

Meeting report series

Report of the 3rd Funders Constituent Committee Meeting

Teleconference

September 28, 2017

Participants

Dr Daria Julkowska, E-RARE-3 Consortium, France (Chair)
Prof Hugh Dawkins, Western Australian Department of Health, Australia (Vice Chair)
Dr Pedro Cortegoso Fernández, Carlos III Health Institute, Spain
Ms Christine Cutillo, National Center for Advancing Translational Sciences, NCATS/NIH, USA
Dr Iiro Eerola, European Commission, DG Research and Innovation, Belgium
Dr Adam Hartman, National Institute of Neurological Disorders and Stroke, NINDS/NIH, USA
Dr Daniel Lavery, Loulou Fondation, USA
Dr Teri Manolio, National Human Genome Research Institute, NHGRI/NIH, USA
Dr Alexandre Mejat, AFM Telethon, France
Dr Lucia Monaco, Telethon Foundation, Italy
Dr Irene Norstedt, European Commission, DG Research and Innovation, Belgium
Dr Melissa Parisi, National Institute of Child Health and Human Development, NICHD/NIH, USA
Dr David Pearce, Sanford Research, USA
Dr Senkei Umehara, Japan Agency for Medical Research and Development (AMED), Japan
Dr Sonja van Weely, The Netherlands Organisation for Health Research and Development (ZonMW), the Netherlands
Dr Lu Wang, National Human Genome Research Institute, NHGRI/NIH, USA

Dr Marlène Jagut, Paris, France – Scientific Secretariat (SciSec), France
Dr Anneliene Jonker, Paris, France – Scientific Secretariat, France
Dr Lilian Lau, Paris, France – Scientific Secretariat, France

Apologies

Dr Christopher Austin, National Center for Advancing Translational Sciences, NCATS/NIH, USA
Dr Cindy Bell, Genome Canada, Canada
Dr Oleg Kvlividze, Children’s New Hospitals Management Group, Georgia
Dr Paul Lasko, Canadian Institutes of Health Research, Canada
Dr Jean-Louis Mandel, French Foundation for Rare Diseases, France
Dr Katherine Needleman, Food and Drug Administration, USA
Prof Willem Ouwehand, National Institute for Health Research, UK

Dr Hyun-Young Park, Korea National Institute of Health, South Korea

Dr Gualtiero Ricciardi, Istituto Superiore de Sanità, Italy

Dr Ralph Schuster, Federal Ministry of Education and Research, Germany

Dr Sultan Turki AlSedairy, Saudi Human Genome Project, Kingdom of Saudi Arabia

Dr Edward Trimble, National Cancer Institute, NCI/NIH, USA

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Agenda

1. Pilot of iSearch or Uber Research
2. Suggestions from FCC members on which topics members would like to discuss in Tokyo.
3. Research organisations in developing countries.
4. Any other business

REPORT

The Chair of the Funders Constituent Committee (FCC) thanked and welcomed participating members to the teleconference, aimed to discuss the pilot for iSearch, the FCC agenda for the Tokyo meeting, and connections to research organizations in developing countries.

1. Pilot of iSearch

1.1 Test access of iSearch

- ▶ Chair of FCC and Director of Orphanet, have requested access to test the use of iSearch
- ▶ This pilot will allow the FCC to determine if iSearch is useful for its purpose
 - Ask a number of questions and do a limited number of analyses of the funding landscape
 - Piloting working group to come up with first questions and identify how to exploit tools

1.2 Piloting iSearch

- ▶ Working group to generate questions of interest for the pilot, that is time-limited
 - Refine keywords so that searches can be as specific as possible
 - Another potential question: who is currently running programs with developing countries or indigenous populations?
- ▶ iSearch uses publicly available data as well as NIH data with specific elements
 - iSearch shows not only projects funded, but also allows users to look at projects that did not make the funding cut. This data cannot be accessed outside the NIH.
- ▶ Clarify that the data can be accessible afterwards
 - Currently available as NIH-controlled access behind its firewall
- ▶ Pilot to be run before Tokyo, so first results can be shared at the meeting

→ Action: Piloting members to send a list of questions by October 10 to Daria and the Sci Sec

→ Action: Piloting members to send their availability for a 1h TC in the week of 16-20 October

2. Tokyo Agenda

The CA meeting in Tokyo will have a half-day breakout session dedicated to the FCC. Another half-day session is dedicated to strengths and challenges in rare diseases research in Japan. Three topics have been identified for discussion at the FCC breakout. Each topic will be discussed for one hour approximately. Each of the themes will be prepared in advance by a working group that will propose the details for presentations and discussion.

