



RARE  
DISEASES  
INTERNATIONAL

# PROGRESS AND MEMBERSHIP OF INTERNATIONAL FEDERATIONS

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EURORDIS Council of European Federations,  
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# WHAT IS RDI?

The Global Alliance of People Living with Rare Diseases of all Nationalities across all Rare Diseases

It is an informal network that will eventually be registered and have a legal identity – a later decision by its members.

Initial focus is on taking action and gaining experience of working together.



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# WHY IS RDI NEEDED?

- ▶ To unite, expand and reinforce the RD movement of patient organisations and patient advocates
- ▶ To enable the emergence of RD as a public health & research priority internationally and to enable the emergence of a strong common voice on behalf of the +300 million people living with rare diseases (PLWRDs) around the world
- ▶ To influence international organisations (UN, WHO)
- ▶ To enable the local rare disease patient groups to act at national, regional, international levels and to interact with other areas in the field of RDs
- ▶ Most international initiatives are ad hoc and linked to a particular diseases. RDI is an opportunity to work globally and to create a global RD community

through advocacy and exchange of experience.



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# MISSION STATEMENT

► *To be a strong common voice on behalf of people living with rare diseases around the world*



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# VISION

- RDI is patient-centric, patient-driven, patient-led
- RDI recognizes patients with RDs are all linked and most are genetically related
- As the voice of all PLWRD around the world, RDI will work to give visibility to RDs in the global agenda
- As a result of RDI, RD patients worldwide and their families will experience increased public health services and support



# GENERAL OBJECTIVES

- To promote rare diseases as an international public health and research priority through public awareness and policy-making
- To represent members and people living with rare diseases at large, in international institutions and forums
- To enhance the capacities of members through information, exchange, networking, mutual support and potentially joint actions



# BACKGROUND

- EURORDIS adopt international orientation in EURORDIS Strategy 2010-2015
- EURORDIS Partnerships with NORD, CORD, JPA, RVA, RPU
- RDI preliminary at ICORD Tokyo 2012
- EURORDIS with IAPO Survey carried out in Sept 2013 to evaluate level of interest = 64 respondents from 37 countries = 98% replied that they would be interested in joining RDI
- RDI “concept” presented and tested at conferences in 2014 including NORD Regional Meeting in Alexandria VA in Nov 2014
- RDI Pre-Formation Group & 1<sup>st</sup> Business Meeting at IRDiRC Conference in Shenzhen China in Oct 2014
- EURORDIS Round Table of Companies in Brussels in Feb 2015
- Official Launch & Inaugural Meeting in Madrid in May 2015



# RDI LAUNCH EVENT MAY 28, 2015 MADRID, SPAIN



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# WHAT WILL RDI DO?



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# ADVOCACY

## ► Promote the Joint Declaration “Rare Diseases: an International Public Health Priority” – 10 recommendations

1. Enhanced Visibility
2. Patient Empowerment
3. Creation and Dissemination of Information
4. International cooperation in services to patients
5. Improve prevention, screening and timely diagnosis
6. The right to Universal Access to Healthcare
7. National and international networking of specialised expertise
8. Promotion of mobility of experts and patients to access adequate care
9. International coordination of Research
10. Shaping Policy at international level based on common values



# GAINING RECOGNITION AT THE UNITED NATIONS

- Introduce concept and foster understanding of rare diseases in international institutions (UN, ECOSOC, WHO, PAHO, OECD)
- UN Committee for Rare Diseases (through the Conference of NGOs with Consultative Status to the United Nations' Economic and Social Council)
- Rare Disease Day recognised by the WHO
  - Eg. World Hepatitis Day or
- A Report on RD presented to the WHO World Health Assembly
- A Position Paper on RD presented at the UN General Assembly
- Why not ...a UN Resolution on Rare Diseases!



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# INFORMATION & NETWORKING



- Website

[www.rarediseasesinternational.org](http://www.rarediseasesinternational.org)

to be launched very soon!

- Online Discussion Group
- RDI mailing list
- Annual RDI Conference
- Facilitate regional meetings back to back to other existing conferences
- Short-term Exchange and Internship Capacity Building Programme



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# AWARENESS



- Promotion of RD awareness worldwide through active participation in and promotion and expansion of Rare Disease Day
- Rare Disease Day 2015 Campaign + 80 countries

New participants: Madagascar, Bolivia and Bangladesh



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# RESEARCH

- Coordination with and encourage participation in the International Rare Disease Research Consortium
- Goal:
  - 200 new therapies for RDs by 2020
  - Means to diagnose most rare diseases by 2020
- IRDiRC 3 patients reps at ExeCo
- Therapies Scientific Committee: Yann Le Cam, Elected Chair & 1 EURORDIS staff
- Need to broaden patient participation and make it more representative of the world

