



Rare Diseases International 2017 Activity Report

RDI is a global alliance of people living with a rare disease of all nationalities across all rare diseases. RDI brings together rare disease patient organisations from around the world as well as international rare disease-specific federations to create the global alliance of rare disease patients and families.

RDI was launched in 2015 to create a strong common voice on behalf of all people living with a rare disease around the world, to advocate for rare diseases as an international public health priority, to represent its members, and to enhance their capacities.

Ultimately, RDI will help:

- Unite, expand and reinforce the movement of people living with a rare disease to speak with one strong voice.
- Establish rare diseases as a public health priority in more countries and regions around the world as well as at the global level.
- Put rare diseases on the agenda of United Nations and other international organisations.
- Strengthen rare disease patient groups' capacity to act at local, national, regional and global levels.

Progress in 2017

A growing global movement of people living with a rare disease

Governance

RDI's Council is elected by full members and ensures a credible and effective governance of RDI. It also advises on the programme's long term strategy and actions. In April 2017 all full members of RDI had the opportunity to vote on-line to replace the following members of the Council:

John Dart – DEBRA International (retired)

Ramaiah Muthyala – Indian Organization for Rare Diseases (end of mandate)

Alfredo Toledo – Iberoamerican Alliance for Rare Diseases (end of mandate)

Ritu Jain from DEBRA International and Kin Ping Tsang from Retina International were newly-elected and Alfredo Toledo was re-elected for another 3-year mandate. A total of 12 nominations were received and 29 out of 36 full members voted.

As of June 2017, the Council included the following 7 prominent rare disease patient representatives:



Name of representative	Organisation	Year of Election	End of mandate
elected till 2020			
Kin Ping Tsang	Retina International	2017	2020
Alfredo Toledo	Ibero American Rare Disease Alliance	2017	2020
elected till 2019			
Durhane Wong-Rieger	Canadian Organization for Rare Disorders	2016	2019
Yann Le Cam	EURORDIS - Rare Diseases Europe	2016	2019
elected till 2018			
Megan Fookes	Rare Voices Australia (resigned in September 2017)	2016	2018
Lisa Phelps	National Organization for Rare Disorders	2016	2018
Ritu Jain	Debra International	2017	2018

A growing membership base

An engaged membership base is key to establish an effective global alliance of people living with a rare disease. From starting out with 20 member organisations in 2015, RDI had 49 member organisations at the end of 2017. Full member umbrella organisations represent patient groups from 27 national alliances, 4 pan regional networks and 11 international disease-specific federations. Through RDI members, rare disease patients are represented in more than 100 countries worldwide.

The following rare disease patient umbrella organisations were the last to join RDI in 2017:

- World Alliance of Pituitary Organisation Association
- Nordic hypoPARA Organisation
- Cutis Laxa International
- Organisation for Rare Diseases India

The list of RDI members is continually updated and published on rarediseasesinternational.org.



Establishing an international dialogue to make rare diseases a public health priority

RDI's Advocacy Committee

The Advocacy Committee was created at the end of September 2017. Yann Le Cam, Chief Executive Officer of EURORDIS is the Chair of the Committee. 15 patient representatives from RDI's member organisations, plus a representative from the International Alliance of Patient Organisations, take part in this Committee and represent a wide range of countries and diseases:

- EURORDIS – Rare Diseases Europe
- National Organization for Rare Disorders (United States)
- Canadian Organization for Rare Disorders
- Federación Colombiana de Enfermedades Raras (Colombia)
- Pulmonary Hypertension Latin Society (Latin America and the Caribbean)
- Indian Organization for Rare Diseases
- BLACKSWAN Foundation (Switzerland)
- DEBRA International
- Ehlers-Danlos Society
- Rare Diseases Croatia
- Rare Diseases South Africa
- World Federation of Hemophilia
- International Federation for Spina Bifida & Hydrocephalus
- Federación Española de Enfermedades Raras (Spain)

