



**INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM**

GOVERNANCE

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

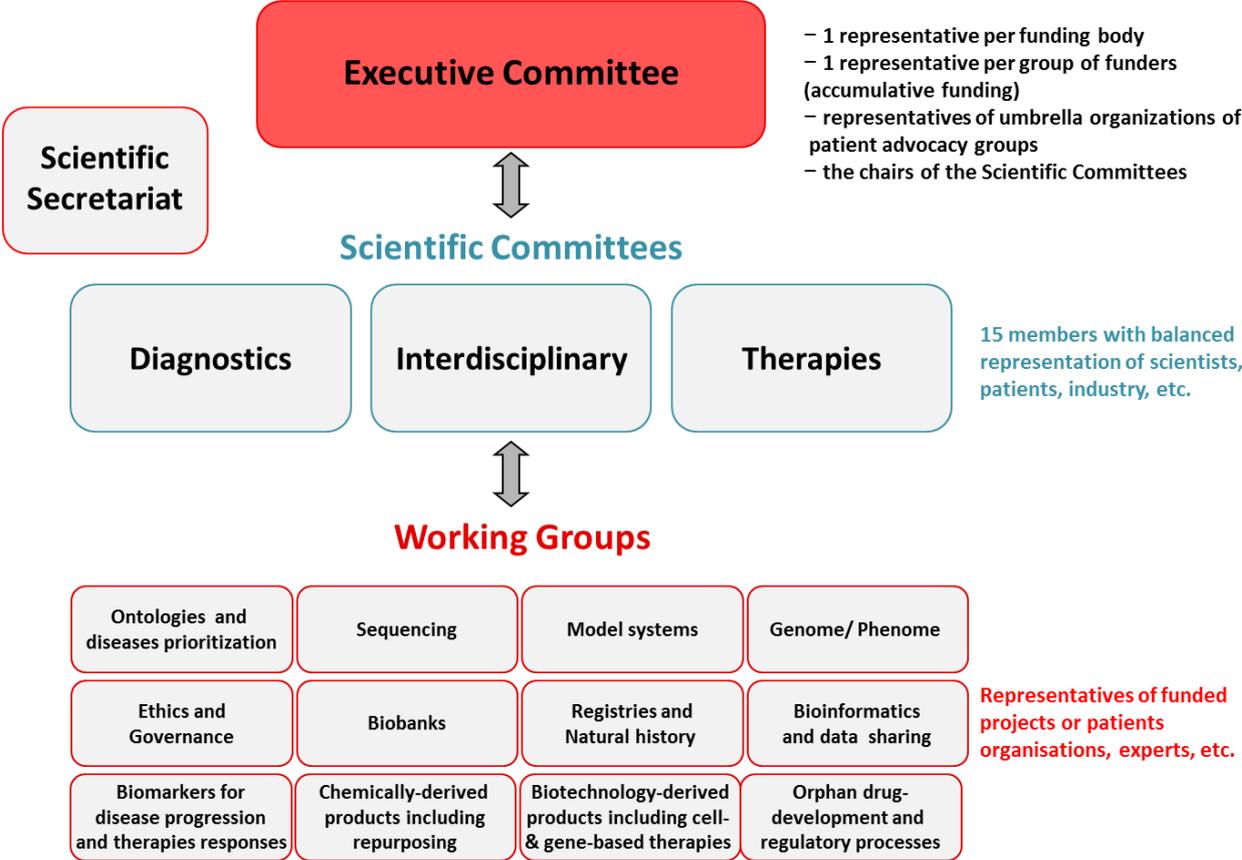
IRDiRC teams up researchers and organisations investing in rare diseases research (hereinafter referred to as funding bodies) in order to achieve two main objectives by the year 2020, namely to deliver **200 new therapies** for rare diseases and means to diagnose **most rare diseases**.

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,
- ▶ streamlining ethical and regulatory procedures.

IRDiRC GOVERNANCE

IRDiRC is governed through an Executive Committee, three Scientific Committees and twelve Working Groups. 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 the small funders), representatives of patient umbrella organisations, and the Chairs of each of the three Scientific Committees. 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 \$10 million US 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 Jan 2010 contributing to IRDiRC objectives should also be disclosed to IRDiRC to help 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 organizations 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., \$10 million US 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.

- **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 enough 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.

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
- ▶ Assess and report on progress made by the Working Groups (i.e., projects funded)
- ▶ 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, roadmap to reach IRDiRC goals in their scientific area
- ▶ Define the missions of the Working Groups.

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 twice 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 stay as 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.

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.

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

IRDiRC Working Groups

Mandate of the Working Groups

The Working Groups:

- ▶ Identify major ongoing national, European or international initiatives in its field of interest to maximize worldwide awareness of these projects.
- ▶ Point out the problems and difficulties in the scope of the WG that ultimately prevent or delay in developing new diagnostics and therapies
- ▶ Recommend actions or solutions to resolve gaps, problems and difficulties in the scope of the WG

- ▶ Cooperate to ensure synergies of all research projects within the scientific area of the working group, by exchanging results, expertise, experiences and information
- ▶ Analyse and propose standards to be promoted (e.g., quality standards, performance indicators) for RD research
- ▶ Report back on the implementation of IRDiRC policies and guidelines in RD research projects and propose changes where necessary
- ▶ Report to the Scientific Committee that established the WG

Composition of the Working Groups

The working groups are composed of representatives from funded projects that contribute to IRDiRC objectives within the scientific domain of the working group, additional experts and representatives of patient organizations that are nominated by the Executive Committee or the relevant Scientific Committee. Composition of the working groups should have a geographically balanced representation. New members are included at regular intervals. Generally, working groups consist of about 5-10 individuals. Members of the working groups nominate a coordinator (chair) and define the modus operandi of the group. One or several members of the Scientific Committee participate in each working group to facilitate the bidirectional exchange of ideas. The composition of the working groups should be available to the Executive Committee for information.

Meetings of the Working Groups

Working Groups meetings are organized by the Scientific Secretariat via tele- and videoconferencing or webcasting. Working groups are expected to meet every 3 months, but may convene more frequently at their own discretion.

List of Working Groups

- ▶ Working Groups reporting to the Diagnostics Scientific Committee:
 - Ontologies and disease prioritisation
 - Sequencing
 - Model systems
 - Genome / Phenome

- ▶ Working Groups reporting to the Interdisciplinary Scientific Committee:
 - Ethics and governance
 - Biobanks
 - Registries and natural history

- Bioinformatics and data sharing
- ▶ Working Groups reporting to the Therapies Scientific Committee:
 - Biomarkers for disease progression and therapy response
 - Chemically-derived products including repurposing
 - Biotechnology-derived products including cell- & gene-based therapies
 - Orphan drug-development and regulatory processes

IRDiRC Scientific Secretariat

Mandate of the Scientific Secretariat

The mandate of the scientific secretariat is to provide organisational and communication support to the IRDiRC consortium 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 the IRDiRC consortium by:

- ▶ organizing meetings of the IRDiRC members
- ▶ keeping all members updated
- ▶ assuring secretarial work for the consortium
- ▶ conducting, upon request, the preparation of any document necessary to the IRDiRC committees and working groups, such as bibliographic search or synthesis on a thematic
- ▶ 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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