The International Rare Diseases Research Consortium (IRDiRC)
International Rare Diseases Research Consortium

- History
- Vision and Goals
- Members
- Governance and Operating Model
- Task Forces and Working Groups
- Publications, News and Events
- State-of-Play Reports
- Recognized Resources
- Get involved
IRDIRC was founded in 2011 to accelerate breakthroughs in rare diseases by coordinating research funding.

2010
- 1st workshop in Iceland organized by the European Commission and US National Institutes of Health

2011
- Official launch of IRDIRC with two goals: “to contribute to the development of diagnostic tools for most RDs and of 200 new therapies by 2020”

2012
- Open Calls for New Members: ongoing basis ever since

2015
- Establishment of Task Forces to tackle specific rare disease research topics

2017
- New Strategic Vision 2027: “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”

Today
- Today IRDIRC has tripled in size
- Numerous peer reviewed publications, recommendations and guidelines to expedite research and innovation
- Driving force is the sharing of collective intelligence in a non-competitive space

https://irdirc.org/about-us/history/
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 Goals by 2027

**Goal 1**
Diagnosis Within One Year Research Pipeline

**Goal 2**
1000 New Therapies

**Goal 3**
Methodologies to Assess Impact

IRDiRC Thematic Research Actions

- Shortening the Diagnostic Journey
- Developing New Therapies
- Fostering clinical research
- Stimulating Multi-stakeholder Engagement
- Access to Care and Methodologies

https://irdirc.org/about-us/vision-goals/
Members collaborate, stimulate, coordinate, and leverage output of rare diseases research efforts around the world

- 60 member organizations and over 100 experts
- Unite research funders and companies investing in RD research
  - Each organization funds research its own way
  - Funded projects adhere to a common framework
- Umbrella patient advocacy groups
- 4 Scientific Committees
  - Diagnostics
  - Therapies
  - Interdisciplinary
  - Regulatory

IRDiRC actors

Research Funders

Companies

Patient Advocate Groups

Scientists

Collective Intelligence
Global Expertise
Complementary Competencies
Driver of International Initiatives

https://irdirc.org/constituent-committees/
https://irdirc.org/scientific-committees/
Governance and Operating Model

Consortium Assembly

Scientific Secretariat

Operating Committee

Member Organizations (60 members)
+ Chair and Vice Chair of Scientific Committees

Chair and Vice Chair of Consortium Assembly, and Constituent and Scientific Committees

Constituent Committees
- Companies
- Patient Advocates
- Funders

Scientific Committees
- Regulatory
- Interdisciplinary
- Therapeutics
- Diagnostics

Task Forces and Working Groups

https://irdirc.org/about-us/governance/
Task Forces & Working Groups

Task Forces (TFs) and Working Groups (WGs) are the instruments adopted by IRDiRC to address actionable topics identified by the Consortium and reflecting the evolving priorities for RDs.

**Task Force**
- **Time limited**: 12-18 months.
- **Objectives and scope**: Clearly pre-defined.
- **Commitment**: Monthly conference calls and in-person meeting.
- **Composition**: Leverage on the expertise of internal and external experts.
- **Output**: Recommendation paper, technical application.

**Working Group**
- **Time limited**: 6-9 months.
- **Objectives and scope**: Exploratory work.
- **Commitment**: Monthly conference calls.
- **Composition**: Leverage on the expertise of internal and external experts.
- **Output**: Recommendation paper, IRDiRC report.

[https://irdirc.org/activities/task-forces/](https://irdirc.org/activities/task-forces/)
Publications, News and Events

IRDiRC has developed **RD community engagement instruments** to present its activities, provide a forum for discussions and influence global RD research.

- Recommendations, tools or guidelines by TFs and WGs, and IRDiRC reviews and commentaries are published as peer-reviewed articles or IRDiRC reports.
- Dissemination via the IRDiRC website, newsletters, social media and member/partner channels effectively widens awareness and influence beyond the RD community.
- Biannual conference engaging researchers, clinicians, patient advocates, industry leaders and policy makers from all continents.

State-of-Play Reports aim to inform stakeholders at large of developments in the field of rare diseases research in order to support decisions of policy makers and research funders. At the same time, the report is focused on informing the rare diseases community at large of the achievements and of observed trends, which shape the future of research and development for rare diseases. It is based on a systematic survey of published articles, in scientific journals and press releases.
**Recognized Resources**

**IRDiRC Recognized Resources** label is a quality indicator, based on a specific set of criteria, created to highlight key resources which, if used more broadly, would accelerate the pace of translating discoveries into clinical applications. Any resource compliant with the criteria may apply for the label. Selected platforms, tools, standards, and guidelines must be of fundamental importance to the international rare diseases research and development community.

https://irdirc.org/resources-2/irdirc-recognized-resources/
Get involved

**Become a Member**

**Funders and Companies**
The organization should invest in rare disease R&D for minimum USD 10 million over a 5-year period.
It includes expenses associated with the organization’s internal and/or third-party RD innovation and improvements.
The organization should complete the Letter of Intent and send it to the Scientific Secretariat of IRDiRC.

**Patient Umbrella Groups**
The group should represent patients with different rare diseases (umbrella group) and cover a large region or country.
The organization should complete the Letter of Intent and send it to the Scientific Secretariat of IRDiRC.

**Scientific Contribution**
External experts can apply to become member of one of the IRDiRC Scientific Committees and/or to join a Task Force. Candidates should send their CV, a biosketch and a letter of motivation to the Scientific Secretariat of IRDiRC. Candidates will be contacted based on the specific needs of IRDiRC Scientific Committees and Task Forces.

https://irdirc.org/get-involved/become-a-member/
https://irdirc.org/get-involved/scientific-contribution/
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