and the International Alliance of Patient Organisations – IAPO



Yann Le Cam
EURORDIS
Chair



Maureen Smith
CORD-Canada



Angela Chaves
FECOER-
Colombia



Kelly du Plessis
RDSA-South Africa



Ramaiah
Muthyala
IORD-India



Paul Melmeyer
NORD



Ritu Jain
DEBRA
International



Lara Bloom
EDS



Sanja Peric
RD-Croatia



Migdalia Denis
PHLS



Lieven Bauwens
IFSBH



Alba Ancochea
FEDER-Spain



Mark Brooker
WFH



Chiara Ciriminna
BLACKSWAN
FOUNDATION



Kawaldip
Sehmi
IAPO



Simone Boselli
EURORDIS
Europe



The aim of this Committee is to speak with one voice to address the issue of rare diseases at a political and international levels, in line with RDI's Joint Declaration - *Making Rare Diseases an International Public Health Priority*. The Committee is responsible for:

- Developing and recommending priorities, initiatives and policies for RDI
- Preparing position papers, statements or declarations
- Reviewing any position, statement, declaration or petition considered for endorsement by RDI

In the last quarter of 2017, the Advocacy Committee met twice in October and November. As part of its work, the Committee has drafted and recommended for adoption to the Council the 'RDI Policy on developing, recommending and adopting Advocacy Initiatives'.

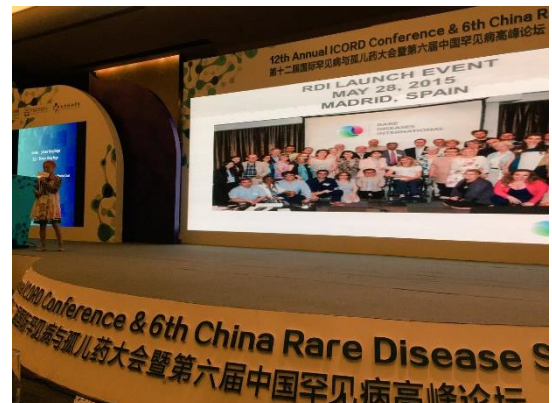
The first milestone of the Advocacy Committee has been to respond to the Open Consultation on the 13th General Work Programme (GWP 13) of the World Health Organization, which will cover the period 2019-2023 and serve as the organising framework for two Programme Budgets 2020-2021 and 2022-2023, as well as the strategic basis for resource mobilization. RDI submitted contributions in the two rounds of consultations, in October and November 2017.

Collaboration with the International Conference on Rare Diseases & Orphan Drugs (ICORD)

As part of the Memorandum of Understanding with ICORD, RDI's collaboration with ICORD focuses on supporting the organisation of ICORD's annual conference to enhance effective communication and to exchange best practices in order to address common issues in rare diseases and orphan products.

RDI is included in the Programme Committee of the ICORD Conference so that sessions on themes that are stated priorities of RDI will be included in the programme.

RDI's chair, Durhane Wong Rieger was an active member of the Programme Committee and ensured a stronger patient dimension all along the Programme. She spoke on behalf of RDI at the XII ICORD Conference and 6th China Rare Diseases Summit in Beijing, China, in September 7-10, 2017.



Progress in discussions towards placing rare diseases on the WHO's agenda

Statement from the Director – General of the WHO

In the weeks leading to Rare Disease Day 2017, RDI received a statement of support from Dr. Tedros Adhanom Ghebreyesus at the time candidate to the post of Director- General of the WHO. He was elected in this position in May 2017.

"Rare diseases represent a major challenge in public health that has been largely ignored. Consequently, this is a field in public health and research that would certainly benefit from globally concerted action and international collaboration".

Dr Tedros Adhanom Ghebreyesus, Director-General of the WHO



“The Right to Health: The Rare Disease Perspective” RDI policy event, Geneva, February 2017

To mark the occasion of Rare Disease Day on February 10, 2017, RDI held the first international rare disease policy event. This was an event in partnership and with support from the BLACKSWAN Foundation and EURORDIS, and with the participation of IRDiRC, the NGO Committee for Rare Diseases, and Orphanet.

The event was the first of its kind to be organised in Geneva, at the Campus Biotech, and gathered 20 international speakers and panellists, all experts in the fields of public health, human rights, scientific research and patient advocacy and the health industry. Discussions revolved around why and how rare diseases should be included in the global health agenda.



