

Rare Diseases International 2018 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.

The objectives of RDI are to:

- 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 the United Nations and other international organisations.
- Strengthen rare disease patient groups' capacity to act at local, national, regional and global levels.

Progress in 2018

A growing global movement of people living with a rare disease

Legal Incorporation

In 2018 RDI was established as a fully-fledged NGO with a legal personality and financial responsibility. The registration process was completed in November 2018. RDI is legally incorporated in France under French law.

Members present at the 4th Annual RDI Meeting in Vienna adopted the Statutes of the new organisation and voted to become incorporated as a legally independent organisation.

RDI and EURORDIS signed a Memorandum of Understanding that details EURORDIS' continuous support for 5 years (2019-2023)



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, all full members of RDI had the opportunity to vote online to replace the following outgoing members of the Council:

Megan Fookes - Rare Voices Australia (stepped down in September 2017)

Lisa (Phelps) Sarfaty - NORD

Ritu Jain - Debra International

Name of representative	Organisation	Year of Election	End of mandate
KP Tsang	Retina International	2017	2020
Alfredo Toledo	ALIBER - Iberoamerican RD Alliance	2017	2020
Durhane Wong-Rieger	Canadian Organization for Rare Disorder	2016	2019
Yann Le Cam	EURORDIS - Rare Diseases Europe	2016	2019
Angela Chaves	FECOER Colombia	2018	2021
Lisa (Phelps) Sarfaty	NORD	2018	2021
Ritu Jain	Debra International	2018	2021

Angela Chaves of Federación Colombiana de Enfermedades Raras (10 votes) was newly-elected and Ritu Jain of DEBRA International (10 votes) and Lisa (Phelps) Sarfaty of National Organization for Rare Disorders (13 votes) were re-elected for a 3-year mandate. A total of 10 nominations were received and 25 full members voted.

Angela Chaves stepped down from the Council in July and Alfredo Toledo in September. The Council invited two patient representatives to fill in temporarily those positions until the next General Assembly in February 2019. The patient representatives invited by the Council were: Rachel Yang from Chinese Organization for Rare Disorders and Jesús Navarro for the Alianza Iberoamericana de Enfermedades Raras.



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 57 member organisations at the end of 2018. Member umbrella organisations represent patient groups from 34 national alliances, 5 pan regional networks and 14 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 joined RDI in 2018:

1. Asociación de Familiares y Afectados de Lipodistrofias
2. International Gaucher Alliance
3. Genetic Alliance South Africa
4. MCT8- AHDS Foundation
5. National Organisation for Rare Diseases of Serbia
6. Rare Diseases Sweden
7. Philippines Society for Rare Disorders
8. Thalassaemia Foundation International

The list of RDI members is continually updated and published online at:
rarediseasesinternational.org/members

Building a community of global patient advocates



RDI's 4th annual meeting, Vienna, Austria

RDI's Annual Meeting took place on May 10th in Vienna, Austria, back to back to the European Conference on Rare Diseases and Orphan Medicinal Products (ECRD 2018). Delegates from 33 member organisations attended the General Assembly.

One third of the member delegates came from outside Europe: from Argentina, Brazil, Colombia, USA, Canada and all the way from India, Iran, China, Japan, Singapore, Hong Kong and South Africa.

The General Assembly and Membership Meeting was followed by an open session that attracted more than 100 participants from all stakeholder groups to discuss tools, means and actions to advocate for rare diseases in the United Nations system.



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

RDI's Advocacy Committee

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.



Yann Le Cam
EURORDIS
Chair



Maureen Smith
CORD-Canada



Angela Chaves
FECOER-
Colombia



Kelly du Plessis
RDSA-South Africa



IORD-India



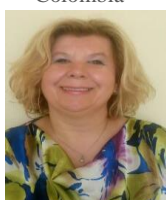
Paul Melmeyer
NORD



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

Progress in discussions to place rare diseases on the WHO's agenda

Statement from the Director – General of the WHO

In February, the WHO issued a Statement on Rare Disease Day 2018 from Director-General Dr. Tedros Adhanom Ghebreyesus

"We are working for a world where no one is left behind. On Rare Disease Day, we welcome further discussions with the rare diseases community on how we can strengthen cooperation to ensure people with rare diseases can access the health services they need."

