



Fostering patient involvement in international research

International Rare Disease Research Consortium

Sharon F. Terry, Genetic Alliance



Disclaimer

*I do not speak on behalf of the
International Rare Disease Research
Consortium – these are my own
thoughts and have not been shared
with the Executive Committee*

International Rare Disease Research Consortium

Cooperation at international level to stimulate, better coordinate and maximize output of rare disease research efforts around the world

IRDiRC – basic principles

- ▶ Cooperation at international level to stimulate, better coordinate & maximize output of rare disease research efforts around the world



- ▶ Teams up public and private organizations investing in rare diseases research
- ▶ Research funders with relevant programs >\$10 million US over a 5-year period can join & work together
- ▶ Each organization funds research its own way
- ▶ Funded projects adhere to a common framework

Goals of IRDiRC by 2020

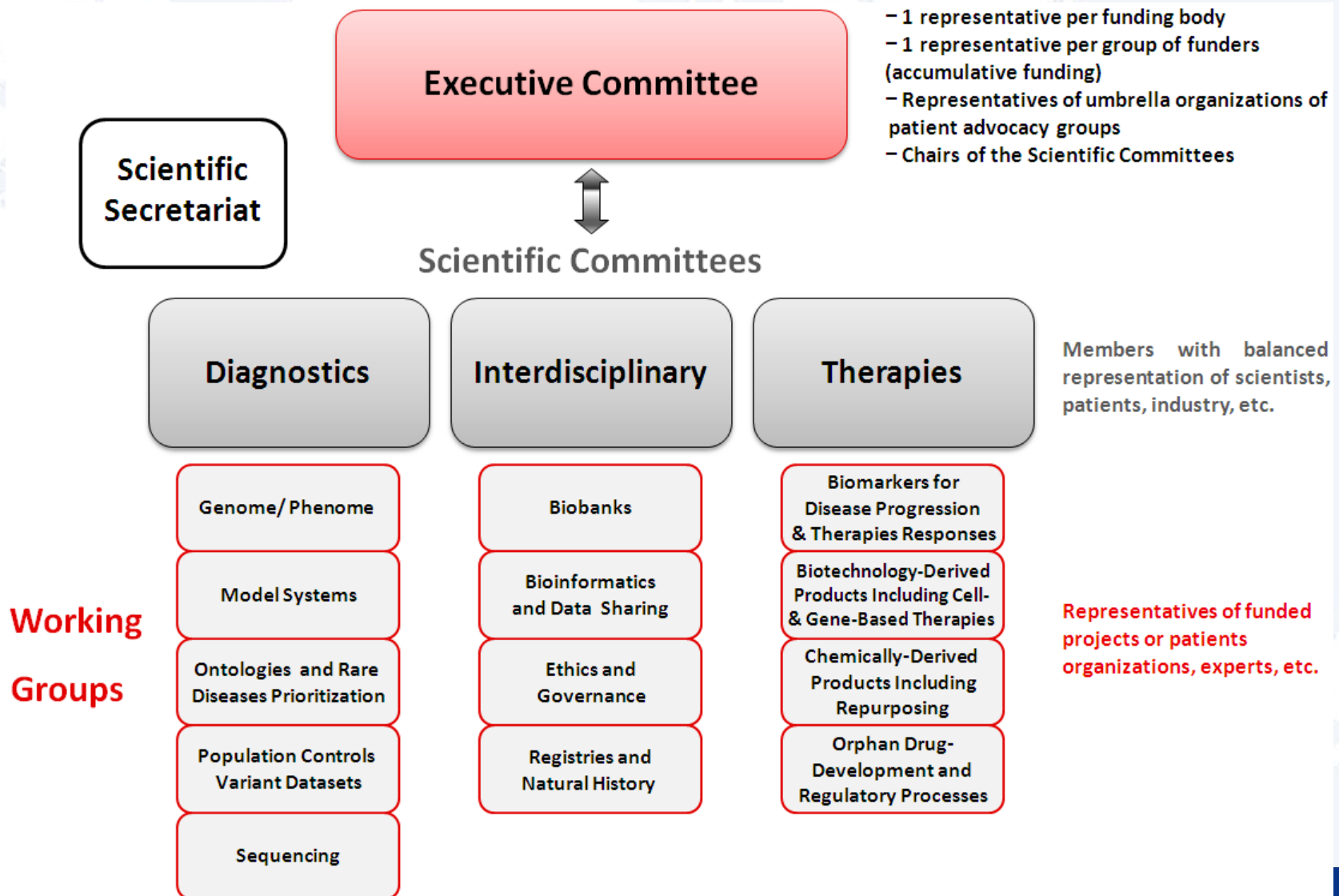
200 new therapies
for rare diseases



Means to diagnose
most rare diseases



Governance structure



Executive Committee: FUNDING AGENCIES



Paul Lasko (chair of IRDiRC)