- ▶ How each of FCC members tackles the new goals:
 - Which are the new programs/ initiatives ongoing?
 - Occasion to present the EJP on Rare Diseases.
 - Occasion to present the new Work Programme 2018 -2020 of the EC
 - Discuss what are current funding opportunities, topic prioritization, and possibilities to build partnerships for future funding calls
 - *[Post –meeting note: Discuss opportunities vis-à-vis the goals and priorities previously determined in planning/roadmap exercise*
 - *How is each member and the FCC, as whole, going to contribute to the proposed goals and actions of the FCC?]*
- ▶ International collaboration on RD Clinical Research Networks
 - Presentation of plans for RDCRNs in the US, ERNs in Europe, other equivalents in different parts of the world
 - How to establish connections and bring international partners in
 - How to establish durable international collaborations, which are the obstacles now,
 - How to exploit the potential of less developed countries to join them into the networks from the start?
 - Volunteers needed to set up discussion framework of this part of the agenda
 - *[Post-meeting note: How does FCC interface in this regard with the new TF?]*
- ▶ Ethical, legal and social implications (ELSI) of RD research funding, RD healthcare and therapies
 - A number of countries have funding calls on ELSI aspects (For example: <https://grants.nih.gov/grants/guide/pa-files/PA-17-444.html>)
 - Discuss ways to reference ELSI in RD research
 - Study use cases
 - Opportunity to learn and see how this can be projected to other counties
 - Looking at public health impact
- ▶ FCC Chair, together with SciSec, will write a short note to all groups and agree with them on the preparation of the Tokyo meet

→ Action: set up call re activities toward IRDiRC goals

→ Action: set up call re RDCRNs

→ Action: set up call re ELSI considerations

3. Research organisations in developing countries.

- ▶ Collaboration with developing countries is a transversal issue
- ▶ The ECRD 2018 Congress includes a session on international collaborations
 - Gather actors from developing countries and identify their needs
 - Take advantage of this session and discuss with invited speakers on how do they see the support and collaboration with IRDiRC (what would be the most pertinent for them)

- Produce a short report that will serve as initial recommendations to bring back to the FCC for discussion
 - ▶ FCC to think of how to create a socket/portal/platform for organisations in developing countries and how they can participate according to their possibilities
 - How can developing countries benefit from the knowledge that is already available?
 - For example, NCATS recently released the Toolkit for Patient-Focused Therapy Development Project (<https://rarediseases.info.nih.gov/toolkit/>). While this is patient-focused, not research focused, it can be used as a starting point
- Action: distribute initial recommendations from ECRD session to FCC for discussion + make it transversal as part of any discussion/topic of the FCC

4. Any other business

- ▶ The European Commission currently has a public consultation on a communication, regarding the “Transformation of Health and Care in the Digital Single Market.”
 - Specific mention of rare diseases in this communication
 - FCC members asked to review and submit comments
 - Consultation is open till October 12, 2017
 - https://ec.europa.eu/info/consultations/public-consultation-transformation-health-and-care-digital-single-market_en
- Action: Interested FCC members to send in comments to the EC on this communication

Action Items and deliverables

- ▶ Check with iSearch on access rights in order to start pilot
- ▶ Send questions for iSearch by **October 10**
- ▶ Send availability for teleconference
- ▶ Prepare framework for FCC discussions in Tokyo
- ▶ Volunteer to prepare discussion framework on RDCRN
- ▶ After ECRD, distribute initial recommendations from session to FCC for discussion
- ▶ Send in comments on EC communication