

Meeting report series

Report of the 2nd Patient Advocates Constituent Committee Meeting

Teleconference
October 12, 2017

Attending

Sharon Terry, Genetic Alliance, USA (Chair)

Nicole Millis, Rare Voices Australia

Ramaiah Muthyala, Indian Organization for Rare Diseases (I-ORD)

Yukiko Nishimura, Advocacy Service for Rare and Intractable Diseases' multi-stakeholders in Japan (ASrid)

Durhane Wong-Rieger, Canadian Organization for Rare Disorders (CORD)

Christine Cutillo, National Center for Advancing Translational Sciences, NCATS/NIH, USA

Marlène Jagut, Paris, France – Scientific Secretariat (SciSec), France

Anneliëne Jonker, Paris, France – Scientific Secretariat, France

Lilian Lau, Paris, France – Scientific Secretariat, France

Absent

Béatrice de Montleau, EURORDIS, Europe

Kelly du Plessis, Rare Diseases South Africa

Kevin Huang, Chinese Organization for Rare Disorders (CORD)

Peter Saltonstall, National Organization for Rare Disorders (NORD)

Agenda

1. Background on IRDiRC and this committee
2. Introductions
3. Action charge template for Patient Advocacy Constituent Committee roadmap
4. Preparation for the Tokyo meeting

REPORT

The Chair of the Patient Advocates Constituent Committee (PACC) thanked and welcomed participating members to the teleconference, aimed at introducing all members, setting up the charge template for the PACC roadmap, and preparing for the Tokyo meeting.

1. Background on IRDiRC and this committee

- ▶ Almost everyone is already involved with, or has been in contact with, IRDiRC
- ▶ Originally set up to help accelerate rare diseases research, with goals on diagnostics and therapies
- ▶ With the achievement of these goals, IRDiRC has set out to continue on a new set of goals, which were released recently
- ▶ With the growth of IRDiRC, patient involvement has become even more crucial, hence the set-up of the PACC, in parallel to the Funders Constituent Committee (FCC), and the Companies Constituent Committee (CCC)

2. Introductions

2.1 PACC member organisations

- ▶ PACC currently consists of 9 member organisations
 - Advocacy Service for Rare and Intractable Diseases' multi-stakeholders in Japan (ASrid)
 - Canadian Organization for Rare Disorders (CORD)
 - Chinese Organization for Rare Disorders (CORD)
 - Rare Diseases Europe-EURORDIS
 - Genetic Alliance
 - Indian Organization for Rare Diseases (I-ORD)
 - National Organization for Rare Disorders (NORD)
 - Rare Diseases South Africa
 - Rare Voices Australia
- ▶ Other umbrella organisations are expected to join shortly

2.2 Representatives present on the call

Durhane Wong-Rieger

- ▶ President and CEO of the Canadian Organization for Rare Disorders
- ▶ CORD is a national umbrella organization involved in national policy development for rare diseases and policies in a regulatory setting

- ▶ Partners with CIHR, and in that capacity also a small funder themselves
- ▶ Conference host and involved in patient training
- ▶ Durhane is also Chair of Rare Diseases International (RDI), and in this setting works on global policy development for rare diseases

Nicole Millis

- ▶ Executive Officer for Rare Voices Australia
- ▶ Key mission and activities:
 - Advocate for a national rare diseases framework
 - Advocate for rare diseases policy awareness
 - Focus on: diagnosis, access to treatment, coordinated care, access to services, research, data collection
 - Facilitate a national alliance of rare disease registries

Ramaiah Muthyala

- ▶ Founder of I-ORD
- ▶ I-ORD is almost 15 years old, representing Indian rare diseases patients across the world
 - Member of several organizations, such as RDI
- ▶ Key mission and activities:
 - Raise awareness and influence national rare disease policy
 - Involve patients in research every way they can
 - Organise conferences
 - Work on access to drugs and access to diagnostic care at a more reasonable price

Yukiko Nishimura

- ▶ President of NPO ASrid, representing rare and intractable diseases
 - Intermediate organisation, working with various stakeholders
- ▶ Core members of ASrid are from a scientific background
 - Members involved in scientific platforms
 - Members involved in ethical committees

Sharon Terry

- ▶ President of Genetic Alliance
- ▶ Chair of PACC
- ▶ Member of IRDiRC's Operating Committee

Scientific Secretariat

- ▶ Anneliene Jonker: Project Manager at IRDiRC Scientific Secretariat
- ▶ Christine Cutillo: Special Assistant to Dr Christopher Austin, Chair of Consortium Assembly and Director of NCATS, involved in strategic development initiatives within IRDiRC
- ▶ Lilian Lau: former Project Manager, now works in tandem with Christine and for Dr Austin, involved in strategic development initiatives within IRDiRC
- ▶ Marlene Jagut: Communication Manager at IRDiRC Scientific Secretariat

3. Action charge template for Patient Advocates Constituent Committee roadmap

3.1 Action charge template

- ▶ As new goals have been defined, it is time to set up a roadmap on how to achieve these goals
 - All goals are research-oriented, aligned with IRDiRC as research organization
- ▶ Patient involvement is increasingly important for research
 - Important for each member to contribute to IRDiRC's new roadmap
- ▶ Action template has been sent to every member, together with instructions
 - Members are asked to fill out these templates, and send back to Sci Sec
 - Sci Sec and PACC Chair will compile and summarize
- ▶ Exchanges primarily via email, and also discussion at Tokyo meeting
- ▶ Proposed actions are not set in stone, and can be altered, added or deleted
- ▶ Actions should be SMART: specific, measurable, achievable, result-focused and time-bound

3.2 Overarching points of the charge template

- ▶ What do the goals mean globally?
 - Drugs are not accessible in every region nor every healthcare system
 - How do we assess the impact of rare diseases therapeutics and access issues globally?
 - Diversity worldwide should be considered
 - This perhaps is best addressed in goal 3

3.3 Specific points on goal 1

- ▶ How do we create a feedback process in the health care system, so that there is a low tolerance for delayed diagnosis?
 - It is not enough to just educate the medical community
 - Important to strengthen the health care system
- ▶ Work with algorithms for awareness to identify possible rare diseases

3.4 Specific point on goal 2

- ▶ Identification of potential drugs
 - To think about how the PACC can assist in this process

Action items and deliverables by October 23

- ▶ Send comments on any of the goals to the email group
- ▶ Review the suggested actions and fill in any of the cells in the template
- ▶ Send suggested actions