



**INTERNATIONAL  
RARE DISEASES RESEARCH  
CONSORTIUM**

**GOVERNANCE**

Updated: February 2015  
(Previous version: September 2013)



**IRDIRC**

The International Rare Diseases Research Consortium (IRDiRC) brings together members that share the common goals and principles described in this document and have agreed to work in a coordinated and collaborative manner within a multinational consortium.

**IRDiRC AIMS**

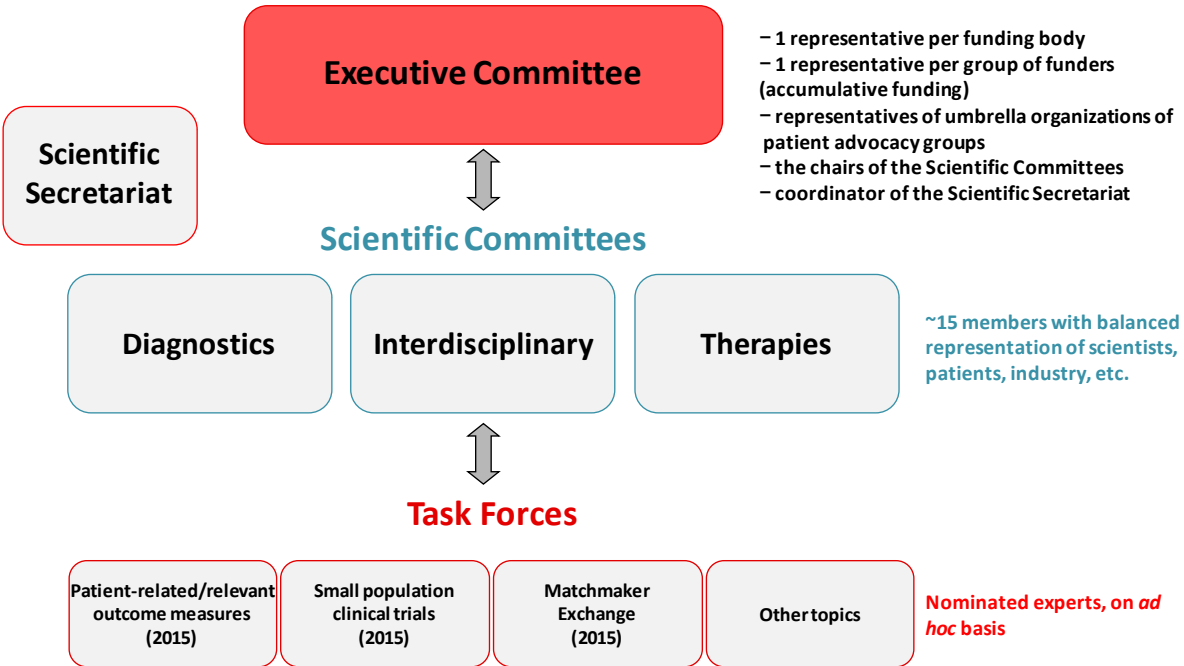
IRDiRC teams up researchers and organisations investing in rare diseases research (hereinafter referred to as funding bodies) in order to achieve two main objectives by the year 2020, namely to deliver **200 new therapies** for rare diseases and means to diagnose **most rare diseases**.

A number of major challenges will need to be addressed through collaborative actions to reach these goals such as:

- ▶ Establishing and providing access to harmonised data and samples,
- ▶ Performing the molecular and clinical characterisation of rare diseases,
- ▶ Boosting translational, preclinical and clinical research, and
- ▶ Streamlining ethical and regulatory procedures.

**IRDiRC GOVERNANCE**

IRDiRC is governed through an Executive Committee and three Scientific Committees, aided by *ad hoc* Task Forces that replace Working Groups which have completed their missions. The Scientific Secretariat provides organisational and communication support. The mandate and composition of these bodies are described below:





## **IRDiRC Executive Committee**

### **Mandate of the Executive Committee**

The IRDiRC Executive Committee:

- ▶ Adopts IRDiRC policies and guidelines
- ▶ Coordinates research funding strategies to address identified research priorities such as those proposed by the Scientific Committees
- ▶ Monitors progress and provides updates to funding bodies
- ▶ Reviews nominations of and accepts new members to the Scientific Committees
- ▶ Agrees on a communication strategy that ensures timely and correct dissemination regarding IRDiRC objectives and progress made
- ▶ Provides a forum for resolution of any conflicts, should they arise

### **Composition of the Executive Committee**

The IRDiRC Executive Committee is composed of one representative per funding body, or group of funders (for small funders), representatives of patient umbrella organisations, the Chairs of each of the three Scientific Committees, and the Project Coordinator of the Scientific Secretariat. These individuals are nominated and appointed according to the following principles:

- **IRDiRC Funding Bodies**

To be considered as an IRDiRC funding member, the funding body should commit a minimum of US\$ 10 million over 5 years of future funding of research projects/programmes contributing towards IRDiRC objectives.

