

Meeting report series

Report of the 8th Funders Constituent Committee Meeting

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Participants

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Agenda

1. Welcome and introduction/roundtable of participants Funders Constituent Committee
2. Activity A: Call for tenders for funding project database, current analysis and funding collaboration
 - a. Analysis of the funding landscape
 - b. Presentation MyScienceWork
 - c. Discussion on the detailed need of funders
3. Ethical/Legal (ELSI) aspect of RD research funding, RD healthcare and therapies
4. Next steps FCC: IRDiRC Roadmap 2019

REPORT

1. Welcome and roundtable of participants

The Chair of the Funders Constituent Committee (FCC) thanked and welcomed participating members to the meeting, aimed to discuss the outcomes of the call for tenders for a funding project database, that was part of Activity A and the next steps in this project, as well as the next steps of the Ethical/Legal (ELSI) working group and the agenda for 2019.

2. Activity A: Call for tenders for funding project database, current analysis and funding collaboration

2.1.1 Analysis of the RD research landscape: State of Play 2010-2018

The coordinator of the Sci Sec presented the start of the analysis of the rare diseases research landscape. The analysis is done to identify gaps and overlaps in funding, and to eventually help with identifying new opportunities. Current analysis, based on data on rare disease projects provided from all FCC funders over the period 2010-2017, covers:

- ▶ Type of funding
 - Public
 - Private
 - Kind of funding instrument (research, networking, career...)
- ▶ Data analysis
 - Per specific disease/group of diseases
 - Per country
 - Over time
 - Developing countries
- ▶ Identifying gaps & overlaps
 - Disease/group of diseases (the most studied, the never studied...)
 - Categories of projects (basic/pre-clinical/clinical... and more granular categories)
 - Phases for clinical trials

In addition, the following analyses are foreseen:

- ▶ Total number of research projects and clinical trials
- ▶ Distribution by
 - Basic research
 - Pre-clinical research
 - Clinical research

- Other studies
- ▶ Distribution by subcategories
 - Research projects
 - Clinical trials
- ▶ Distribution by medical domain
 - Research projects
 - Clinical trials
 - Drugs in development/approved
- ▶ Comparison of distribution research projects/CTs per medical domain and distribution of medical domains (Weight of research vs weight of medical domain)
- ▶ Coverage of RD
 - In total
 - By medical domain
 - By classes of prevalence
 - Distribution of research by RD
- ▶ Comparison of types/subtypes of research by medical domains
- ▶ Comparison between countries

Additional queries will be investigated once the MyScienceWork tool is in place, but could be:

- ▶ By classification node (e.g. group of diseases)?
- ▶ By disease?
 - Over time
 - By research category
 - ...

The question arose to have CPT codes or other codification or nomenclature of rare diseases, to assist in a more heterogeneous retrieval and curation.

- ▶ It would help in a more automatic way of retrieving projects related to rare diseases
- ▶ It would also assist in a more reliable curation and thus analysis of the data
- ▶ However, currently it is hard to implement this on the funders side
 - Therefore this was part of the call for tenders, to search for key words and automatically add codification/ nomenclature, and afterwards this will be manually validated.

2.2 Presentation MyScienceWork

- ▶ 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.
- ▶ MyScienceWork was selected as the tender, and has started to set up the first analysis and tools.

MyScienceWork

- ▶ Company founded in 2010, with the idea to help democratize science
 - 70 M research publications, 12 M patents
- ▶ Data-driven solution to analyze scientific content, foster innovation, and drive strategic research decisions
 - Based on natural language processing and machine learning
- ▶ Proposed solutions to the analysis and capture of data were discussed but most important is to define what questions all FCC members want to address.

Idea to set up a kind of funder identity card

- ▶ Collect typical information on each funder, for example:
 - Typology of funder: private/public
 - Type of funding provided: intra or extramural research
 - Accounting of funding: per year, per funding opportunity, etc
 - Type of access to grant results: what are the links to access? what are the obstacles?