Dr. Tedros Adhanom Ghebreyesus, Director-General of the WHO

In 2018, RDI's Advocacy Committee met twice to discuss and give feedback on a Proposal of Collaborative Framework with the WHO.

The Collaborative Framework was discussed with high-level officials at the WHO Secretariat in Geneva, throughout 2018 in view of formalising collaboration with WHO on specific activities.

As part of the ongoing discussions, Dr. Rudiger Krech, Director of Universal Health Coverage and Health Systems accepted to deliver a key note inspirational speech at ECRD 2018 Vienna.



Advocacy actions in the United Nations system

The NGO Committee for Rare Diseases

The NGO Committee for Rare Diseases (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 Sustainable Development Agenda.

RDI is a Member of 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. RDI's Chair, Durhane Wong-Rieger was nominated to be RDI's representative at the NGO Committee for Rare Diseases.



RARE
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Lieven Bauwens,
IF Secretary General



Yann Le Cam,
EURORDIS CEO



Anders
Olauson,
Chairman of
Agrenska



Kawaldip Sehmi,
IAPO CEO



Alain Weill,
WFH President



Durhane Wong-Rieger
Chair of the RDI Council



Soon-Young Yoon,
UN main representative for
IAW in New York.

In 2018 the NGO Committee for Rare Diseases submitted the following official written Contributions:

'The Right to Health in Rare Diseases' 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* → presented by the Deputy High Commissioner for Human Rights Kate Gilmore 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: → presented by Special Rapporteur Catalina Devandas at 73rd session UN General Assembly Third Committee (Oct 2018) New York.



Including Rare Diseases in the Human Rights & Disability Agendas

On 15 and 16 May, RDI Director Paloma Tejada participated in an Expert Group Meeting convened by the Special Rapporteur on the right of persons with disabilities to the highest attainable standard of physical and mental health. The meeting took place at the UN Office in Geneva and served to consult with disability organisations on the content of the Report.



Rare Diseases were mentioned in the Report at the UN General Assembly in September.

“Health systems must respond to the needs of the diversity of persons with disabilities. [...]. States should consider developing and implementing policies and practices targeting the most marginalized groups of persons with disabilities (e.g., persons with multiple or severe impairments, rare diseases or deaf-blindness) in order to accelerate or achieve de facto equality in access to health care.”



As NGOs with ECOSOC Consultative Status, Thalassaemia International Federation, the Agrenska Foundation, and the International Federation for Hydrocephalus and Spina Bifida – IF, submitted a **Official Written Statement on Rare Diseases** to the 38th Session of the Human Rights Council (18 June – 6 July, Geneva) endorsing the messages laid out in the NGO Committee for Rare Diseases publication 'The 'Right to Health' in Rare Diseases'.

An Official Oral Statement on Rare Diseases and Disability was delivered by Clara Hervas (EURORDIS) on behalf of NGO Committee for Rare Diseases at the **Conference of States parties to Convention on the Rights of Persons with disability in New York – 12 to 14 June 2018**



Rare Diseases mentioned for the first time at World Health Assembly

In May, RDI Director Paloma Tejada delivered an Oral statement at the 71st World Health Assembly of the World Health Organisation (WHO) in Geneva. The statement is the product of a joint collaboration between a number of organisations holding the status of 'special relations with the WHO' (Thalassaemia International Federation, World Federation of Hemophilia, International Alliance of Patients' Organizations and March of Dimes), RDI and the other members of the NGO Committee for Rare Diseases.

The statement is anchored to agenda item '11.7 Preparation for the third High-level Meeting of the General Assembly on the Prevention and Control of Non-communicable Diseases' which was held in September 2018. It calls on Member States to “not leave behind significant but often neglected rare diseases, each of which affect relatively small numbers of patients but collectively affect at least 300 million people globally”.



