



IRDIRC

RECOMMENDED

- ▶ LABEL HIGHLIGHTING TOOLS/STANDARDS/GUIDELINES CONTRIBUTING TO IRDIRC OBJECTIVES
- ▶ IDENTIFICATION OF KEY RESOURCES FOR RESEARCH COMMUNITIES TO ACCELERATE CLINICAL TRANSLATION

▶ GOALS OF “IRDIRC RECOMMENDED” LABEL

- ▶ Quality indicator of tools/standards/guidelines based on specific criteria
- ▶ Public label visible on and by resources
- ▶ Fundamental to rare diseases research & development community
- ▶ Encourages long-term sustainability of tools/standards/guidelines

▶ ASSESSMENT CRITERIA (WHERE APPLICABLE)

- ▶ Within IRDIRC's focus and mission (mandatory)
- ▶ Serves the international community (mandatory)
- ▶ Functional and accessible with minimal downtime
- ▶ Has development and maintenance team
- ▶ Clear terms-of-use and licence policies
- ▶ Adhesion to ethical and privacy policies and requirements
- ▶ Quality control and life cycle management processes
- ▶ Financially viable for at least 3 years post accreditation
- ▶ Freely available non-commercial product
- ▶ Evidence of core impacts (e.g. number of users/visits)

▶ APPLICATION AND REVIEW PROCESS

- ▶ Application open to all project leaders wishing to contribute to IRDIRC goals
- ▶ Call for proposals launched March 2015
- ▶ Submissions and application reviews conducted on an ongoing basis
- ▶ Peer-reviewed reports submitted for approval by IRDIRC Scientific Committee



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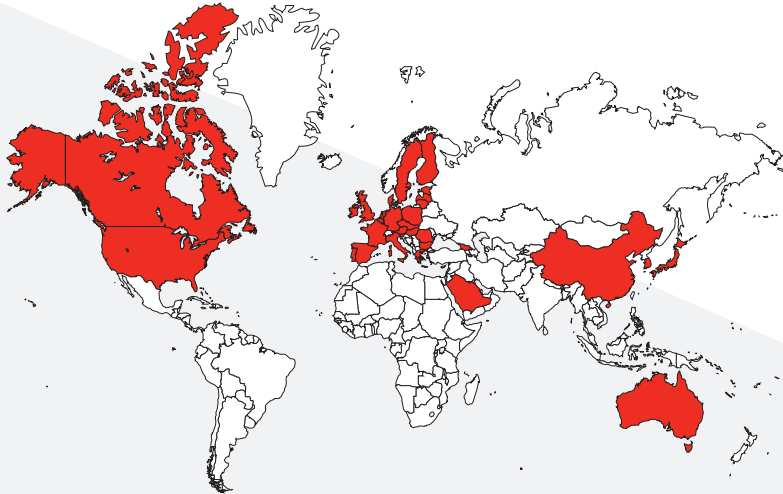
INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM

▶ IRDiRC teams up researchers and organisations investing in rare diseases research to achieve two main objectives:

- ▶ **Deliver 200 new therapies for rare diseases by 2020**
- ▶ **Develop the means to diagnose most rare diseases by 2020**

▶ Members are:

- ▶ **Funding organisations spending more than 10 million USD over 5 years in research projects contributing to IRDiRC objectives**
- ▶ **Government, academia, industry and patient organisations**



Funding organisations from

- Europe
- Asia
- Middle East
- North America
- Australia

www.irdirc.org



ACTIVITY PLAN

2015-2016

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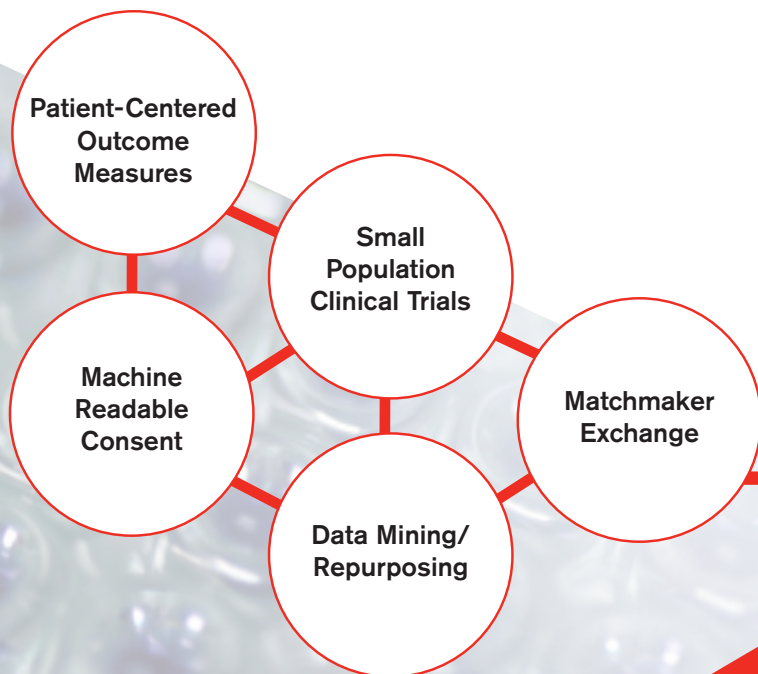
INTERNATIONAL
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TASK FORCES



OBJECTIVES

- ▶ High leverage projects with strong translational potential and international scope
- ▶ Targeted action to produce results by 2020
- ▶ Projects not covered by international initiatives
- ▶ Actionable projects with secured human and financial resources
- ▶ Coordination with other organisations to identify gaps and needs



ROLE OF TASK FORCES

MANDATE

- ▶ Organisation of topic-specific workshops
- ▶ Production, dissemination and implementation of workshop outcomes

COMPOSITION

- ▶ Leaders of research projects in the specific fields selected for action
- ▶ Experts and key players from:
 - Academia
 - Industry
 - Regulatory
 - Advocacy groups

○ Matchmaker Exchange

○ Machine Readable Consent

○ Patient-Centered
Outcome Measures

○ Small Population
Clinical Trials

○ Data Mining/
Repurposing

Q4

Q1

Q2

2015

2016