Each funding body can nominate one representative to the Executive Committee. Previous investments since 1 January 2010 contributing to IRDiRC objectives should also be disclosed to IRDiRC to enable networking of presently active research teams.

In the interest of keeping the size of the Executive Committee manageable, countries with more than one committed funding body may consider designating a single common representative.

All membership requests must be sent in writing to the Chair of the IRDiRC Executive Committee. The Chair decides whether to approve or decline each request.

- **IRDiRC group of funders**

Funding organisations that would like to contribute to IRDiRC, but who cannot reach the minimum required investment for membership and/or provide funding for only a single rare disease or a subgroup of rare diseases, may form a group of funders that together reach the threshold for membership (i.e., US\$ 10 million over a 5-year period). Each such group of funders can nominate one representative to the Executive Committee.

- **Umbrella organisations of patient advocacy groups**

The Executive Committee invites representatives of umbrella organisations of patient advocacy groups to serve on the Committee. These umbrella organisations should represent broad patients' interests for a wide variety of diseases and geographical area. The representatives will be considered as full members of the Executive Committee.

- **IRDiRC Scientific Committees**

The Scientific Committees are represented by the respective Chairs at Executive Committee meetings. The Chairs have voting rights in all matters except in the event of actual or potential conflicts of interest.

- **IRDiRC Scientific Secretariat**

The Scientific Secretariat is represented by the Coordinator of Support-IRDiRC, a European Commission-funded contract supporting the activities of the Scientific Secretariat, at Executive Committee meetings, with voting rights in all matters except in the event of actual or potential conflicts of interest.

- **Observers and representatives of advisory bodies**

Upon proposal from a member, the Executive Committee can decide to invite observers to its meetings or representatives of organisations that may be given an advisory role such as regulatory bodies. Such invitations shall be done by the Chair in consultation with the members of the Executive Committee.

## **Meetings and Rules of Procedure**

The Executive Committee meets at least twice a year. The Chair of the Committee calls the meetings and prepares the meeting agenda with input from the other Committee members.

The Executive Committee aims to take decisions by consensus. If a decision cannot be reached by consensus, it is reached through a majority vote, based on the number of members present at the respective meeting.

In a meeting, the participation of 50% of the voting members of the Executive Committee is considered to constitute a quorum. If a quorum cannot be reached at a meeting, the Chair of the Committee may seek prior input from sufficient members to constitute a quorum (for instance e-mail voting ahead of time should Executive Committee members be unavailable at the date of the meeting).

Replacements of Executive Committee members are possible as Executive Committee members represent their organisations. Substitution of alternate representatives is made by the authorising person in the organisation.

The Executive Committee can take decisions by e-mail (written procedure) unless any member opposes the use of this procedure with regard to a particular matter. In a written procedure, a majority of members needs to give its active or tacit approval to render the decision valid.

Unless an exception to this rule can be duly justified, all items for decision at any given meeting should be communicated to the Executive Committee members at least fourteen calendar days in advance of the meeting date. A majority of the Committee members may nonetheless agree that urgent matters be tabled for discussion and decision at a meeting until the adoption of the final meeting agenda.

### **Chair of the Executive Committee**

The IRDiRC Executive Committee elects a Chair from among its members. The Chair is elected for a maximum period of three years. The main responsibilities of the Chair include convening and chairing the meetings of the Executive Committee, approving new members and supervising the Scientific Secretariat.

### **Vice Chair of the Executive Committee**

The IRDiRC Executive Committee elects a Vice Chair from among its members. The main responsibilities of the Vice Chair include stepping in to chair the meetings of the Executive Committee in the absence of the Chair, and assist the Chair on requested tasks.

The Vice Chair will be shortlisted for the election of the next Chair, alongside other candidates.

