Report of the 6th Funders Constituent Committee Meeting

Vienna, Austria
May 16, 2018

Participants

In person

Dr Daria Julkowska, Paris, France (Chair Funders
Constituent Committee (FCC))
Dr Takeya Adachi, Tokyo, Japan
Dr Christopher Austin, Bethesda, USA
Dr Gareth Baynam, Perth, Australia
Dr Cindy Bell, Ottawa, Canada
Dr Iiro Eerola, Brussels, Belgium
Ms Yuko Endo, Tokyo, Japan
Dr Aikichi Iwamoto, Tokyo, Japan
Dr En Kimura, Tokyo, Japan
Dr Denis Lacombe, Brussels, Belgium
Prof Paul Lasko, Montreal, Canada

Dr Teri Manolio, Bethesda, USA
Dr Irene Norstedt, Brussels, Belgium
Dr Marie-Christine Ouillade, Paris, France
Dr Manuel Posada, Madrid, Spain
Dr Lucia Monaco, Milan, Italy
Dr Ralph Schuster, Bonn, Germany
Dr Stefan Schreck, Brussels, Belgium
Dr Domenica Taruscio, Rome, Italy
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By phone

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Agenda

1. Welcome of participants Funders Constituent Committee
2. ELSI research: Results questionnaire and next steps
3. Call for tenders: deliverables database Activity A
4. Future collaboration for funding calls: results questionnaire and next steps
5. International collaboration with developing countries: summary session ECRD

EXECUTIVE SUMMARY

▶ Ethical, legal and social implications (ELSI) are aspects of rare diseases research that has recently received increased attention. The Funders Constituent Committee (FCC) discussed the outcome of a short survey on funding opportunities for ELSI research, and potential gaps to overcome in the future.

▶ The Coordinator of the Scientific Secretariat (Sci Sec) presented the call for tenders for a system that allows to systematically track rare disease funding worldwide. Deliverables and steps in the project where presented, in order to allow all members of the FCC to provide their input.

▶ The FCC discussed the preliminary results of the questionnaire for funding collaboration. While not all FCC members were able to fill out the survey prior the meeting, it did show that most collaborative activities are possible for the majority of funders, especially using well-established schemes. In order to set up collaborative calls, topics need to be defined well in advance, and the review process needs to be well-defined prior to the launch of the call.

REPORT

1. Welcome of Participants Funders Constituent Committee

The Chair of the Funders Constituent Committee (FCC) thanked and welcomed participating members to the face-to-face FCC meeting. Main agenda items included: Ethical, Legal and Social Implications (ELSI) of Rare Diseases, and the result of the questionnaire to investigate current ELSI funding opportunities and gaps, the call for tenders, discussing the deliverables for the funding database, the first results of the questionnaire on funding call collaboration, and an initial discussion on international collaboration with developing countries.

2. ELSI research: Results questionnaire and next steps

2.1 ELSI Questionnaire

A working group of the FCC, started 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 this session. The main themes of this questionnaire were:
- ▶ What are current ELSI funding opportunities?
- ▶ Which topics should be covered by ELSI funding calls?
- ▶ What are common ELSI-related issues and questions?
- ▶ What are potential opportunities for international collaboration?

2.2 Results ELSI Questionnaire

Very few FCC members had ELSI funding calls specific to rare diseases, most of which has ELSI components as part of a general call. There are a number of calls where there is an ELSI element: either in general calls, or in several specific other dedicated calls.

In more detail, current programs with an ELSI component can be divided into four groups:
- ▶ Personalized Medicine
- ▶ Genomics and Gene Discovery
- ▶ Health
- ▶ Social Sciences and Ethics

The questionnaire went also more in detail in which topics should be covered by ELSI rare diseases funding calls, thereby addressing some of the gaps that are currently not sufficiently covered. These topics can be categorized in 5 groups, being:
- ▶ Data collection

- Data sharing
- Burden of rare disease
- Consent and patient information
- Patient engagement

The topics that FCC members think should be covered by funding might not be specific to rare diseases, as all can be related to common diseases as well, but the field of rare diseases does bring a specific emphasis to these topics. The uniqueness of rare diseases:
- Burden of rare diseases
  - Genetic
  - Economic
  - Life plan (personal, employment)
  - Family-related
- Patient organization involvement
- Emerging technology impact on rare diseases (e.g., CRISPR used to treat rare diseases before common diseases)
- These are all related to each other!