Access to the tool

- ▶ At current stage, only IRDiRC members and Orphanet will have access to the tool

→Continue analysis of RD funding landscape

2.3 Discussion on the detailed need of funders

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. Very preliminary results were discussed at the Vienna meeting, but now that most FCC members filled out the questionnaire; the full conclusions were discussed, and the main conclusions are:

2.3.1 Funding instruments

Several questions in the survey concerned funding instruments and possibilities.

- ▶ FCC members have various funding instruments in their portfolio
 - Dedicated calls for research, support grants for the creation of research networks and dedicated international partnership grants are the most used funding instrument, but all funding instruments indicated in the survey are used by multiple funders
 - FCC members see most possibilities for funding alignment in dedicated calls for research, and dedicated international partnership grants
- ▶ The legal framework of the majority of funders allows co-funding
- ▶ Most funders can only finance applicants working in national institutions (independently of the nationality of the applicant), however some can also fund applicants working in other countries

2.3.2 Research funding

- ▶ As a whole, the FCC funders have dedicated calls on a large portfolio of topics dedicated to rare diseases, from very basic research to very clinically oriented topics.
 - The type of research that can be funded is equally varied, for example basic research, databases, clinical studies and other topics
- ▶ The eligibility criteria for grants are very dependent on the program
 - Eligibility criteria that are common for many funders are that grants are only open to researchers working in national institutions, and that the amount of money is capped
 - The types of institution that can be funded are universities, non-university research institutions, health care institutions, research centers, hospitals, and others. Only in specific programs, small and medium enterprises or pharmaceutical companies can also be funded, but these programs often ask for a certain percentage of own budget to be brought in
- ▶ Several funders have specific clauses or constraints for particular funding initiatives
 - Most often, there is a clause related to data sharing or a clause related to intellectual property included, but the specific clause was not investigated

2.3.3 Funding cooperation

- ▶ The most envisaged possibility for collaborative funding opportunities are bi- or multinational agreement and mutual recognition and therefore alignment of call agendas, which are possible for the majority of funders

2.3.4 Funding process

In order to move forward with possible funding cooperation, the FCC investigated several questions regarding the administrative process of setting up and granting a call.

- ▶ On average, most funders need new funding opportunities to be included 18-24 months in advance of opening the funding opportunity.
 - Even though FCC members do not indicate to go beyond 24 months, this is still a considerable time frame.
- ▶ Once the grant is awarded, it generally takes up to 6 months for the PI to receive the money, but small differences in grant awarding can generally be taken up by the project.
- ▶ The average duration of funding initiatives is 3 years, with most funders funding projects for 5 years' maximum.
- ▶ There are a varied number of bodies involved in the decision-making process for funding opportunities, but most evaluation processes are similar
 - Different actors, such as ministries, board of directors, coordinators, research councils, and program coordinators are involved
 - The IRDiRC FCC members are in direct contact with them and can help in establishment of collaborations
- ▶ The outcomes of a funding call are generally evaluated by a mandatory peer review process

2.3.5 Towards collaborative opportunities

The FCC also discussed what could be first steps towards collaborative funding opportunities.

- ▶ FCC members mostly saw collaborative opportunities in the existing established schemes as E-Rare program, the future EJP RD, in calls with the OOPD's Natural History Program, in calls with mutual recognition
- ▶ Two topics from the IRDiRC Roadmap exercise are indicated as potential topics for a collaborative funding call, and one that resulted from the ELSI Working Group
 - Social Sciences and Humanities – call on ELSI funding in rare diseases
 - Promote rare disease methodology development for health technology assessment and health economics research to evaluate disease burden and patient access to diagnostics and therapies
 - Topic aligned with goal 3 of the IRDiRC goals
 - Currently needs more knowledge in order to set up this first call, therefore it might first need a scientific workshop, to tackle the general issues around this topic, and then a call could be prepared
 - Data sharing – standards and use cases

2.3.6 Conclusions

There is an interest and the potential to set up a funding collaboration strategy, but it will still need some further investigation to set up a first joint call:

