INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

GOVERNANCE

Updated: November 2018

(Previous versions:
Sept 2013, Feb 2015, Mar 2016, Apr 2017)

IRDiRC

Table of Content

IRDiRC Goals

The International Rare Diseases Research Consortium (IRDiRC) unites stakeholders that share a joint commitment to rare diseases research and the common principles described in this document, and have agreed to work in a coordinated and collaborative manner within a multinational consortium.

At its launch in 2011, IRDiRC set two main goals to be achieved by the year 2020, namely: to deliver 200 new therapies for rare diseases, and the means to diagnose most rare diseases. These were largely accomplished by early 2017 and spurred the Consortium to establish new audacious goals for the following decade (2017-2027):

IRDiRC's vision for the field:

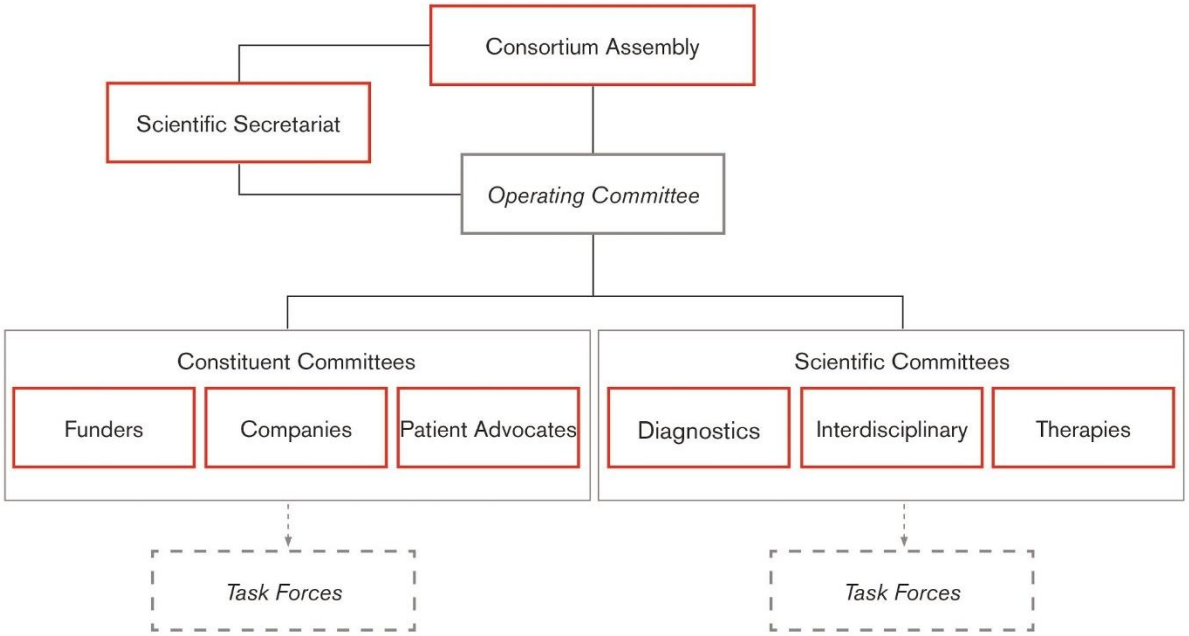
- **Vision:** Enable all people living with a rare disease to receive an accurate diagnosis, care, and available therapy within one year of coming to medical attention.

IRDiRC is committed to achieving the following three goals in the upcoming decade, through collaboration among its researchers, and organizations investing and advocating in the field of rare diseases research, in order to advance the realization of its vision for the field:

- **Goal 1:** All patients coming to medical attention with a suspected rare disease will be diagnosed within one year if their disorder is known in the medical literature; all currently undiagnosable individuals will enter a globally coordinated diagnostic and research pipeline.
- **Goal 2:** 1000 new therapies for rare diseases will be approved, the majority of which will focus on diseases without approved options.
- **Goal 3:** Methodologies will be developed to assess the impact of diagnoses and therapies on rare disease patients.

IRDiRC Governance

IRDiRC is governed through a Consortium Assembly, an Operating Committee, three Constituent Committees and three Scientific Committees, aided by *ad hoc* Task Forces. The Scientific Secretariat provides organizational and communication support.



The mandate and composition of these bodies are described below.

