

## Meeting report series

# Report of the 23rd Interdisciplinary Scientific Committee Meeting

Teleconference

July 13, 2018

## Participants

Dr Petra Kaufmann, AveXis, USA – Chair

Dr Domenica Taruscio, ISS, Italy – Vice Chair

Dr Stephen Groft, NCATS, USA

Prof Matthew Might, Hugh Kaul Precision Medicine Institute, USA

Mr David van Enckevort, University of Groningen, The Netherlands

Dr Marlène Jagut, Scientific Secretariat, France

Dr Lilian Lau, Special assistant to IRDiRC CA Chair, France

Dr Anne Laure Pham Hung d'Alexandry d'Orengiani, Scientific Secretariat, France

## Apologies

Dr Dixie Baker, Martin, Blanck and Associates, USA

Dr Angel Carracedo, University of Santiago de Compostela, Spain

Ms Gema Chicano Saura, University of Murcia, Spain

Dr Melissa Haendel, OHSU, USA

Prof Ken Ishii, AMED, Japan

Dr Edmund Jessop, NHS England, UK

Prof Hanns Lochmüller, Faculty of Medicine of Freiburg, Germany

Dr Elizabeth McNeil, Bluebird Bio, USA

## Agenda

1. Debrief of the Vienna ISC meeting
2. Update on Activity G - Clinical Research Networks in Rare Diseases
3. Update on IRDiRC-GA4GH Model Consent Clauses for Rare Diseases Task Force
4. Development of proposal for Activity D - Natural history and registry (NH/R) platform for use in real world evidence (RWE) data collection
  - a. Leadership of ISC for this activity
5. Election of the ISC Chair and/or Vice Chair
6. Any other business

## REPORT

### 1. Debrief of the Vienna ISC meeting

A report reflecting the discussions of the ISC at the face-to-face meeting in Vienna was sent to all members

- ▶ Encouraged to take note of discussion points, in particular for upcoming activities
- ▶ Minor correction and feedback to be sent in by the ISC Vice Chair
  - Error on the name of the EUROCAT (instead of EuRRECa) project
  - Feedback related to Privacy-Preserving Record Linkage (PPRL)

→ ISC members to review the report and provide feedback

### 2. Update on Activity G - Clinical Research Networks in Rare Diseases

Activity G aims to survey the ecosystem of national/supranational clinical research networks and develop policy and funding recommendations based on the experience of these networks in order to reinforce rare diseases research.

- ▶ Activity G proposal was presented in Vienna but was not approved by the Consortium Assembly
  - Need to clarify and define the objectives and aims for the activity
  - Need to expand background to include all existing initiatives
- ▶ The proposal was edited and updated based on feedbacks received
  - Re-presented to the CA on July 9, 2018
  - Well-received during the teleconference, with additional comments
    - Task Force should not work on regulatory aspects which is out of IRDiRC's scope
    - Task Force should provide guidance to support the cooperation between different clinical research networks but NOT to harmonize standards
  - Electronic vote for approval by the CA currently underway
    - Result expected by July 22, 2018
  - If approved, background research and preparatory activity can begin during the summer
- ▶ Important to include innovative tools in this Task Force
  - Increased use of digital/medical technologies
  - Ways to have ambulatory assessment given geographically dispersed patient populations

### 3. Update on IRDiRC-GA4GH Model Consent Clauses for Rare Diseases Task Force

Model Consent Clauses (MCC) Task Force is a collaborative effort between IRDiRC and GA4GH, led by Petra Kaufmann, Bartha Knoppers and Jack Goldblatt.

- ▶ Activity updates:
  - Group has already assembled language of various consent forms
  - Face-to-face meeting on September 6-7, 2018

- Will discuss and identify common elements critical to rare diseases when designing consent forms
  - Goal is to maximize patients' participation in research studies in term of use of data, collaboration
- ▶ Later in the year, the Task Force will seek for feedback and input on their work from the ISC
- ▶ Expected outputs:
  - A white paper on model consent clauses for rare diseases research
  - An application for the guidelines to be an IRDiRC Recognized Resources
  - Publication of recommendations on the IRDiRC website

#### **4. Development of proposal for Activity D - Natural history and registry (NH/R) platform for use in real world evidence (RWE) data collection**

The basic premise of this activity relates to natural history and real-world data collection to accelerate rare disease therapy research and development.

