IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

Meeting report series

Report of the 8th Patient Advocacy Constituent Committee Meeting

Teleconference
September 17, 2018

Participants

Sharon Terry, Genetic Alliance, USA – Chair
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Marlène Jagut, Scientific Secretariat, France
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Apologies

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Agenda

1. Information regarding upcoming PACC meetings
2. Activity B: Overarching next steps
3. Activity F: Preliminary discussion

REPORT

1. Information regarding upcoming PACC meetings

The Patient Advocacy Constituent Committee (PACC) will meet every month and calendar invites have been sent to all members. The next meetings will take place on:

- **Wednesday, October 10, 2018** at 8 AM EDT (Washington DC) / 9 PM JST (Tokyo) / 2 PM CEST (Paris)
- **Monday, November 5 2018** at 8 AM ET (Washington DC) / 10 PM JST (Tokyo) / 2 PM CET (Paris)
- **Friday, November 30, 2018** at 8 AM ET (Washington DC) / 10 PM JST (Tokyo) / 2 PM CET (Paris)
- **December 6 or 7, 2018** in Brussels as part of IRDiRC Consortium Assembly meeting

2. Activity B: Overarching next steps

Activity B: Identify barriers to patient participation in rare disease R&D and recommendations to remove them

Objective of the activity: Leverage IRDiRC's stakeholder and geographic representation to conduct a complementary scan of barriers to and recommendations for patient participation in rare diseases research. To accomplish this, recommendations will be developed to (1) inform future IRDiRC activities, (2) facilitate better patient engagement across geographic areas with shared resources, (3) determine strategic areas for new funding initiatives, and (4) determine alignment of current efforts with needs.

Prior discussions:

- The PACC decided to take advantage of the players within IRDiRC who represent a broad swath of stakeholders within rare diseases research already committed to IRDiRC's mission and efforts
  - Focus groups were determined to likely be the best way to receive broad geographic representation of issues. Standardization will be important across focus groups.
- Prior discussions noted: timeline is short but manageable; focus groups should be done F2F whenever possible to facilitate information exchange; multidisciplinary Task Force (TF) will be responsible for determining and defining the explicit implementation details (e.g., protocols, procedures, questions, budget); there are other existing efforts in this space so best to focus this collaborative endeavor on specific areas of need.
- Need to identify external experts in areas of need and IRDiRC members willing to participate in this activity's TF; see Section 3.

Timeline:

- A timeline was included in the proposal sent to / approved by the Consortium Assembly
  - Need to update and refine the timeline, especially in the context of approaching experts who will need to plan their work for coming months

→ Refine and detail Activity B timeline

3. Activity F: Preliminary discussion

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. Currently in the process of developing a strawman statement.
- Draft statement by the PACC Chair: simplified statement without specific recommendations
- PACC members agreed on the importance of such a statement
    - It will illustrate the commitment of the PACC.
    - It delineates the added value of patient engagement in therapy development.
- Discussion points to consider in drafting a revised statement
    - "Access worldwide" could be amended to emphasize access in developing nations.
    - In terms of protocol and structure of clinical trials, include the word "design".
        - It is a step where patients' perspective is important, particularly on the feasibility of a trial.
    - In terms of lay communication of the value of the trial, add the notion of "patients benefit".
        - Value can have different meaning depending on stakeholders.

→ Create a revised statement for Activity F
→ Review the revised statement

Next steps and actions

- Refine and detail Activity B timeline
- Solicit for interested members from the Chair and Vice Chair of IRDiRC Committees at the next OpComm
- Identify other appropriate IRDiRC Committee members to directly approach and invite to the Activity B TF for cross-Committee representation
- Create a revised statement for Activity F
- Review the revised statement