1. *Consortium Assembly*

1.1 Mandate of the Consortium Assembly

The IRDiRC Consortium Assembly:

- Acts as the primary forum for information exchange for issues influencing IRDiRC goals and activities
- Coordinates scientific and policy efforts to address identified research priorities proposed by the Constituent and Scientific Committees, that will advance IRDiRC goals
- Adopts IRDiRC policies and guidelines
- Reviews nominations and accepts new members to the Scientific Committees
- Reviews proposals and approves new Task Forces
- Nominates and appoints Task Force members, in concert with the Scientific Committees
- Reviews and agrees on communication strategies that ensure timely and accurate dissemination of information regarding IRDiRC objectives and progress made

1.2 Membership of the Consortium Assembly

All membership requests must be sent in writing to the Chair of the IRDiRC Consortium Assembly and the Scientific Secretariat. For IRDiRC membership consideration, the organization submits a Letter of Motivation stating the reasons for desiring to join IRDiRC, and a Letter of Intent signed by the organization's legal representative. The Operating Committee will review membership requests and determine whether to approve or decline. IRDiRC actively seeks and encourages organizations from non- or under-represented geographical regions to join due to the critical importance for IRDiRC to be representative of the global rare disease community.

The Consortium Assembly members are responsible for timely responses and transfer of information back to their organizations. To remain in good standing, Consortium Assembly members must attend/participate in at least two Consortium Assembly meetings and/or teleconferences per year. If not, the member will be notified of a 6-month probationary period, during which time s/he must attend a meeting. Inactive members' membership will be terminated, and they will be removed from the IRDiRC listing and Consortium Assembly-related communications. (Also see Section 1.5: "Meetings and Rules of Procedure")

As a condition of membership (except for umbrella organizations of patient advocacy groups), the Scientific Secretariat will collect updated information regarding funding commitment toward IRDiRC objectives on an annual basis to ensure that member organization continue to meet the minimum commitment.

Additionally, the Scientific Secretariat will also collect, in writing, the developments at member organizations relevant to the IRDiRC mission and to Consortium Assembly members on a bi-annual basis. The information provided should be non-proprietary and include 2-3 key points per year as a high-level collection of programmatic activities relevant to the IRDiRC goals and vision.

1.3 Composition of the Consortium Assembly

The IRDiRC Consortium Assembly is comprised of one representative per member funding body, group of funders (for small funders), company, umbrella patient advocacy organization, and the Chair and Vice Chair of each of the three Scientific Committees.

These individuals are approved according to the following principles:

1.3.1 Government and non-profit funding bodies

To be considered as an IRDiRC Funding member, the government and/or non-profit funding body should commit a minimum of US$ 10 million over 5 years of future funding of research projects/programs contributing towards IRDiRC goals. Each government and/or non-profit funding body can nominate one representative to the Consortium Assembly, who will also serve on the Funders Constituent Committee.

1.3.2 Group of funders

Funding organizations that would like to contribute to IRDiRC, but which cannot reach the minimum required investment for membership on their own 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 Consortium Assembly, who will also serve on the Funders Constituent Committee.

1.3.3 Companies

To be considered as an IRDiRC Companies member, the company should commit a minimum of US$ 10 million over 5 years of future funding of research projects/programs contributing towards IRDiRC goals. Each company can nominate one representative to the Consortium Assembly, who will also serve on the Companies Constituent Committee.

1.3.4 Umbrella patient advocacy organizations

To be considered as an IRDiRC Patient Advocacy Group member, the umbrella organization must be a patient organization (1) representing broad patients' interests for all rare diseases in at least one country or larger area and (2) contributing to research that shares and will advance the IRDiRC vision and goals (e.g., developing and providing tools to accelerate research, diagnostic and therapeutic development, evaluation of processes). It is essential that all individuals affected by rare conditions have a voice to enhance visibility and international collaboration, and mutual

exchange of knowledge and experience. In general, IRDiRC expects patient advocacy groups that are members of an umbrella organization already a member of IRDiRC to be represented appropriately by that organization within IRDiRC. Once approved, each umbrella organization can nominate one representative to the Consortium Assembly, who will also serve on the Patient Advocates Constituent Committee. IRDiRC recommends that the organization nominate a representative who has direct experience with a rare disease and has worked in the interest of patients for at least a year.

1.3.5 IRDiRC Scientific Committees

Chairs and Vice Chairs of Scientific Committees are members of the Consortium Assembly and represent their respective Scientific Committee, as a whole; each Scientific Committee gets one vote. All other Scientific Committee members are not Consortium Assembly members, but upon proposal from a Consortium Assembly member, may be invited as observers to its meetings. Such invitations will be issued by the Chair of the Consortium Assembly in consultation with the Operating Committee members.