- Canadian Institutes for Health Research
- Canada



Stephen Katz

- National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIH)
- USA



Christopher Austin

- National Center for Advancing Translational Sciences (NIH)
- USA



Nicolas Lévy

- French Foundation for Rare Diseases
- France



Pedro Cortegoso Fernández

- National Institute of Health Carlos III
- Spain



Pamela McInnes

- Office of Rare Diseases (NIH)
- USA



Hugh Dawkins

- Western Australian Department of Health
- Australia



Pierre Meulien

- Genome Canada
- Canada



Ruxandra Draghia-Akli

- European Commission
- Europe



Lucia Monaco

- Telethon Foundation
- Italy



Enrico Garaci

- Istituto Superiore de Sanita
- Italy



Katherine Needleman

- Office of Orphan Products Development (FDA)
- USA

Executive Committee: FUNDING AGENCIES



Marie-Christine Ouillade

- French Muscular Dystrophy Association
- France



Bertrand Schwartz

- Agence Nationale de la Recherche
- France



Willem Ouwehand

- National Institute for Health Research
- UK



Danilo Tagle

- National Institute of Neurological Disorders and Stroke (NIH)
- USA



Melissa Parisi

- National Institute of Child Health and Human Development (NIH)
- USA



Edward Trimble

- National Cancer Institute (NIH)
- USA



Hyun-Young Park

- Korea National Institute of Health
- South Korea



Santa Tumminia

- National Eye Institute (NIH)
- USA



Jeffery Schloss

- National Human Genome Research Institute (NIH)
- USA



Sonja van Weely

- The Netherlands Organisation for Health Research and Development
- Netherlands



Ralph Schuster

- Federal Ministry of Education and Research
- Germany



Heikki Vilen

- Academy of Finland
- Finland

Executive Committee

INDUSTRY



Karen Aiach

- Lysogene
- France



Andrea Chiesi

- Chiesi Farmaceutici S.p.A.
- Italy



Luc Dochez

- Prosensa
- Netherlands



Diane Goetz

- PTC Therapeutics
- USA



Carlo Incerti

- Genzyme
- USA



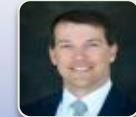
Robert Mashal

- NKT Therapeutics
- USA



Brett Monia

- Isis Pharmaceuticals
- USA



Albert Seymour

- Shire
- USA

RESEARCH & DEVELOPMENT SERVICE



Mao Mao

- WuXi AppTec Co., Ltd.
- China

Executive Committee

RESEARCH ORGANIZATIONS



Denis Lacombe

- European Organisation for Treatment & Research on Cancer (EORTC)
- Belgium



Ning Li

- Beijing Genomics Institute (BGI)
- China



David Pearce

- Sanford Research
- USA

CONSORTIUM OF RESEARCH INSTITUTES



Qing K Wang

- Chinese Rare Disease Consortium
- China

HOSPITALS



Oleg Kvlividze

- Children's New Hospitals Management Group
- Georgia

RARE DISEASE PROJECT



Sultan Turki AlSedairy

- Saudi Human Genome Project
- Kingdom of Saudi Arabia

CONSORTIUM OF FUNDING BODIES



Daria Julkowska

- E-RARE 2 Consortium
- Europe

Executive Committee

PATIENT ORGANIZATIONS



Béatrice de Montleau

- European Organisation for Rare Diseases (EURORDIS)
- Europe



Peter Saltonstall

- National Organization for Rare Disorders (NORD)
- USA



Sharon Terry

- Genetic Alliance
- USA

How to Involve More Patients in the Executive Committee

► Executive Committee

- ↪ Umbrella organizations – usually large national, regional, and international, well established bodies
- ↪ 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 are considered as full members of the Executive Committee.

How to Involve More Patients Broadly

- ▶ Serve on the various scientific committees
 - ↪ Diagnostic
 - ↪ Interdisciplinary
 - ↪ Therapeutic
- ▶ Serve on 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

Overall

- ▶ Consider the policies within your country
- ▶ Advocate for more collaboration between academia, industry and government
- ▶ Encourage sharing of precious resources
- ▶ Collaborate internationally
- ▶ Present evidence for the need whenever possible
- ▶ Celebrate diversity and commonality

To Contact IRDIRC

- ▶ <http://www.irdirc.org>
- ▶ Speak with any member: Yann Le Cam, Béatrice de Montleau, Peter Saltonstall, Sharon Terry
- ▶ Email the Secretariat: Ségolène Aymé