140 participants explored ways to address inequality and improve access to health for the people living with a rare disease around the world.

In addition to a high-level official of the Department of Essential Medicines and Health Products at the World Health Organization, the distinguished line-up of speakers included the United Nations Special Rapporteur on the Right to Health, the co-Chair of the United Nations Secretary General’s High-Level Panel on Access to Medicines, the Director of the United Nations Development Program (UNDP) and a Senior Health Policy Analyst from the Organisation for Economic Co-operation and Development (OECD).

Key messages

- A human rights-based approach to rare diseases is relevant to the United Nations Sustainable Development Goals of the 2030 Agenda «Leave no one behind» and needs to be included in strategies to achieve Universal Health Coverage.
- Research and access are interdependent and need to be tackled as a continuum.
- Research and access to expensive medicines need to be balanced with the care and support needed for chronic and disabling diseases.



The importance of holding international meetings was emphasised as a means to:

- Help focus attention on rare diseases as a global health priority.
- Give opportunities for patient groups to share rare diseases trends and challenges in their country or region, and to give weight to their advocacy efforts.
- Exemplify the importance of joining top-bottom approaches from high-ranked international organisations with grassroots approaches.

The conference was streamed across social media.



The NGO Committee for Rare Diseases

The NGO Committee for Rare Diseases (United Nations, New York) is a substantive committee established under the umbrella of the Conference of NGOs in Consultative Relationship with the United Nations (CoNGO), since November 2016. The main objective of the NGO Committee for Rare Diseases is to bring visibility and understanding about rare diseases to the United Nations and align rare diseases with the UN 2030 Agenda: the Sustainable Development Goals.

At the end of 2017, RDI was officially accepted as a Member by the Inception Executive Board of the NGO Committee for Rare Diseases. The members of the Executive Board are: EURORDIS, Agrenska, World Federation of Hemophilia, International Federation for Spina Bifida and Hydrocephalus, International Alliance of Patients' Organisations and the International Alliance of Women.

The Inception Executive Board will have an important role in shaping the Work Plan of the Committee and guiding the direction that it should take in the future. RDI's Chair, Durhane Wong-Rieger was nominated to be the patient representative at the NGO Committee for Rare Diseases.

Capacity building for global patient advocates (Barcelona, June 2017)

RDI's third annual meeting

RDI's achievements at the international level were presented to all members attending RDI's third annual meeting held in Barcelona, in June 2017.

Over 50 participants from 23 countries were able to network with patient advocates from around the world, learn more about recent developments in international rare disease advocacy and receive information to become further involved in RDI's activities.

Participants also discussed ways in which they could contribute to RDI's efforts to put rare diseases in the global health and development agenda through their Ministries of Health and Foreign Affairs or through their Permanent Representations to the UN in New York or Geneva.



The event was an opportunity to further members' understanding of the UN system and the synergies between rare diseases and the Sustainable Development Goals 2030 Agenda with its mission to 'leave no one behind'. Information about the different types of UN Resolutions and strategies to obtain a UN Resolution on Rare Diseases were also included in the discussions.



Patient leaders from 12 different countries had a chance to give an overview of rare disease policy, trends and challenges in their region of the world.



Express 2017 Expert Patient and Researcher EURORDIS Summer School

15 RDI fellows from India, Malaysia, South Africa, Argentina, Brazil, Colombia, Mexico, Venezuela and Uruguay, who attended RDI's third annual meeting, stayed on for another week to attend the EURORDIS Summer School.

The training aims to provide participants with the knowledge and skills needed to become experts in medicines research and development. provide the training each year.

Topics covered include: clinical trials methodology, clinical research, ethics in medicines development, regulatory affairs, health technology assessment and marketing authorisation.



The 2017 Summer School brought together a record number of 60 students from 25 countries. Sessions in English were run in parallel to a first-ever Spanish edition.

As well as welcoming patient advocates from new disease areas and new regions of the globe, the 2017 Summer School also brought new partnerships including Rare Diseases International, the Canadian Institutes for Health Research and the University of Leiden Medical Centre.