

Meeting report series

Report of the 7th Funders Constituent Committee Meeting

Teleconference

October 29, 2018

Participants

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Dr En Kimura, Japan Agency of Medical Research and Development, AMED, Japan
Dr Daniel Lavery, Loulou Fondation, USA
Dr Christopher McMaster, Canadian Institutes of Health Research, Canada
Dr Lucia Monaco, Telethon Foundation, Italy
Dr Katherine Needleman, Food and Drug Administration, USA
Dr Melissa Parisi, National Institute of Child Health and Human Development, NICHD/NIH, USA
Dr David Pearce, Sanford Research, USA
Dr Manuel Posado, Carlos III Health Institute, Spain
Dr Daniel Scherman, French Foundation for Rare Diseases, France
Dr Ralph Schuster, Federal Ministry of Education and Research, Germany
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Apologies

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Dr Oleg Kvlividze, Children’s New Hospitals Management Group, Georgia
Dr Alexandre Mejat, AFM Telethon, France

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Agenda

1. Outcome call for tenders
2. Outcome and analysis funding collaboration survey
3. Plan of report on ELSI funding issues
4. Agenda of Brussels meeting

REPORT

The Chair of the Funders Constituent Committee (FCC) thanked and welcomed participating members to the teleconference, aimed to discuss ongoing matters; the results of the call for tenders, the results of the funding collaboration survey, the next steps on ELSI funding and the agenda for the Brussels meeting.

1. Outcome call for tenders

- ▶ A call for tenders was launched in July to initiate the primary objectives of Activity A -- *Global database and platform for RD funding analysis and collaboration* – through the creation of a comprehensive rare disease database and platform.
 - Aim is to systematically track and analyze global rare disease funding landscape, and coordinate that of all IRDiRC members.
 - Expectation is that this effort will provide a tool that allows for in-depth analysis of funded projects and the rare diseases research funding landscape at an international level – to enable better understanding, address the gaps in research, and provide a basis for further funding coordination.
- ▶ Two applications received
 - Proposals evaluated by CA Chair, FCC Chair, and current Sci Sec Coordinator
 - Had individual calls with both applicants, during which they presented their proposal and examples of previous work.
 - Applicant 1 proposes a database (built off extensive, prior work), with limited possibilities for analysis.
 - Applicant 2 does not have an existing database, but proposes a technical solution to set up the database and provide analysis.
 - Decision has been made for Applicant 2, due to the fact that they were more open for the specific needs of IRDiRC.

The contract is currently getting started. By the end of the year, the proof concept should be delivered.

- ▶ Members asked to provide a short company profile by email on the selected company, explaining how they are going to work.
- ▶ Ideally, in the Brussels meeting, more information will be provided on how the company will work, and if available, the first proof of concept will be presented.

→ Investigate if is possible to share the first proof of concept with all FCC members at the time of the meeting

2. Outcome and analysis funding collaboration survey

In parallel to the data collection and the funding database, FCC has started to investigate a first step towards stronger funding collaboration. In order to do so, all FCC members were asked to fill out a

funding collaboration questionnaire. This questionnaire mostly investigated procedures, different functioning modes and possibilities and constraints for future collaboration. Most FCC members filled out the questionnaire; the full conclusions will be discussed in the Brussels meeting, but the main conclusions are:

- ▶ The large majority expressed the interest to work together and expressed the interest in doing so
 - In addition, all FCC members have the possibility to establish
- ▶ There is an absolute need to plan joint calls in advance
 - Calls should be planned at least 18-24 months in advance.
 - If we want to discuss a joint call in Brussels, the call will take place in 2020/2021.
- ▶ There are different types of funding collaboration possible
 - Joint funding calls are possible for some members; others have the possibility to set up parallel or complementary initiatives.
 - The kind of collaboration possible is something that should be explored further in the Brussels meeting.

In order to prepare for the Brussels meeting, the full results/ initial analyses will be shared with all members in advance of the meeting

→ Send out full results/ initial analyses with all members prior to the Brussels CA meeting

3. Plan of report on ELSI funding issues

A working group of the FCC started to set out to investigate current ELSI funding opportunities and gaps, by means of a questionnaire. The goal of this survey was thereby gain an overview of the current ELSI landscape in the world of rare diseases. All FCC members were asked to fill out the questionnaire, and the results were discussed in the Vienna session.

Updates since the Vienna breakout:

- ▶ The working group has been joined by a professor in ELSI at the University of Miami.
- ▶ The working group has written an outline, to be used for a workshop/ report/ paper.
- ▶ One or more calls are needed with the working group, prior to the start of writing, in order to have a better focus of the potential paper.

Simultaneously, E-Rare is planning a workshop dedicated to rare diseases and social sciences and humanities, in order to eventually set up a call dedicated to the topic, that will be integrated in the European Joint Programme on Rare Diseases.

- ▶ Mid-September, the set-up of this workshop was discussed.
- ▶ A collaboration is proposed between a number of E-Rare funders and the IRDiRC ELSI WG
 - Plan to have a joint teleconference in mid-end November.
 - Think further on when the workshop will be, and who should be invited to the workshop, and to discuss on how the call is envisaged (Workshop is planned in summer 2019, in order to prepare for the call in 2021).
 - Should be added to the Brussels agenda for further discussion.

- Set up a new call with the ELSI WG to discuss the outline and paper
- Set up a call with the ELSI WG and the E-Rare funders, to discuss the E-Rare workshop

4. Agenda of Brussels meeting

The CA meeting in Brussels will have a half-day breakout session dedicated to the FCC, that will take place on the first day of the meeting, December 6, in the afternoon. Several topics are suggested for the agenda for this face-to-face meeting of the FCC, which is ideally a mix between already launched initiatives and activities that have yet to be started.

- ▶ Future collaboration for funding calls
 - Should be the core of the Brussels discussion.
 - Will be an occasion to discuss the full results and next steps of the funding collaboration survey
 - Will allow the FCC to determine next steps for funding cooperation and collaboration
- ▶ Ethical/Legal (ELSI) aspect of RD research funding, RD healthcare and therapies
 - FCC members filled out a survey, results compiled by the Sci Sec
 - Follow up with next steps
- ▶ Call for tenders for funding project database
 - Presentation by successful Applicant 2
 - Details about the tool that is going to be put in place
- ▶ International collaboration with developing countries
 - Research funding with and in developing countries
 - Identify those that could have a concrete role in this activity
- ▶ Possible activities for IRDiRC Roadmap 2019
 - Agenda on how to pursue the different actions

This agenda will be sent out along with the report of the call, and all members are asked to reflect on this, and contribute potential additional topics for the agenda.

- Send in topic suggestions for the Brussels meeting

Action Items and deliverables

- ▶ Investigate if is possible to share the first proof of concept with all FCC members at the time of the meeting
- ▶ Send out full results/ initial analyses with all members prior to the Brussels CA meeting
- ▶ Set up a new call with the ELSI WG to discuss the outline and paper
- ▶ Set up a call with the ELSI WG and the E-Rare funders, to discuss the E-Rare workshop
- ▶ Send in topic suggestions for Brussels meeting