- ▶ The instruments to establish collaboration are bi- and multinational collaborations and mutual recognition (well established schemes like E-Rare & future EJP RD were indicated as to be exploited)
 - One question was not included, being are your current opportunities/ projects/ programs open for collaboration
- ▶ Most of the funders have similar evaluation processes (peer review) and finances research teams from their national institutions
 - The procedure to award grants needs to be established up front
- ▶ The max. time to establish common (new) funding opportunity is of 18 – 24 months and should be taken into consideration for FCC actions
 - There is a need to share confidentially topics in advance
 - There is a need to establish procedures to share confidential topics
- ▶ The max. time to grant is around 6 – 9 months (helpful in alignment of starting dates of the multinational projects)
- ▶ Most of the funding initiatives implemented at different institutions have a medium life duration → 3-5 years
- ▶ There is an interest to set up calls together with patient organizations
 - Would require guidelines on how to launch a call together
 - Might provide a possibility to reach scientific communities members that do not yet interact together
- ▶ The majority of respondents indicated interest in common activities on data sharing – standards and use cases (foster data sharing, promulgate standards, create awareness on standards and promote their implementation).
- ▶ The promotion of rare disease methodology development for health technology assessment and health economics research to evaluate burden and patient access to diagnostics and therapies was selected as most interesting topic for collaboration. Funders indicated possibility of funding of health sociology and economics → **possible action to implement?**

2.3.7 Next steps

- ▶ Set up a funders identity card, as also discussed in topic 2.2
- ▶ Investigate further data sharing clauses implemented by IRDiRC funders
 - What type of clause funders put for a data sharing? – Is it about an obligation of consent?
 - About obligation to store data on publicly available platform?
- ▶ Investigate the opportunities for sharing
 - Share confidential information prior to calls but also share information on sequential opportunities.
 - Might be possible for a small funder to have a small call earlier, which in turn prepares for another initiative.

→ Set up a questionnaire for the IRDiRC Funder identity card

3. Ethical/Legal (ELSI) aspect of RD research funding, RD healthcare and therapies

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 first discussed in the Vienna session.

Updates since the Vienna breakout:

- ▶ The working group has written an outline, to be used for a workshop/ report/ paper.
- ▶ Simultaneously, E-Rare is planning a workshop dedicated to rare diseases and social sciences and humanities

The next steps in this Working Group are:

- ▶ Set up a few calls to further discuss and precise the outline of the paper
- ▶ Better integrate IRDiRC Goal 3 into the paper
- ▶ Query for interested members to join the Working Group

Meanwhile, E-Rare has set up a working group to set up a strategic workshop, in preparation for a funding call a year later:

- ▶ Workshop will be around July 2019, but still needs further discussion
- ▶ A pre-workshop paper will be very useful, so there is hope that the paper that the IRDiRC ELSI WG is working on can be used in preparation

→Draft first version ELSI paper

4. Next steps FCC: IRDiRC Roadmap 2019

The FCC is currently working on different topics:

- ▶ Activity A: Global Database and Platform for RD funding Analysis and Collaborations
 - Analysis of the funding landscape
 - Via tender MyScienceWork
 - Funding collaboration and alignment
 - First steps are made via the survey on funding collaboration
- ▶ Activity D: Facilitating the Conduct of Natural History Studies Related to Rare Diseases
 - Joint Activity of ISC, FCC, TSC, CCC, PACC
- ▶ Activity G: Clinical Research Networks
 - Joint Activity of ISC, FCC and TSC
- ▶ ELSI Working Group
 - Activity within FCC with some external experts

The FCC discussed if additional topics should be added:

- ▶ Two additional items came up:

- On June 17, 2019, the HIRO meeting will take place at the NIH
 - Meeting will discuss the data sharing for conditional funding
 - FCC members will provide feedback on the subject by the end of Q1 2019
- There is a lot of interest for a collaborative project with the FCC and CCC
 - Possibility for a multidisciplinary Working Group?

Action Items and deliverables

- ▶ Continue analysis of RD landscape
- ▶ Set up a funder identity card questionnaire
- ▶ Draft ELSI paper