- ▶ This activity has attracted interest from several IRDiRC Constituent and Scientific Committees
  - ISC will lead this effort, with support from the TSC, CCC, FCC and PACC
  - Previous work done by (now decommissioned) IRDiRC Working Group on Registries and Natural History will also be consulted
- ▶ A teleconference among interested stakeholders will take place on July 20, 2018 to discuss this idea further and define a path forward

#### **5. Election of the ISC Chair and/or Vice Chair**

The Chair announced her intention to step down from her role as her new professional responsibilities preclude her from actively lead the ISC; she will however continue to contribute in her capacity as ISC member and part of the leadership of the MCC Task Force. She is also available to answer any question regarding the role and responsibilities of ISC Chair any interested member may have.

- ▶ Any ISC member, including current Vice Chair, may put forward his/her candidacy for the position
  - Should the Vice Chair decide to bid for the position of the Chair, the position of the Vice Chair will also be opened for new candidacy
    - Post meeting note: The Vice Chair will contest for the position of the Chair, therefore both positions of Chair and Vice Chair are open for nominations
- ▶ Procedures of the election
  - Nomination/application period
    - Will open for 2-3 weeks following the call of nomination
    - Interested ISC member sends in a short bio and motivational paragraph
    - Please clearly indicate the position (Chair or Vice Chair) contested
  - Electronic voting period
    - Sci Sec will compile applications received and send document to all ISC members
    - Electronic (and anonymous) vote will open for 1 week
  - Result announcement

- The Sci Sec will announce the outcome of the electronic vote

- ISC Vice Chair to inform the Sci Sec regarding her decision if to be an ISC Chair candidate
- Sci Sec to send an email to all ISC members with candidacy and election procedures
- Interested ISC members to send a short bio and motivational paragraph to the Sci Sec

## 6. Any other business: Profile of potential new ISC members

The mandate of several members will end in early 2019. The search for potential new ISC members should commence shortly to ensure maintenance of ISC's capacity and expertise.

- ▶ In order to reach a wide pool of candidates with relevant expertise, a document with the profile sought after is being prepared and may be published, pending nomination strategy decided and could include one or more following elements:
  - Nominations by ISC members
  - Nominations by Consortium Assembly members
  - Announcements of openings at international rare disease conferences
  - Announcements of openings in the IRDiRC newsletter
  - Announcements of openings on the IRDiRC website and social media account
- ▶ Based on the discussion the ISC had in Vienna, the following profiles were defined:
  - Overall profile for Scientific Committee members:
    - Visionary and strategic leaders
    - Thinkers and doers
    - Play well with others to advance the goal of the Committee
    - Experts with a broad awareness of the field
    - Collectively diverse in terms of expertise, gender, and geography
    - Able to commit fairly substantial amount of time to the Committee in the upcoming three years
  - Specific profile for the ISC in term of expertise:
    - Health Technology Assessment (HTA) and its related fields
    - Payers and insurers across different jurisdictions
    - Ethical, legal and social implications of research
- ▶ Further feedback regarding the profile:
  - To include expertise in psycho-social aspects of rare diseases (e.g. psychiatrists, sociologists, psychologists)
  - To bear in mind that members will work with the ISC for at least 3 years
    - Important emphasis on high-level, broad awareness and not only focused expertise given the role of the SC to prepare and advise Consortium Assembly on future IRDiRC scientific programming

- Sci Sec to send the draft profile description for potential new members to ISC members for review
- ISC members to send comments/suggestions to update the profile document

## Next steps and actions

- ▶ Review and provide feedback/corrections to the Vienna meeting report
- ▶ Communicate the PPRL article as soon it is online
- ▶ Inform ISC members on candidacy and election procedures for the position of ISC Chair
- ▶ Send a short bio and motivational paragraph for the position of ISC Chair
- ▶ Review and provide feedback to the draft profile of potential new ISC member
- ▶ Send suggestions of experts in health economics