## **Non-disclosure and Non-conflict of Interest**

All members of the Executive Committee sign confidentiality and non-conflict of interest declarations.

Members of the Executive Committee shall not seek nor act in any way, either in their personal capacity or as a representative of the nominating organisation, as to take undue advantage of, or exercise undue influence on, any aspects regarding the implementation of IRDiRC.

Members of the Executive Committee may participate in projects funded according to IRDiRC objectives, either in their personal capacity or as a representative of the organisations to which they belong. They may also participate in the evaluation or selection of proposals for funding according to IRDiRC objectives.

However, should any item on the agenda in a given meeting be of any relevance, actual or potential, for a proposal for funding that a member, or the organisation to which he/she belongs, has submitted or is likely to submit, the member shall inform the Chair of the Executive Committee of the situation without undue delay. The Chair of the Executive Committee ensures the appropriate handling of any potential conflict of interest. The Committee member can be requested to abstain from the deliberations and/or leave the room for the discussion of the concerned item/subject.

Moreover, the Executive Committee members inform the Chair of any interests, actual or potential, explicitly or not explicitly stated above, which may be considered as prejudicial to their role on the Committee.

When a member of the Executive Committee is in breach of the requirements set out above, he/she shall be considered as no longer being in a position to continue his/her duties as a member of the group.

## **Transparency**

The members should respect the confidential character of the discussions at the Executive Committee meetings.

The names of the members of the Executive Committee are made public via the IRDiRC website. Summary reports from the meetings are published on the IRDiRC website unless the Executive Committee decides otherwise.



## **IRDiRC Scientific Committees**

IRDiRC has three Scientific Committees, one each for Diagnostics, Therapies and Interdisciplinary aspects of rare disease research, advising the Executive Committee on research priorities and progress made from a scientific viewpoint.

### **Mandate of the Scientific Committees**

The Scientific Committees:

- ▶ Act as scientific coordinating bodies
- ▶ Propose research priorities for consideration by the Executive Committee
- ▶ Propose policies and guidelines for adoption by the Executive Committee
- ▶ Identify actionable projects and contribute to the organisation of workshops
- ▶ Contribute to the establishment of Task Forces to push forward selected projects
- ▶ Evaluate, validate and share the outcomes of project workshops
- ▶ Propose reviewers to review submissions for “IRDiRC Recommended”
- ▶ Present the Rapporteur’s report for “IRDiRC Recommended”
- ▶ Address arising issues of scientific nature
- ▶ Organise the scientific programme of IRDiRC conferences as they occur
- ▶ Encourage exchange of protocols and best practices, and agree on standard operating procedures, quality standards and a roadmap to reach IRDiRC goals in their scientific area

### **Composition of the Scientific Committees**

Each Scientific Committee is composed of approximately 15 members with a balanced expertise and representation from academia, patient organisations, diagnostics, pharmaceutical industry, and regulatory bodies.

Scientific Committee members are appointed by the Executive Committee from a list of candidates nominated by:

- ▶ Funding bodies represented in the Executive Committee
- ▶ Patient organisations representing a group of diseases, or being umbrella organisations
- ▶ Industry associations (biotech, diagnostics and pharmaceutical)
- ▶ Research organisations active in rare disease research
- ▶ Learned societies
- ▶ Foundations active in the area of rare diseases

Individuals representing funders in the Executive Committee cannot be nominated or selected to also participate in a Scientific Committee.

The duration of the initial mandate of the Scientific Committee members is three years with the possibility of renewal for an additional period of three years. The mandate of a Scientific Committee member can be terminated for reason of non-participation at the discretion of the chair of the Scientific Committee. In that case, in consultation with the Chair of the Scientific Committee, the Executive Committee will decide whether a replacement appointment is necessary, and a call for nomination will be made.

The Chair of each Scientific Committee is elected by the members of that Scientific Committee.

### **Meetings of the Scientific Committees**

Each Scientific Committee meets at least once a year. The meetings are organised by the Scientific Secretariat and the travel expenses are covered by SUPPORT-IRDIRC.

Members of the Scientific Committees are nominated for their individual expertise and are not empowered to delegate attendance at a meeting to a substitute.

### **Non-disclosure and Non-conflict of Interest**

Members of the Committees shall not seek nor act in any way as to take undue advantage of, or exercise undue influence on, any aspects regarding the implementation of IRDiRC, including but not limited to common policies and guidelines.