1.4 Other Participants of the Consortium Assembly

1.4.1 IRDiRC Scientific Secretariat

The Scientific Secretariat is represented at Consortium Assembly meetings, except in matters in which it has, or could reasonably be perceived to have, a conflict of interest. The Scientific Secretariat representative(s) does not have voting rights at meetings of the Consortium Assembly.

1.4.2 Observers

Upon proposal from a member, the Consortium Assembly can decide to invite observers, such as representatives of regulatory bodies or learned societies, to its meetings; an observer may be given an *ad hoc* advisory role. Such invitations shall be issued by the Chair of the Consortium Assembly in consultation with the members of the Operating Committee. Invited observers do not have voting rights at meetings of the Consortium Assembly.

1.5 Meetings and Rules of Procedure of the Consortium Assembly

The Consortium Assembly meets at least twice a year in-person and an additional few times by teleconference. The Chair of the Consortium Assembly calls the meetings and prepares the meeting agenda with input from the other Consortium Assembly members. To remain in good standing, Consortium Assembly members must attend/participate in at least two meetings and/or teleconferences per year.

Consortium Assembly members represent their organizations. If a member is unavailable to attend a meeting, substituted participation by an alternate representative can be made by the

authorizing person in the organization; advance notice must be given in writing to the Chair of the Consortium Assembly and the Scientific Secretariat. Alternatively, if a member cannot attend a meeting, sending comments via email beforehand in response to meeting documents will also count as participating in the meeting.

In a meeting, a quorum is present when 50% of the Consortium Assembly voting members are in attendance and participation.

The Consortium Assembly aims to make 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, assuming a quorum is present.

If a quorum cannot be reached at a meeting, or if timing is of the essence, the Chair of the Consortium Assembly may call for a vote or ask for feedback by written procedure, such as e-mail and online survey. In any case of decision making, a quorum of all members (i.e. 50%) must respond to the request for its results to be valid.

Unless an exception to this rule can be duly justified, all items for decision-making at any given meeting should be communicated to the Consortium Assembly members at least fourteen calendar days in advance of the meeting date. At any time, members of the Consortium Assembly, Constituent Committees, Scientific Committees, and Task Forces can send items to the Scientific Secretariat for the Consortium Assembly to consider and discuss.

1.6 Chair of the Consortium Assembly

The IRDiRC Consortium Assembly elects a Chair from among its members. The Chair is elected for a maximum period of three years and can be nominated for re-election to a further term, not to exceed two consecutive terms. The main responsibilities of the Chair include advancing the Consortium's goals and activities, convening and chairing the meetings of the Consortium Assembly and Operating Committee, and overseeing the Scientific Secretariat.

1.7 Vice Chair of the Consortium Assembly

The IRDiRC Consortium Assembly elects a Vice Chair from among its members. The Vice Chair is elected for a maximum period of three years and can be nominated for re-election to a further term, not to exceed two consecutive terms. The main responsibilities of the Vice Chair include stepping in to chair the meetings of the Consortium Assembly and Operating Committee in the absence of the Chair, and assist the Chair on requested tasks. The Vice Chair is not automatically Chair-elect; s/he may be shortlisted for the election of the Chair, alongside other candidates.

2. *Operating Committee*

The Operating Committee meets regularly to prepare and advance IRDiRC activities, process information, and enable more effective management of the consortium as a whole. The Operating Committee is not an executive decision-making body.

2.1 Mandate of the Operating Committee

The Operating Committee:

- Monitors progress of IRDiRC activities and goals
- Manages progress, processes information, and provides updates to members
- Reviews nominations and accepts new members to the Consortium Assembly
- Reviews Consortium Assembly meeting agendas
- Keeps the Consortium Assembly informed of all activities of the Operating Committee
- Reviews membership needs of the Scientific Committees
- Provides a forum for resolution of any conflicts, should they arise

2.2 Composition of the Operating Committee

The Operating Committee consists of the Chair and Vice Chair of the Consortium Assembly, the Chairs of the Constituent and Scientific Committees, and the Scientific Secretariat. The Vice Chairs of the Committees support the respective Chairs, are integral in information exchange, and attend Operating Committee meetings in the absence of the Chairs.

2.3 Meetings of the Operating Committee

The Operating Committee conducts regular teleconferences and aims to meet at a monthly interval. At any time, members of the Consortium Assembly, Constituent Committees, Scientific Committees, and Task Forces can send items to the Scientific Secretariat for the Operating Committee to consider and discuss.