# BUILDING BRIDGES WITH OTHER STAKEHOLDERS: SHAPING RD ECO-SYSTEM



- International Alliance of Patients' Organizations (IAPO)
- International Conference on Rare Diseases & Orphan Drugs (ICORD)
- International Federation of Pharmaceutical Manufacturers & Associations (IFPMA)
- International Rare Diseases Research Consortium (IRDiRC) + patient involvement
- International Society of Human Genetics (ISHG)
- Orphanet International (10 countries ex Europe)

# GOVERNANCE

The preliminary phase of the initiative has been steered by a **Pre-Formation Group**

1. Europe (EURORDIS)
2. USA (NORD)
3. Canada (CORD)
4. Japan (JPA)
5. China (CORD)
6. India (I-ORD)
7. France (AMR)
8. Ibero-American pan-regional alliance (ALIBER)
9. DEBRA International
10. IPOPI



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# 28 MEMBERS SO FAR

1. Alliance Maladies Rares (France)
2. ALIBER-Alianza Iberoamericana de Enfermedades Raras
3. Arabic Organisation for RareDiseases (Associate)
4. Associacao Brasileira de Enfermedades Raras
5. Canadian Organization for Rare Disorders
6. Chinese Organization for Rare Disorders
7. Cyprus Alliance for Rare Disorders
8. Debra International (Epidermolysis Bullosa)
9. EURORDIS
10. Federación Argentina de Enfermedades Poco Frecuentes
11. Federación Espanola de Enfermedades Raras (Spain)
12. Federacion Mexicana de Enfermedades Raras
13. Findacure (Associate)
14. Indian Organization for Rare Diseases



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# 28 MEMBERS SO FAR

15. IPOPI – International Patient Organization for Primary Immunodeficiencies

16. Japan Patient Association

17. Malaysian Rare Disorders Society

18. Naevus Global (large congenital melanocytic naevi)

19. New Zealand Organization for Rare Disorders

20. National Organization for Rare Disorders USA

21. Pro Rare Austria

22. Rare Diseases South Africa

23. Rare Voices Australia

24. Retina International (Retinitis Pigmentosa and allied retinal dystrophies)

25. Hongkong Alliance for Rare Disorders

26. Genetic Alliance Australia

27. Pulmonary Hypertension Latin Society

28. Blackswan Foundation (Associate)



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# WHO CAN BE A MEMBER?

- Umbrella organisations representing several rare diseases in one country
  - National Alliances
- Umbrella organisations representing the same disease or family of diseases in several countries
  - International Federations
- Umbrella organisations representing national alliances or other patient groups in several countries across a geographical region



# MEMBERSHIP CRITERIA

- Rare disease organisation, according to EU prevalence criteria (5 / 10 000)
- The Governing Boards should be usually made up of a majority of rare disease patients or family of patients
- Financial transparency and diversified funding showing independence and minimisation of risk of conflict of interest
- Non-profit status
- Proven activities such as patient support and/or advocacy activities and/or research

Applications reviewed on a case by case basis

Waivers possible



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# ASSOCIATE MEMBERSHIP

- ▶ Patient organisations or any other entity contributing to the objectives of RDI by their mission and work.
- ▶ Associate members cannot be elected to or vote at the Council of RDI
- ▶ To apply, go to:

<http://www.eurordis.org/content/rare-diseases-international#membershipapp>



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# WHAT'S IN IT FOR FEDERATIONS?

- Only 4 International Federations so far
- We need more Federations!
- Strength in numbers. More members. Stronger voice. More power.
- We need to build critical mass to count and be heard
- Federations are already international and have a great deal to contribute from their experience
- There is no initiative that links RD disease specific networks on common RD issues
- Win win . More attention to RDs = More attention to your disease



# BENEFITS FOR YOUR MEMBERS OUTSIDE OF EUROPE

- RDI presents opportunity for POs to **leverage international voice** to raise awareness, support, and action locally
- RDI provides opportunity for patient networks to **collaborate on regional issues** and insure RDs included in health and economic policies, e.g., UHC
- RDI can **highlight RD challenges endemic to LMIC's** and encourage international attention to address them
- RDI provides opportunity for RD patients in countries emerging to rare diseases to **get support from countries that are further down the line**
- RDI provides opportunity for RD patients in regions where RD not yet meaningfully recognised to **feel empowered and connected**



# HOPE TO SEE YOU AT ECRD 2016!

- ▶ RDI satellite meeting and elections of RDI Council



- ▶ 8th European Conference on Rare Diseases & Orphan Products - May 2016, Edinburgh, Scotland



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