Members of the Scientific Committee may participate in projects funded according to IRDiRC objectives, either in their personal capacity or as a representative of the organisations to which they belong. They may also participate in the evaluation or selection of proposals for funding according to IRDiRC objectives.

However, should any item on the agenda in a given meeting of a Scientific Committee be of any relevance, actual or potential, for a proposal for funding that a member, or the organisation to which he/she belongs, has submitted or is likely to submit, the member shall inform the Chair of the Executive Committee and the Chair of the relevant Scientific Committee of the situation without undue delay. He/she can be requested to abstain from the deliberations and/or leave the room for the discussion of the concerned item/subject.

Members of the Scientific Committees inform the Chair of the IRDiRC Executive Committee and the Chair of the relevant Scientific Committee of any interests, actual or potential, explicitly or not explicitly stated above, which may be considered as prejudicial to their independence.



When a member of the Scientific Committee is in breach of the requirements set out above, he/she is considered as no longer being in a position to remain a member of the group.

## **Transparency**

The names of the members of the Scientific Committees are made public via the IRDiRC website. Summary reports from the meetings of the Scientific Committees are published on the IRDiRC website unless the Executive Committee decides otherwise.

## **Nomination and Decision Procedure for the Scientific Committees**

Each funding body represented in the Executive Committee can nominate one person to one of the three Scientific Committees.

The Chair proposes the composition of the Scientific Committees from the nominations received for decision by the Executive Committee.

If there is no vacant position on a specific Scientific Committee at the time of a nomination, the Chair may propose an alternative arrangement until a position becomes available.

In the event of vacancies on the Scientific Committees, the Executive Committee may ask for nominations from patients and industry associations, learned societies and research organisations. All nominations are sent in writing to the Chair of the IRDiRC Executive Committee.

## IRDiRC Task Forces

Working Groups' brainstorming periods and discussions have reached maturity, completing the mission they have been mandated. Active members of Working Groups with specific expertise may be invited to participate in *ad hoc* Task Forces to push forward policy change in specific research areas selected as prioritized topics by the Executive Committee.

### Objectives of Task Forces

Task Forces will be constituted according to the following objectives:

- ▶ Topics specific to rare diseases
- ▶ High leverage projects with strong translational potential and international scope
- ▶ Actions for international scope and relevance
- ▶ Projects that have not been covered by international initiatives
- ▶ Well targeted action, with potential to produce results before 2020
- ▶ Actionable projects with secured human and financial resources
- ▶ Clear objectives and timelines to improve participation and member motivation
- ▶ Coordination with other organisations to identify gaps and needs
- ▶ Alignment and harmonisation of projects with other international initiatives

### Mandate of Task Forces

Task Forces:

- ▶ Organise topic-specific workshops
- ▶ Review and validate concept papers for the workshops
- ▶ Review and validate the final workshop programmes
- ▶ Contribute to the workshops
- ▶ Produce and disseminate workshop reports
- ▶ Push forward implementation of workshop outcomes

### Composition of Task Forces

Members of Task Forces are nominated based on their specific expertise in the selected fields and include key players of diverse backgrounds (e.g. academia, industry, regulatory, advocacy) to ensure different needs are met. Priority is given to project leaders of research projects funded by IRDiRC members in the specific fields selected for action.



## **IRDiRC Scientific Secretariat**

### **Mandate of the Scientific Secretariat**

The mandate of the Scientific Secretariat is to provide organisational and communication support to the IRDiRC and its various members, and thereby contribute to the development of policies and guidelines aimed at accelerating research on rare diseases and at reinforcing international research cooperation.

It supports the work of IRDiRC by:

- ▶ Organising meetings of the IRDiRC members
- ▶ Keeping all members updated
- ▶ Assuring secretarial work for the Consortium
- ▶ Conducting, upon request from and/or in direct consultation with the IRDiRC Committees or Task Forces, the preparation of any document necessary such as bibliographic research or synthesis on a theme
- ▶ Organising workshops to advance the work of the Task Forces
- ▶ Collecting and diffusing pertinent information and results to the researchers funded by IRDiRC members
- ▶ Disseminating results of IRDiRC projects with different means of communication: website, newsletters, communication materials, conferences

The Scientific Secretariat reports to the Executive Committee for its work plan and its activity report.



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