2.3 Next steps

The FCC discussed the possibilities to move forward for this topic:
- Report on extant data from survey?
  - There is currently a fair amount of interesting data, that not everyone is aware of
    - Is there a need to further expand / systematize data?
  - Format: publication, website?
- Explore better existing ELSI recommendations worldwide
  - Better integration and communication around existing recommendations and initiatives, such as BBMRI ethical recommendations, RD-Connect ELSI recommendations, IRDiRC Recognized Resources on ELSI issues
- Address the lack of expertise in reviewing ELSI grants applications
  - All agencies encounter ELSI issues in their grant applications, but some operationalize these issues better as others
    - ELSI issues should become a systematic part of grant review
    - Would guidelines for funders on ELSI issues be necessary?
  - Possible collaboration with business schools or other unexpected sources of ELSI expertise
  - Invite experts from outside the rare disease world to review specifically this part of grants
  - Organize a dedicated session at an IRDiRC Conference
  - Not yet ready to tackle a dedicated joint ELSI funding call
- Prioritize of ELSI topics
  - Discuss with other committees to better align across all IRDiRC

- ○ Organize a workshop (together with E-Rare) to discuss and address gaps
  - ▪ E-Rare plans to organize a workshop on this topic in Q1-2 2019, to bring more attention to social sciences and humanities topics, and to mature further the emphasis on these topics in funding calls
- ○ Opportunity to sharpen the focus on ELSI issues and evaluate better the focus on specific ELSI elements
- ○ Determine and address what the main ELSI issues are that prevent us from reaching the IRDiRC Goals

→Set up call with FCC WG to expand and report on ELSI funding issues and prepare a report (as outcome of the questionnaire) to be published at least on IRDiRC website (as a start)
→ Set up call with other committees to discuss on how to better align this topic across IRDiRC and produce recommendations for funders to be included in the funding opportunities
→ Continue discussions with E-Rare to shape the workshop: main idea should be "which are the ELSI issues that will prevent IRDiRC from achieving its goals"
→ Discuss at the OpComm TC the possibility of integrating an ELSI/social sciences & humanities (including economic aspects) dedicated session in the next IRDiRC conference

3. Call for tenders: deliverables database Activity A

In order to set up a system that allows to systematically track rare disease funding worldwide, and go one step further than currently possible, the CA discussed and approved the possibility in January 2018, to ask for an amendment of the current Scientific Secretariat (Sci Sec) contract, in order to allow subcontracting this part, in order to create this dedicated rare disease database. This would thereby help funders fulfill the original idea behind IRDiRC, being able to track, understand and coordinate rare disease funding. At present, there are several general funding databases, and some databases specific to rare diseases funding (such as Orphanet), and this database would combine the completeness of a general database, and the knowledge of a specific database. The Coordinator of the Sci Sec presented the call for tenders that is currently in preparation.

In summary, this database should allow to:
- ▶ Inform all IRDiRC Funders to find out what the other funders are funding, thereby providing a full overview of the rare disease funding landscape including RD research trends or identification of key centers/institutions/countries leading specific topics
- ▶ All IRDiRC funders to connect directly to the database (with an IRDiRC dedicated password), to ask funding related questions
- ▶ Replace the yearly call for data from funders. Funders will be send yearly an overview of their rare disease funding, for verification
- ▶ Have a certain degree of flexibility, to adapt to the needs of funders throughout the next few years

The FCC suggested that the mechanisms to recover the data from new or existing funders should be put in place by the subcontractor. It is important as the majority of FCC members cannot deliver for example

an excel file with all funded projects. However, all funders can indicate the link to where the data is available on line.

