participated in this kind of research?
 - If yes, tell us about your experience
 - How did you hear about it?
 - Did you understand what was happening?
 - Did you receive results after?

- Were you reimbursed for your time and any expenses you incurred?
 - If not, why do you think you have not participated?
 - Prompt for not having heard about it, not having the time to do it, finding the description of research too complex or simplistic, or not relevant in your opinion?
- ▶ How would you like to find the answer to the question you posed at the beginning?
- ▶ Anything else you would like to share?

Feedback on the questions:

- ▶ The questions are stated in a rather broad fashion, but should be clear enough due to the skilled framing
 - Questions should be set out in such a way that they can work for countries where there is “a lot” of patient engagement in research, and where patients are almost not at all involved in research
- ▶ Main theme: what are the barriers to find answers?
 - There is a possibility that there is prejudice in the conversation, so careful framing is needed
- ▶ When we talk about research, what do we mean?
 - Do we mean clinical trials?
 - We do not want to limit the discussion necessary to this aspect only
 - We should not go into detail too much from the start, so leave room for statements, but do not continue on this
 - For most people, rare disease research is therapy focused research, but we should not forget accurate diagnostic (tools) research as well
 - Add question: What do you think that makes research useful for rare disease people?
 - We might need to define some of the aspects of research in the educational slides (diagnostics, treatment, clinical trials, etc)

→ Send in further comment on the questions

1.3 Format focus groups

The focus groups are ideally small, 8-15 people, so that there is space for an open discussion with every participant.

- ▶ Focus group will unfortunately be limited by participants that are able to travel to the patient advocacy group’s headquarters.
- ▶ 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

A couple of important points should be kept in mind before starting the focus groups

- ▶ Keep the working space as open as possible, listening to all individual members of the focus groups, try to prevent participants from pushing them for a specific answer.
 - Try to keep potential prejudice out as much as possible
 - All PACC members have a certain commitment to research, and therefore, they are likely to bring a bias, but it is important to realize that this bias is innate in all members
- ▶ Give ground rules beforehand, informing participants
 - Give everyone the possibility to talk
 - Let everyone finish their story/argument
 - Do not try to educate participants
- ▶ Ideally, we want to control a number of variables, to have the focus groups run as much as possible in the same fashion worldwide, but this is difficult

2. Next steps Activity F – Review of strawman’s position statement

This activity pertains to the issuance of a position statement of the PACC including specific recommendations on (1) model for applying Goal 2 (i.e., therapy development) internationally, and (2) model for inclusion of patients’ perspectives in therapy development.

- ▶ PACC members had previously decided to table this activity until the outcomes of Activity B are determined. However, there seems to be interest in developing a statement on the application of Goal 2, while the other IRDiRC Committees working on its implementation strategies.
 - At the last call they first reviewed a draft statement, to which all previous edits are now integrated.
- ▶ Draft statement: simplified statement without specific recommendations, updated with comments from last month’s PACC discussion
- ▶ Discussion points to consider in further revising this statement
 - Could use some further clarification
 - Put in bullet points to make the statement more readable
 - IRDiRC cannot “make sure”, so suggest to change to “promote”
 - Statement should be a balance between ambition and realistic expectations

→ Review and comment on the revised statement

Next steps and actions

- ▶ Send in further comment on the questions
- ▶ Review and comment on the revised statement for Activity F