3. *Constituent Committees*

IRDiRC has three Constituent Committees, one each for the major areas of representation – Funders, Companies, and Patient Advocates. While sharing and coordination of science and the work of scientists is central to IRDiRC goals, so are also sharing and coordination of the work of these three constituents.

3.1 Mandate of the Constituent Committees

The Constituent Committees:

- Act as their constituency's coordinating body
- Identify overlap of priorities and activities, and gaps within each constituent space
- Identify common roadblocks across each constituent space worldwide
- Determine how the constituency will contribute to the goals
- Implement Task Forces and coordinate solutions to address priorities and gaps
- Oversee the activities and progress of Task Forces
- Inform Consortium Assembly and other committees of scientific and programmatic needs
- Coordinate solutions through Task Forces, publications, and other actions

3.2 Composition of the Constituent Committees

Each Constituent Committee is composed of Consortium Assembly members who are identified as part of that constituency. The Chair and Vice Chair of each Constituent Committee are elected by the respective Committee members, and approved by the Operating Committee. The Chair and Vice Chair of each Constituent Committee are elected for a maximum period of three years and can be nominated for re-election to a further term, not to exceed two consecutive terms.

3.3 Meetings of the Constituent Committees

Each Constituent Committee meets in-person during the breakout sessions of Consortium Assembly in-person meetings. Each Constituent Committee also meets by teleconferences convened by the Chair of the respective Constituent Committee.

4. *Scientific Committees*

IRDiRC has three Scientific Committees, one each for Diagnostics, Therapies, and Interdisciplinary aspects of rare diseases research. The Scientific Committees identify scientific issues common to many or all members that limit the achievement of IRDiRC goals, while promulgating their findings and advising the Consortium Assembly on research priorities and progress made from a scientific viewpoint.

4.1 Mandate of the Scientific Committees

The Scientific Committees:

- Act as scientific coordinating bodies
- Encourage exchange of protocols and best practices
- Agree on standard operating procedures, quality standards and a roadmap to reach IRDiRC goals in their scientific area
- Report to the Consortium Assembly with regards to Committee and Task Force activities and progress toward IRDiRC goals in their scientific area
- Propose research priorities for consideration by the Consortium Assembly
- Propose policies and guidelines for adoption by the Consortium Assembly
- Identify actionable projects that would advance IRDiRC goals within their focus area and bring those proposals to the Consortium Assembly
- Contribute to the establishment of Task Forces to advance selected projects
- Nominate members of Task Forces, in concert with the Consortium Assembly
- Oversee the activities and progress of Task Forces
- Evaluate, validate and share the outcomes of Task Forces
- Review and approve submissions for "IRDiRC Recognized Resources"
- Address arising scientific issues within their purview
- Contribute to the preparation of annual State-of-Play report
- Contribute to the organization of scientific programs at IRDiRC conferences or IRDiRC sessions at external conferences

4.2 Composition of the Scientific Committees

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

The Chair and Vice Chair of each Scientific Committee are elected by the respective Committee members, and approved by the Operating Committee. The Chair and Vice Chair of each Scientific Committee are elected for an initial period of three years and can be nominated for re-election

to a further term, not to exceed two consecutive terms, and limited by the mandate of their Scientific Committee membership.

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

Under exceptional circumstances, individual Consortium Assembly members can be nominated to also participate in a Scientific Committee under the stipulation that they are acting in their capacity as an individual scientist and not representing their organization, as a whole. In cases of conflict of interest, the member must recuse him/herself from such discussions and note that to the Chair and Vice Chair of the Scientific Committee, and the Scientific Secretariat.

4.3 Nomination and Membership Procedure of the Scientific Committees

Scientific Committee members are approved by the Consortium Assembly. 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.

In the event of vacancies on the Scientific Committees, the Chairs of the Scientific Committee identify gaps of expertise and complementary areas, and consult with the Operating Committee to put forward a call for nominations. All nominations are sent in writing to the Chairs of the Scientific Committees and the Scientific Secretariat.

Candidates may be nominated by:

- Internal
  - Member organizations represented in the Consortium Assembly
  - Members of IRDiRC Scientific Committees
- External
  - Umbrella patient organizations
  - Industry associations (biotech, diagnostics and pharmaceutical)
  - Research organizations active in rare diseases research
  - Learned societies
  - Foundations active in the area of rare diseases
  - Direct contact via the website to the Scientific Secretariat

The Chairs of the Scientific Committees propose an appropriate composition of committee membership from the nominations received. If there is no vacant position on a specific Scientific Committee at the time of a nomination, a candidacy may be shelved or an alternative arrangement may be proposed until a position becomes available.