4. Future collaboration for funding calls: results questionnaire and next steps

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. Approximately half of the FCC members filled out the questionnaire; the members of the FCC therefore discussed the preliminarily results of the questionnaire, that FCC members provided on a confidential basis. Questions discussed the following elements:

- ▶ Funding instruments
- ▶ Research funding
- ▶ Funding cooperation
- ▶ Funding process
- ▶ Ways towards collaborative opportunities

Next steps

- ▶ To complete a better understanding of the funding collaboration landscape
- ▶ Mutually inform ourselves better of the upcoming funding
- ▶ Exchange with IRDiRC PACC – first step can be taken in the CA wrap-up session
- ▶ Better understand evaluation process and process of review
- ▶ All FCC members to understand how they can inform their decision makers, and working on establishing relationship

→ All FCC members that have not yet done so to fill out the survey in order to understand collaborative funding opportunities
→ Set up a dedicated NIH meeting to discuss alignment and procedures of different NIH members in the context of FCC planned actions
→ Dedicate time in all FCC calls to mutually inform ourselves better of the upcoming funding topics
→ Set up call with IRDiRC PACC Chair/ Vice Chair to exchange ideas on setting up calls with patient organizations
→ Better understand the evaluation process and process of review
→ All FCC to establish contact with their decision makers, in order to establish a working relationship for a joint call

5. International collaboration with developing countries: summary session ECRD

At ECRD, sessions were dedicated to international collaboration with "developing" countries. The FCC members discussed and summarized the sessions, as a way to start the discussion on international collaboration with "developing countries."

5.1 Summary

There are few collaborative projects between "developed" and "developing" countries (such as H3Africa), but they are not widespread. In order to expand on these projects, several difficulties and opportunities need to be taken into account:

▶ There is, from both sides, a desire to work together, but especially from the "developing" side, there is a clear wish that it would be bidirectional traffic, and not a way to impose experiences unilaterally

▶ While rare diseases are important, the environment in "developing" countries put, logically, more emphasis on other diseases that are far more widespread

▶ Currently not clear what the expectations are, what funding members want to do, and what is our role?

▶ IRDiRC membership has a high limit, and travel to meetings is expensive

5.2 First steps and ideas

▶ Rather than reaching out to indigenous populations in "developing" countries, as a first step, we can try to better understand indigenous populations in "developed" countries
  ○ Explore as a starting point

▶ Bringing IRDiRC to "developing" countries to attract and include policy makers, to meet them on home territory, and start the discussion on an equal level
  ○ Align with PACC, as PACC has members from Africa, Asia, and hopefully soon South America; continents were generally we have not FCC members from
  ○ Organize a dedicated meeting in a "developing country" to organize exchange, therefore lowering the participation barrier on the "developing" countries side
  ○ Start of discussion with local policy makers

Main deliverables

▶ Set up call to expand and report on ELSI funding issues and prepare a report (as outcome of the questionnaire) to be published at least on IRDiRC website

▶ Set up call with OpComm to discuss on how to better align and integrate ELSI in IRDiRC agenda and produce recommendations for funders to be included in the funding opportunities

▶ Continue discussions with E-Rare to shape the workshop: main idea should be "which are the ELSI issues that will prevent IRDiRC from achieving its goals"

- Discuss at the OpComm TC the possibility of integrating an ELSI/social sciences & humanities (including economic aspects) dedicated session in the next IRDiRC conference
- Fill out the funding collaboration survey (for members that have not done so) in order to understand collaborative funding opportunities
- Set up a dedicated NIH meeting to discuss alignment and procedures of different NIH members in the context of FCC planned actions
- Dedicate time in all FCC calls to mutually inform ourselves better of the upcoming funding topics
- Set up call with IRDiRC PACC to exchange ideas on setting up calls with patient organizations
- Better understand the evaluation process and process of review
- Establish contact with their decision makers, in order to establish a working relationship for a joint call