



RARE  
DISEASES  
INTERNATIONAL

## ACTION PLAN 2018

### 1. BUILDING A LEGITIMATE RARE DISEASE PATIENT GLOBAL ALLIANCE

#### Membership

Expand membership base

50 Member Organisations at present

Recruit more members in 2018

Target

~ 10 National Alliances

~ 10 International Federations

RDI 4<sup>th</sup> Annual Meeting back to back to European Conference on Rare Diseases & Orphan Products (ECRD Vienna 2018) on May 10

Information stand - ECRD May 11

Regular e-mailings

Webinars on specific topics

Set up virtual forum for members to share and exchange information – *Facebook Workplace*

#### Partnerships

Pursue strategic partnerships with:

- Orphanet
- International Rare Diseases Research Consortium - Patient Advocates Consitutory Committee
- International Federation of Pharmaceutical Manufacturers & Associations (IFPMA) Working Group on Rare Diseases

No renewal of Memorandum of Understanding with ICORD

No renewal of Memorandum of Understanding with BLACKSWAN FOUNDATION

### 2. ADOULATE FOR RARE DISEASES TO BE AN INTERNATIONAL PUBLIC HEALTH PRIORITY & RAISE AWARENESS

Place Rare Diseases in UN Sustainable Development Goals 2030 Agenda under the guiding principle “to leave no one behind” with the relevant political institutions and UN bodies



- Economic and Social Council (ECOSOC)
- Human Rights Council
- Conference of State Parties to the Convention on the Rights of Persons with Disabilities
- World Health Organization
- UN General Assembly, Third Committee (Social, Cultural and Humanitarian Issues)

Develop relations in each of these institutions, in particular with:

UN Special Rapporteurs for Health and Disability

Directors and technical staff at WHO, ECOSOC's Department of Economic and Social Affairs and others

Produce Position Papers, Contributions, Statements

Organise Side Events

Advocate Permanent Missions to UN in Geneva and New York

Mobilise National Alliances to obtain political support from counterparts at the Ministry of Health, Ministry of Foreign Affairs or relevant Ministries in their country

#### RDI Advocacy committee

Composed of 16 patient reps from Member Organisations

Draft Policy on developing, recommending and adopting Advocacy Initiatives

Adopt and submit first Position Papers, Contributions, Statements

Organise Ad Hoc Groups to work on specific topics

- Human Rights
- Disability
- Others to be determined

#### NGO Committee for rare diseases

The NGO CfRD is a Substantive Committee of the Conference of NGOs in Consultative Status with the United Nations (CoNGO) formed to promote multi-stakeholder collaboration and actions for rare diseases within the United Nations system



## RARE DISEASES INTERNATIONAL

Inception Executive Board: EURORDIS, Agrenska, International Alliance of Patient Organisations, International Federation for Spina Bifida and Hydrocephalus, World Federation of Hemophilia, International Women's Federation

RDI to be a member of the Committee and to represent patients in the Committee

RDI and NGO CfRD co-signed Positions

Joint outreach to Permanent Missions to the UN in New York and Geneva

NGO CfRD High Level Meeting in UN Headquarters, New York – Rare Disease Day 2019

### World Health organization

Proposal towards a collaborative framework between RDI and WHO

Application to organise Side events at WHO 71<sup>st</sup> World Health Assembly

Submission of Statement on Rare Diseases at WHO 71<sup>st</sup> World Health Assembly

Develop relations with:

Staff at the Division of Universal Health Coverage and Health Systems

Staff at the Division on Access to Medicines, Medical devices and Assistive technology

Health attaches at the Permanent Missions to the UN in Geneva

### Actions in UN system

Application for NGO consultative status with ECOSOC (via EURORDIS)

Contribution to the Report of the Office of the High Commissioner for Human Rights (OHCHR): *The Right to Health framework to implement health-related SDGs* → to be presented at Human Rights Council 38th session (June 2018) Geneva

*Contribution to the Report of the UN Special Rapporteur on the rights of people with disabilities: The Right of persons with disabilities to the highest attainable standard of health:* → to be presented at 73<sup>rd</sup> session UN General Assembly Third Committee (Oct 2018) New York

Active participation in NGO Committee for Rare Diseases High Level Meeting – Rare Disease Day 2019

Prevention and Control of Non-Communicable Diseases 3<sup>rd</sup> High Level Meeting (September 2018) New York (tentative)

### Towards an evidence-based advocacy

Orphanet: Agree on global rare disease figures to communicate widely



State of the Art of Rare Diseases: Develop a survey based on and using the tool of, the '*State of the Art of Rare Disease Activities in Europe*' to collect and benchmark policy information from countries outside the EU

Rare Barometer Voices: Extend the survey to collect data on specific topics from countries outside Europe

### 3. ADVOCATE FOR PEOPLE LIVING WITH RARE DISEASES IN KEY AREAS

#### Access to medicines

Include Low and Middle-Income countries in actions to improve access to medicines for rare diseases

Include access to medicines for rare diseases in Proposal of Collaborative Framework with WHO

Mobilise political support from Member States through Permanent Representations in Geneva to include access to medicines for rare diseases in WHO agenda

Organise a Roundtable of Innovative Access Programs with IFPMA, WHO, HTAi and other global actors

#### Research

RDI to become member of the International Rare Disease Research Consortium (IRDIRC)

RDI representative to sit at IRDIRC Patient Advocates Constituency Committee (PACC) : composed of US Genetic Alliance, EURORDIS, NORD, CORD, China ORD, RVA, JPA , I-ORD (7/8 are members of RDI)

Contribute to IRDIRC's policy work bringing patient advocate's perspective (eg. Survey on patient engagement in research)

Guide and support RDI patient representatives in IRDiRC's governing and operational bodies (Assembly, Patient Constituency Group, Scientific Committees and Task Forces)

#### Diagnostic

Tie into the *Global Commission to End the Diagnostic Odyssey of Children with Rare Diseases*, a multi-disciplinary expert group (co-Chaired by Shire, Microsoft and EURORDIS – Rare Diseases Europe) to produce a roadmap with solutions to barriers to timely diagnosis for all rare diseases with an emphasis on those affecting children.

RDI Council Members sitting in the Commission (Yann Le Cam and Durhane Wong-Rieger) to secure RDI representation



Involve RDI Members through Topic Groups and Consultations with Members on Global Commission's Recommendations to be published early 2019

Participate in determination of the Recommendations and/or use a basis to develop RDI position

#### 4. BUILD CAPACITIES OF MEMBERS & PATIENT ADVOCATES:

Foster knowledge exchange and training through:

- RDI Conference Programme
- Fellowships

#### 5. STRENGTHEN RDI AS A WELL-GOVERNED AND SUSTAINABLE ORGANISATION

*Set up a solid internal governance structure*

##### A. Legal incorporation

Establish RDI as a fully-fledged NGO with moral and legal personality and financial responsibility

Adoption of Statutes by Members

Submission of Statutes to French authorities

##### B. Governing Council

Annual Council Elections and regular Council Meetings

Revision of By-Laws after adoption of Statutes

Annual Activity & Financial Report 2017

Annual Work Programme: Action Plan & Budget 2018

Develop policy of declaration of potential Conflict of Interest

##### C. Financial sustainability

Memorandum of Understanding signed with EURORDIS - 5 years (2019-2023)

EURORDIS will continue to provide professional services and will continue to secure corporate support

EURORDIS financial and in-kind contributions will be included in RDI Financial accounts