Rare Diseases & Non Communicable Diseases Agenda

- RDI made a submission to the web-based consultation of the WHO Independent High-level Commission on NCDs on May 16, 2018
- Durhane Wong-Rieger and Lisa Sarfaty, Chair and Secretary of RDI, participated in a UN NCD Civil Society Hearing in New York on July 5th

Missions to NYC to meet Permanent Missions and relevant UN agencies in May, June and October 2018

Yann Le Cam, Durhane Wong-Rieger of RDI Council; Clara Hervas and Anders Olauson of the NGO Committee for Rare Diseases, and patient advocates of RDI member organisations: Lieven Bauwens of International Federation for Spina Bifida and Hydrocephalus and Ramaiah Muthyala of Indian Organization for Rare Diseases (representing RDI and NGO Committee for Rare Diseases) met with UN representatives, NGOs in consultative status with UN and Permanent Missions to:

- Consider rare diseases in Political Declaration on NCDs at UN GA High Level Meeting, September 2018. A preambular paragraph with suggested language was sent in the aftermath to the Permanent Missions of Brazil, Colombia and India
- Include rare diseases in WHO Universal Health Care Agenda
- Co-host Rare Disease Day event at UN Headquarters in New York, Feb 2019

In total, RDI and NGO Committee representatives met with diplomats of the Permanent Missions to the UN of 26 countries.

Starting to make the case to include Rare Diseases in Universal Health Care

On December 12th - UHC Day - RDI was invited to participate in a Discussion Panel organised by WHO and Group of Friends of UHC (Japan, Brazil, France, Ghana, Hungary, South Africa, Thailand) at United Nations in New York.

Durhane Wong-Rieger speaking on behalf of RDI was the only NGO representative in the meeting and delivered a message on the importance of including rare diseases in Universal Health Coverage.



Asia-Pacific Economic Cooperation

In November 2018, RDI was invited by the Asia-Pacific Economic Cooperation (APEC) Life Sciences Innovation Forum (LSIF) Rare Disease Network to the launch of a Rare Disease Action Plan that calls for APEC's 21-member economies to improve the economic and social inclusion of individuals living





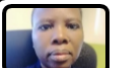








with rare diseases, with clear targets by 2025. The Action Plan was adopted during the APEC CEO Summit 2018 in Papua New Guinea, November 15th to 17th.

The APEC LSIF Rare Disease Network comprises Rare Disease International, Queensland University of Technology and Shire (representing as industry co-chair). The Experts Group aims to define, diagnose and support rare disease patients through innovation, education, building capacity and aligning policies across the 21 APEC member economies.

Patient involvement in Research Policy

RDI was accepted as a member organisation of the International Rare Disease Research Consortium (IRDIRC) in March 2018.

Ritu Jain of DEBRA International was nominated as RDI patient representative in IRDiRC's governing bodies and committees.

	Sharon Terry (Chair) Genetic Alliance, USA		Ramaiah Muthyala I-ORD, India
	Yukiko Nishimura (Vice-Chair) AsRid, Japan		Peter Saltonstall NORD, USA
	Eda Selebasto BORDIS, Botswana		Prasanna Kumar Shirol ORD-I, India
	Durhane Wong-Rieger CORD, Canada		Ritu Jain Rare Diseases International, Singapore
	Kevin Huang CORD, China		Kelly du Plessis RDSA, South Africa
	Virginie Bros-Facer EURORDIS		Nicole Millis Rare Voices Australia, Australia
	Nicole Boice Global Genes, USA		

RDI is represented in the Patient Advocates Constituency Committee. The PACC is composed of 13 umbrella patient groups of which 11 are member organisations of RDI. The Group aims to contribute to IRDiRC's policy work bringing patient advocate's perspective on research.

In 2018 Ritu Jain participated in a number of teleconferences and two face to face meetings of IRDiRC PACC in Vienna in May and in Brussels in December.

Consulting and engaging with our members

Webinars series

In 2018 RDI organised the following Webinars to engage with members:

April 23/ Legal incorporation of RDI and overall Action Plan for 2018

Webinar to go over the Statutes of the new organisation and the details of the agreement with EURORDIS for continued support. This was also the opportunity to discuss with Members, RDI's 2018 Action Plan in view to its adoption at the Membership Meeting in Vienna.

October 19/ Global Commission to End the Diagnostic Odyssey for Children with Rare Diseases

Webinar to provide an introduction to RDI members on the Global Commission to End the Diagnostic Odyssey for Children. This was an opportunity to collect feedback from members as well as review a set of recommendations that the Global Commission is due to release in their report due in February 2019.

October 5/ WHO Collaborative Framework

Webinar to present, discuss and collect input from members of the Advocacy Committee on RDI's proposal of collaboration with the World Health Organization.

November 30 / Towards a United Nations Resolution

Webinar to discuss RDI's strategy towards a United Nations General Assembly Resolution on Rare Diseases, in view of a Global Call to Action to governments at the Rare Disease Day at the United Nations event in February 2019. This was an opportunity to learn how and why a United Nations General Assembly Resolution could be useful to guide National policies favourable to patients.

December 14/ RDI's 2019 Work Programme: Action Plan and Budget

Webinar to present and discuss the activities planned for 2019.

Conference Programme

In the second half of 2018, RDI launched its Conference Programme with Level-1 support (media and visibility) to the following conferences :

RARE X Conference, Johannesburg, South Africa, September 15 -16

NORD Summit, Washington DC, October 15-16

APARDO Policy Forum & Workshop, Singapore, October 27-28

World Orphan Drug Congress, Barcelona, Spain, November 6-8

CARE 18 "un diagnostic pour tous", Paris, France, November 12

V Encuentro Iberoamericano de Enfermedades Raras (ALIBER 5th Conference) Bogota, Colombia, November 20-22



Members

Alliance Maladies Rares
Allianz Chronischer Seltener Erkrankungen
Arabic Organisation for Rare Diseases
Asia Pacific Alliance of Rare Disease Organisations
Associacao Brasileira de Enfermedades Raras
Asociacion de Familiares y Afectados de Lipodistrofias
Association ANNA
Associação Portuguesa CDG
Blackswan Foundation
Botswana Organisation for Rare Diseases
Canadian Organization for Rare Disorders
Chinese Organization for Rare Disorders
Croatian Alliance for Rare Diseases
Cutix Laxa International
Cyprus Alliance for Rare Disorders
DEBRA International
EURORDIS-Rare Diseases Europe
Federación Argentina de Enfermedades Poco Frecuentes
Federación Colombiana de Enfermedades Raras
Federación Española de Enfermedades Raras
Federacion Mexicana de Enfermedades Raras
Federation of European Associations of Patients Affected by Renal Genetic Diseases
Findacure
Genetic Alliance Australia
Genetic Alliance South Africa
Greek Alliance for Rare Diseases
Hong Kong Alliance for Rare Diseases
Iberoamerican Alliance for Rare Diseases
Instituto Vidas Raras
Indian Organization for Rare Diseases
International Federation for Spina Bifida and Hydrocephalus
International Gaucher Alliance
International Niemann-Pick Disease Alliance
International Organisation for Primary Immunodeficiencies
Japan Patient Association
Malaysian Rare Disorders Society
MCT8-AHDS Foundation
Naevus Global
National Alliance for Rare Diseases Support - Malta
National Organisation for Rare Diseases of Serbia
National Organization for Rare Disorders



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New Zealand Organisation for Rare Disorders
Nordic hypoPARA Organisation
Organization for Rare Diseases India
Philippine Society for Orphan Disorders
Pro Rare Austria
Pulmonary Hypertension Latin Society
Rare Disease Foundation of Iran
Rare Diseases South Africa
Rare Diseases Sweden
Rare Voices Australia
Retina International
Romanian National Alliance for Rare Diseases
Russian Patient Association
Thalassemia International Federation
The Ehlers-Danlos Society
The Federation of Esophageal Atresia and Tracheo-Esophageal Fistula Support Groups
World Alliance of Pituitary Organizations
World Federation of Hemophilia