The mandate of a Scientific Committee member can be terminated for reason of non-participation at the discretion of the Chair of the relevant Scientific Committee. In that case, in consultation with the Chair of the Scientific Committee, the Operating Committee will decide whether a replacement appointment is necessary, and a call for nomination will be made.

A member who is unable to serve out his/her term may propose a replacement, subject to consideration among other nominees for approval by the Consortium Assembly.

4.4 Meetings of the Scientific Committees

Each Scientific Committee meets at least once a year in-person, and an additional few times by teleconference, convened by the Chair of the respective Scientific Committee. Travel organization and expenses for in-person meetings are provided by the Scientific Secretariat.

5. *Task Forces*

Ad hoc, time-limited Task Forces are created to advance potential solutions and/or policy recommendations in specific research areas proposed by the Committees, and selected as prioritized topics by the Consortium Assembly and the Operating Committee.

5.1 Objectives of the Task Forces

Task Forces are constituted according to the following objectives:

- Topics specific to rare diseases with clear objectives and timelines
- High leverage projects with strong translational potential and international scope
- Actions for international scope and relevance
- Projects that have not been covered by other international initiatives
- Well targeted, actionable projects with potential to produce results before 2027
- Coordination with other organizations to identify gaps and needs
- Alignment and harmonization of projects with other international initiatives

5.2 Mandate of the Task Forces

Task Forces:

- Organize and contribute to topic-specific workshops
- Review and validate concept papers for their Task Forces
- Produce and disseminate workshop reports and publications
- Push forward implementation of Task Force outcomes
- Develop recommendations, actions, and/or results for their Task Force topic area(s)

5.3 Composition of the 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. Joint Task Forces with international initiatives may include members nominated by partnering initiatives, in addition to members nominated by the IRDiRC Consortium Assembly and Committees.

6. *Scientific Secretariat*

The Scientific Secretariat provides organizational and communications support to IRDiRC and its members, therefore contributing to the development of policies and guidelines aimed at accelerating research on rare diseases, reinforcing international research cooperation, and reaching IRDiRC goals.

6.1 Mandate of the Scientific Secretariat

Scientific Secretariat supports the work of IRDiRC by:

- Organizing meetings of the IRDiRC members
- Keeping members updated on IRDiRC activities and initiatives
- Providing 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 topic
- Organizing 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 activities with various means of communication (e.g., website, newsletters, communication materials, conferences)

6.2 Composition of the Scientific Secretariat

The Scientific Secretariat is led by a Coordinator and consists of a team of staff including Project Manager(s), Information Scientist(s), Communication Manager, and Administrative Assistant.

The Scientific Secretariat reports to the Consortium Assembly and Operating Committee for its work plan and activity report. The Chair of the Consortium Assembly oversees the Scientific Secretariat.

Conflict of Interest and Transparency

Non-Disclosure and Non-Conflict of Interest

Members of the IRDiRC Consortium Assembly and Committees shall not seek nor act in any way, either in their personal capacity or as a representative of the nominating organization, as to take undue advantage of, or exercise undue influence on, any aspects regarding the implementation of IRDiRC, including but not limited to common activities, policies, and guidelines.

Members of the IRDiRC Consortium Assembly and Committees shall inform the Chair of the Consortium Assembly, the Chair of relevant Committee (where applicable), and the Scientific Secretariat of any interests (actual or potential), which may be considered prejudicial to their role and/or their independence. Members can be requested to abstain and/or recuse from certain discussions, deliberations, or votes.

Members of the IRDiRC Consortium Assembly and Committees may participate in projects funded according to IRDiRC objectives, either in their personal capacity or as a representative of the organizations to which they belong, on the condition that such participation is disclosed. They may also participate in the evaluation or selection of proposals for funding according to IRDiRC objectives.

When a member of the IRDiRC Consortium Assembly and Committees is in breach of the requirements set out above, s/he shall be considered as no longer being in a position to continue his/her duties as a member of the group.

Transparency

Members should respect the confidential character of the discussions at the respective meetings. The names of the members of the IRDiRC Consortium Assembly and Committees are made public via the IRDiRC website. Summary reports from the meetings are published on the IRDiRC website unless the Consortium Assembly decides otherwise.

IRDiRC

INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM