



RARE
DISEASES
INTERNATIONAL

2020 ACTIVITY REPORT





I WANT TO JOIN. DUCHENNE MUSCULAR DYSTROPHY. THE NETHERLANDS

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Rare Diseases International (RDI) is the global alliance of Persons Living with a Rare Disease (PLWRD) of all nationalities across all rare diseases. RDI brings together national and regional rare disease patient alliances from around the world, as well as international rare disease-specific federations.

RDI's vision is a world where Persons Living with a Rare Disease and their families experience a better life through greater recognition and support, as well as through improved healthcare, social services and full inclusion in society, and respect for their human rights.

RDI was launched in 2015 as a EURORDIS initiative, and incorporated as a separate legally-registered organisation as of 2019 to create a strong common voice on behalf of all Persons Living with a Rare Disease around the world, advocate for rare diseases as an international policy priority, represent its Members, and enhance their capacities to act locally, regionally, and globally.

THE OBJECTIVES OF RDI ARE

- To promote rare diseases as an international public health, research and human rights priority through public awareness and policy-making;
- To represent its Members and Persons Living with a Rare Disease in international institutions and fora;
- To enhance the capacities of its Members through information, exchange, networking, mutual support, joint actions, as well as through training and workshops.



RARE
DISEASES
INTERNATIONAL

MILESTONES IN 2020

GOVERNANCE

RDI was incorporated under French law as an Association Loi 1901 on 9 October 2018, and has been operating as legally registered organisation since January 2019.

The Statutes (constitution) and the By-Laws (internal rules) of the organisation are available on the website: <https://www.rarediseasesinternational.org/governance/>

The RDI Council unanimously adopted revised By-Laws on 09 April 2020. These By-Laws are the internal rules for good governance, complementing the RDI’s Statutes. RDI Council Members Kin Ping Tsang and Ritu Jain led the process and elaboration of the draft By-Laws. The new document reads simpler, clearly outlines membership criteria, and affirms RDI’s independent status. The revised By-Laws were presented and adopted at the General Assembly in May 2020.

RDI COUNCIL ELECTIONS

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.



Durhane Wong Rieger
RDI CHAIR
(CORD)



Yann Le cam
RDI TREASURER
(EURORDIS)



Lisa Sarfaty
RDI SECRETARY
(NORD)



Rachel Yang
(CORD, CHINA)



Alba Ancochea
(FEDER, SPAIN)



Ritu Jain
(DEBRA INTERNATIONAL)



Kin-Ping Tsang
(RARE DISEASE HONG KONG)

Council elections were organised for April 2020 and all Full Members of RDI had the opportunity to vote online to replace or re-elect the following outgoing Members of the Council:

- Kin Ping Tsang, Rare Disease Hong Kong (RDHK), term 2017 – 2020
- Jesus Navarro, Ibero-American Alliance of Rare Diseases (ALIBER), term 2019 – 2020

COUNCIL MEMBER	ORGANISATION	YEAR OF ELECTION	END OF MANDATE
Alba Ancochea	Federacion Española de Enfermedades Raras (FEDER)	2020	2023
Kin Ping Tsang	Rare Disease Hong Kong (RDHK)	2020	2023
Durhane Wong-Rieger	Canadian Organization for Rare Disorders (CORD)	2019	2022
Yann Le Cam	European Organisation for Rare Diseases (EURORDIS)	2019	2022
Lisa Sarfaty	National Organization for Rare Disorders (NORD)	2018	2021
Rachel Yang	Chinese Organization for Rare Disorders (CORD)	2018	2021
Ritu Jain	Dystrophic Epidermolysis Bullosa Research Association (DEBRA) International	2018	2021

A total of five nominations were received and 19 full Members voted. Kin Ping Tsang was re-elected with nine votes and Alba Ancochea, from Federacion Española de Enfermedades Raras (FEDER), was elected with 12 votes.

RDI SECRETARIAT



In May 2020, **Paloma Tejada** - RDI Director from 2015 to 2020 – decided to accept a position at the IFPMA. RDI would like to warmly thank Paloma for her work and dedication to RDI.

In 2020, two new staff members joined RDI:

- In June 2020, **Flaminia Macchia** succeeded to Paloma Tejada as the RDI Executive Director in an up-scaled responsibility for a new phase of RDI;
- **Hlawulani Mkhabela** joined in March 2020 as the RDI Outreach and Engagement Manager. Hlawulani fosters RDI's relationship with Member Organisations, ensures the coordination of online events, and manages external communication including social media, the RDI website and monthly newsletter;
- In November 2020, **Veronica Lopez Gousset** joined the RDI Team as the Concept and Sustainability Volunteer for the CGN4RD Programme.

RDI also counts on the generosity of two high profile volunteers in New York, Claudia Hirawat and Rasha Alnaibari, who are dedicated to the advocacy work towards a UN Resolution on addressing the challenges of Persons Living with a Rare Disease and their families.



Flaminia Macchia
RDI
Executive Director



Clara Hervas
RDI
Public Affairs Manager



Hlawulani Mkhabela
RDI Outreach &
Engagement Manager



Ayda Ramazzina
RDI Governance &
Development Manager



Matt Bolz Johnson
RDI CGN4RD
Programme Director



Concha Mayo
RDI CGN4RD
Programme Support

Finally, RDI became an administratively registered organisation also in Belgium in December 2020 and, as of June 2020, RDI has a dedicated office space in the Brussels office of EURORDIS, enabling RDI to benefit from a supportive professional environment as well as from office equipment and meeting rooms.



FATHER AND DAUGHTER. SANFILIPPO SYNDROME. USA

COVID-19 RESPONSE

Recognising the impact of the COVID-19 pandemic on the global health community and its implications for the rare disease community, an exceptional session on COVID-19 from the perspective of rare disease patient organisations was added to the original agenda of the RDI Global Meeting.

Following the discussion at the Global Meeting, RDI developed and disseminated a survey for Member Organisations aimed at understanding the impact of the crisis on PLWRD in the countries represented by Members and how the pandemic was shaping the priorities and environment for rare disease patient organisations. The learnings from the survey on the impact of the pandemic on the rare disease community were shared with the World Health Organisation (WHO).

In July 2020, RDI published the common statement on “COVID-19 Responses and Recovery”, written with input from the RDI Advocacy Committee, which elaborates the findings of the survey and concerns expressed by rare disease patient organisations around the world.

Through dissemination and targeted outreach to its Members, RDI encouraged the participation of patient organisations outside Europe in the EURORDIS Rare Barometer Global Survey on COVID-19. Thanks to active dissemination from RDI Organisations, statistically significant responses were collected in a number of countries outside Europe, including South Africa, Chile, Australia and Peru.

In May 2020, RDI developed a website page dedicated to information resources on COVID-19 from rare disease patient organisations, which brings together surveys, statements and care guidelines from various rare disease communities. The dedicated webpage is updated on a regular basis.

A GROWING MOVEMENT OF PERSONS LIVING WITH A RARE DISEASE

A GLOBAL COMMUNITY – RDI MEMBERSHIP

An engaged membership base is vital to establishing an impactful global alliance of Persons Living with a Rare Disease.

Starting with 20 Member Organisations in 2015, RDI united **76 Members** by the end of 2020. New Members include six new national alliances for Argentina, Australia, China, Kenya, Peru and Zimbabwe, as well as seven international federations for specific diseases areas. Through RDI’s Members, the rare disease patient movement is represented in more than 100 countries worldwide.

NEW MEMBERS IN 2020

Alianza Argentina de Pacientes	GlobalSkin - International Alliance of Dermatology Patient Organizations
China Alliance for Rare Disease	Illness Challenge Foundation
Child & Youth Care, Zimbabwe	International Prader-Willi Syndrome Organisation
CMTC-OVM (Cutis Marmorata Telangiectatica Congenita and Other Vascular Malformations)	Leukemia Patient Advocates Foundation
Fabry International Network	Nicolaides-Baraitser Syndrome Worldwide Foundation
Global Alliance of Sickle Cell Disease Organisations	Rare Disorders Kenya
Genetic Support Network of Victoria	

In addition, the following Associate Members were granted RDI Full Membership: National Alliance for Rare Diseases Support – Malta, and the Ehlers-Danlos Society.

The full list of RDI Members is continually updated and published online at the following address: <https://www.rarediseasesinternational.org/members-list/>

Hlawulani Mkhabela is the primary contact and navigator for RDI Member Organisations and Patient Organisations at large.

Kin Ping Tsang, acting on behalf of the RDI Council, supports the review of new membership applications and makes recommendations regarding membership.

Lisa Sarfaty, RDI Council Member, brings her experience on developing capacity building programmes for NORD, to lead the development of RDI's new capacity building activities and programme.

COMMUNICATION & OUTREACH

In 2020, RDI continued to develop its communication and outreach channels to ensure Member Organisations are informed and can meaningfully engage in activities, and all stakeholders understand RDI's priorities and programmes.

The **monthly electronic newsletter** provides an overview of important updates, news, and events pertaining to RDI and the international rare disease community. Disseminated primarily to Members, in 2020 dissemination was broadened to include several key stakeholders and the general public. The newsletter provides a simple and streamlined breakdown of key developments, and serves to remind Members of activities that they can participate in, such as webinars and events, and opportunities for collaboration. The newsletter is also available on the RDI website.

Over the last year, the organisation has significantly extended its visibility on Social Media. **Twitter** allows for quick dissemination of information about programmes and campaigns, event registration, and topics of interest. It also enables RDI to show support for complementary organisations and movements, e.g. UHC2030, NCD Alliance, and International Disability Alliance, as well as share updates from Members and other stakeholders.

In April 2020, RDI created a **LinkedIn Profile**, which features policy papers, statements and articles from RDI and international rare disease stakeholders. In addition, the **RDI YouTube Channel** was launched and includes webinars and advocacy campaigns.



Meet the Patient Advocates Video Series

To put the spotlight on the diverse global community of patient advocates at the heart of RDI, in September 2020, the RDI **“Meet the Patient Advocates Video Series”** was initiated. Each episode tells the story of a unique journey to rare disease patient advocacy, and explores the national environments for PLWRD. Visitors of RDI's website and YouTube Channel may currently view videos from patient representatives based in Australia, Argentina, Ghana, Italy, Singapore and the Netherlands.

The **UHC4RareDiseases Campaign** (see below) launched in October was RDI's first broad social media campaign, which includes a social media toolkit to empower rare disease organisations and the public to advocate for UHC. RDI also collaborated with EURORDIS to run concurrent UHC4RareDiseases social media campaigns in the months leading to **UHC Day on 12 December 2020**.

The RDI website (www.rarediseasesinternational.org) is continuously updated and is the main source of information for the public on RDI activities and the important topics and events on the agenda of the international rare disease community. In July 2020, a language add-on was integrated to the website allowing for limited translation through Google Translate in French, Spanish, Portuguese, and Simplified Chinese.

From November 2020, **Members** also began receiving **weekly email updates** with a quick rundown of important information and actions.

Microsoft Teams is currently **RDI's primary co-working platform**. Over 50 patient representatives used the Microsoft Teams page to access programme resources and contribute to key reports and deliverables for the WHO CGN4RD.

Hlawulani Mkhabela leads the communication with RDI Members to facilitate their full engagement in programmes, events and activities. **Hlawulani** also oversees content and design of the RDI website and develops social media campaigns and content.

RARE DISEASE DAY

Rare Disease Day is the international awareness raising campaign led by the global rare disease community, initiated, led and coordinated by EURORDIS.



RDI facilitates the participation of patient organisations outside Europe in the campaign and represents the voice of the global community at every stage of the campaign.

In 2020, **RDI Member rare disease National Alliances had the opportunity to become official partners of the Rare Disease Day campaign**. Official Rare Disease Day partners are national alliances driving patient engagement and raising awareness of rare diseases in their countries. Through the Rare Disease Day Working Group, supported and facilitated by EURORDIS, **RDI national alliances shaped key elements of the 2020 campaign, including messaging and materials**.

RDI RARE DISEASE DAY PARTNERS 2020

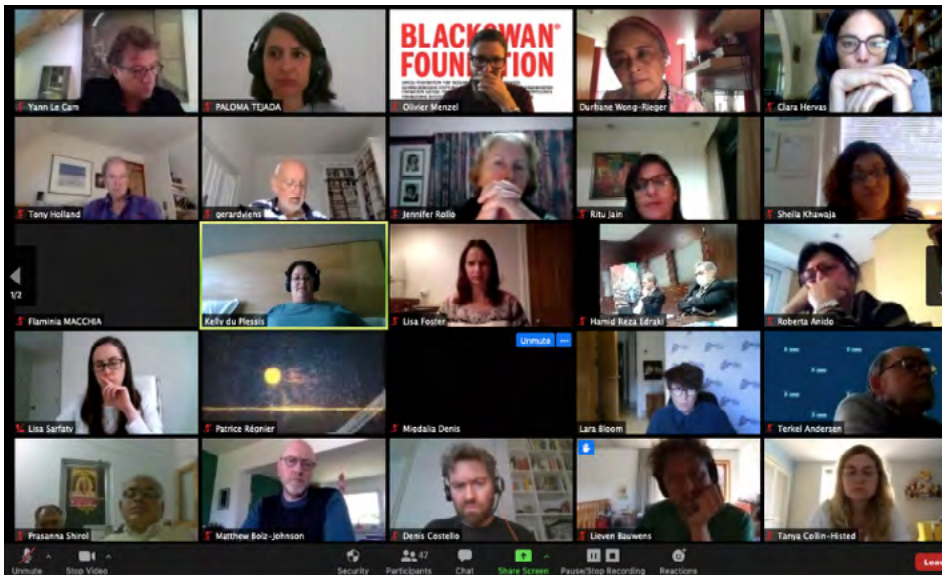
Arabic Organisation for Rare Diseases	Federación Mexicana de Enfermedades Raras	National Organization for Rare Disorders
Associação Brasileira de Enfermidades Raras	Rare Disease Hong Kong (formerly Hong Kong Alliance for Rare Diseases)	Organization for Rare Diseases India
Botswana Organisation for Rare Diseases	Indian Organization for Rare Diseases	Philippine Society for Orphan Disorders
Canadian Organization for Rare Disorders	Instituto Vidar Raras	Rare Diseases Foundation of Iran
Chinese Organization for Rare Disorders	Japan Patient Association	Rare Diseases South Africa
Federación Colombiana de Enfermedades Raras	Malaysian Rare Disorders Society	Rare Disease Ghana Initiative
Federación Argentina de Enfermedades Poco Frecuentes	New Zealand Organization for Rare Disorders	Rare Voices Australia

RDI 6TH ANNUAL MEETING

RDI GENERAL ASSEMBLY AND MEMBERSHIP MEETING

The RDI General Assembly and Membership Meeting took place on 18 May 2020. Planned to coincide with the World Health Assembly in Geneva, the meetings were held completely online in response to restrictions imposed by the COVID-19 pandemic.

54 patient representatives from RDI Member Organisations took part in the meetings. Prior to the General Assembly, RDI Full Members voted online on Resolutions to adopt three documents (Activity Report 2019, Financial Report 2019, and Action Plan & Budget 2020).



Annual General Assembly and RDI Membership Meeting

The Membership Meeting was an opportunity for newly elected Members of the Council to introduce themselves and speak directly to the RDI Members. Also, presented at the Meeting were RDI’s revised By-Laws, which outline the internal rules of governance and procedures of RDI as an incorporated and legally registered organisation.

RDI GLOBAL MEETING

RDI hosted the first exclusively virtual **Global Meeting on 19 May 2020**, bringing together **180 stakeholders from 48 countries** including organisations for PLWRD, industry, academia, research, government and policy makers.

Participants discussed RDI’s advocacy and operational activities aimed at adding rare diseases on the international agenda, as well as the role patient organisations were playing to support the rare disease community through the COVID-19 pandemic.

The 4-hour virtual event opened with a presentation from the Chair of the RDI Council, Durhane Wong-Rieger, on the **successful global rare disease advocacy leading to the inclusion of rare diseases in the historical UN Political Declaration on Universal Health Coverage (UHC)** in September 2019. The discussion was followed by a case study from Alba Ancochea, CEO of the Federacion Española de Enfermedades Raras (FEDER), on rare diseases in Spain’s UHC policies.

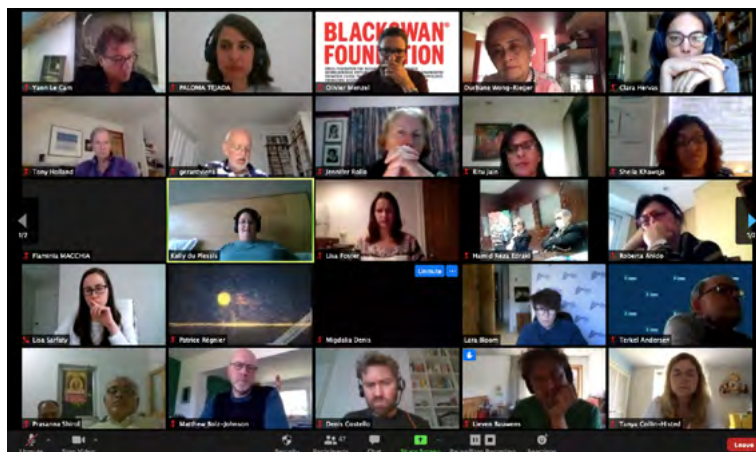
Yann Le Cam, CEO of EURORDIS, introduced **RDI’s Memorandum of Understanding (MoU) with WHO**, signed in December 2019, as well as the framework for collaboration between WHO and the international rare disease community, allowing to move towards the Collaborative Global Network for Rare Diseases (CGN4RD). Ana Rath, Orphanet – INSERM Director, affirmed the importance of working toward a common definition framework for rare diseases, a key part of the MoU with the WHO.

The event concluded with an open discussion on **the impact of COVID-19 on PLWRD across the globe**. Leaders of the patient community in Ghana, South Africa, Latin America, the U.S.A., and Canada described the impact of the pandemic on rare disease patients in their part of the world, as well as their responses to the crisis.

BUILDING A COMMUNITY OF GLOBAL PATIENT ADVOCATES

RDI’S ADVOCACY COMMITTEE

The Advocacy Committee is responsible for developing and recommending priorities, initiatives and policies for the organisation; for preparing position papers or statements or declarations, as well as to review any position, statement, declaration or petition considered for endorsement by RDI.



Yann Le Cam, RDI Council Member and CEO of EURORDIS, is the Chair of the Advocacy Committee. 16 patient representatives from RDI’s Member Organisations, and a representative from the International Alliance of Patient Organisations (IAPO), take part in this Committee and represent a wide range of countries and diseases.

They following patient advocate joined the Advocacy Committee in 2020:

- Salome Mekhuzla, WFH

The following patient advocates left the Advocacy Committee in 2020:

- Heather Richards, WFH
- Maureen Smith, CORD (Canada)



Yann Le Cam
EURORDIS Chair



Salomé Mekhuzla
World Federation
of Hemophilia



Ritu Jain
DEBRA Intern.
APARDO



Kelly du Plessis
RDSA-
South Africa



Ramaiah Muthyala
(FEDER, SPAIN)



Lara Bloom
Ehlers-Danlos
Society



Leir Solis
IPOPI



Luciana Escati
FADEPOF-
Argentina



Eleni Antoniou
Thalassemia
International



Sanja Peric
RD-Croatia



Migdalia Denis
Latin PH Society



Lieven Bauwens
Child-Help



Alba Ancochea
FEDER-Spain



Jesús Navarro
OMER-Mexico
ALIBER



Simone Boselli
EURORDIS



Kawaldip Sehmi
IAPO



**Roberta Anido
de Pena**
FEDEPOF-Argentina

Clara Hervas, RDI's Public Affairs Manager, coordinates the work of the RDI Advocacy Committee, engaging its Members in a process of co-creation of advocacy campaigns via meetings and internal consultations, and keeping them informed on advocacy files of relevance to RDI.

In 2020, the RDI's Advocacy Committee contributed to the elaboration of the following papers:

- The Statement on "COVID-19 Responses and Recovery"
- The UHC advocacy Toolkit
- The Key Asks and Draft UN Resolution on addressing the challenges of PLWRD and their families

RDI warmly thanks the Members of the Advocacy Committee for their time, reactivity and contribution.

FRIENDS. VITILIGO. INDIA





MECKENZIE. SANFILIPPO DISEASE. AUSTRALIA

PARTNERING WITH OTHER SECTORS TO BUILD A SUSTAINABLE ORGANISATION

RDI ALLIANCE OF COMPANIES FOR PATIENT-CENTRED ACTION

RDI's primary means of engaging companies is through the RDI Alliance of Companies, a platform to foster a long-term educational relationship between RDI and companies that have an impact on Persons Living with a Rare Disease. Companies have the opportunity to increase their understanding of the rare disease patient movement and landscape, to inform, and to contribute to its success. The Alliance of Companies aims to:

- Build an international network of companies working in areas that affect rare disease patients. Examples include pharmaceutical and biotechnology, medical equipment and devices, research, information technology, data mining and management, artificial intelligence, transport and logistics.
- Exchange perspectives on issues vital to various industries and to Persons Living with a Rare Disease, as reflected in RDI's advocacy agenda.
- Stimulate companies across many industries to address the needs of Persons Living with a Rare Disease
- Share survey results, key findings, feedback on needs of Persons Living with a Rare Disease
- Provide financial support to advance RDI's mission

In 2020, the following **10 companies** joined the Alliance of Companies: **Actelion, Amicus Therapeutics, BridgeBio, CSL Behring, Kyowa Kirin, Pfizer, Sanofi Genzyme, Roche, Takeda, and Vertex.**

The RDI Secretariat organised **two online meetings** with the members of the Alliance of Companies in 2020. On 23 June, RDI presented the **Global State of the Art for Rare Diseases Resource project** to discuss the new database and obtain the perspectives of companies. On 10 December, RDI introduced the Collaborative **Global Network for Rare Diseases** to present the project plan, priorities and activities.



RDI would like to thank Jill Bonjean, who has led RDI's relationships with private donors since RDI's inception and successfully launched the RDI Alliance of Companies when RDI became a fully-fledged NGO in 2019.

ADVOCACY ACTIONS IN THE UNITED NATIONS SYSTEM

RARE DISEASE DAY POLICY EVENT

On 25 February 2020, RDI organised **“Rare Diseases, leaving no one Behind” – a photo exhibit honouring life with a rare disease** to mark the occasion of Rare Disease Day. Hosted at the United Nations headquarters in Geneva, on the margins of the 43rd Session of the Human Rights Council, the event was supported by the Permanent Missions of Cyprus and Brazil.

Mr. Nikos Christodoulides, Minister of Foreign Affairs of Cyprus, and Mrs. Damares Alves, Minister of Women, Family and Human Rights of Brazil, spoke at the inaugural ceremony, reaffirming support from their respective countries for the rare disease cause as well as the place for PLWRD in UHC policies. UN Permanent Missions and the public attended the inaugural event, and the exhibit was open for nine days to help raise awareness amongst UN representatives and visitors to the UN.

The showcase combined two photographic projects – **‘Unlimited Possibilities’**, a collaboration between world-class photojournalist Petros Petrides and Unique Smiles, a Cyprian civil organisation for rare genetic disorders, and **‘Rare Lives’**, a photographic journey involving 70 families and crossing seven European countries.



RDI Rare Disease Day Policy Event - “Rare Diseases: Leaving No One Behind”

RARE DISEASES & UNIVERSAL HEALTH COVERAGE

Universal Health Coverage for Rare Diseases (#UHC4RareDiseases) Campaign



In October 2020, RDI launched the ‘Universal Health Coverage for Rare Diseases’ (#UHC4RareDiseases) campaign, in a joint effort with EURORDIS-Rare Diseases Europe and members of both umbrella organisations. **The campaign aimed to create awareness and ask policy makers to safeguard equity and consider the needs of Persons Living With a Rare Disease in national UHC strategies and essential health service packages in the lead up to Universal Health Coverage Day (December 12). The campaign will continue to serve as an instrument to advocate for this key topic for the rare disease community at both global and national level.**

The campaign materials were designed with feedback from the RDI Advocacy Committee and comprised of a **template letter, a factsheet, briefing package and social media posts**. National alliances and international federations could share the campaign materials with policy makers from countries that adopted the UN Political Declaration on UHC in September 2019 and committed to strengthening efforts to address rare diseases as part of UHC.



To mark UHC Day 2020, RDI co-organised a webinar with EURORDIS on the 11 December 2020 on ‘Addressing Rare Diseases through UHC’. The **webinar** was attended by 76 participants and included testimonials from the Asia Pacific Organisation for Rare Disorders (APARDO) and the Botswana Organization for Rare Diseases (BORDIS); case-studies on advocating for UHC and using the UHC4RareDiseases toolkit by the Federación Mexicana de Enfermedades Raras (FEMEXER), the Finnish Network for Rare Diseases, and the International Prader-Willi Syndrome Organisation (IPWSO); **as well as a Keynote Closing Address by Dr. Rüdiger Krech, Director of the Department of Ethics and Social Determinants of Health at the WHO.**

Details on the campaign and the webinar available at:
<https://www.rarediseasesinternational.org/uhc4rarediseases/>

Rare Diseases in the UHC2030 Synthesis report



As a member of UHC2030, a multi-stakeholder platform convened by the WHO and the World Bank, RDI continued to raise awareness and represent the voice of Persons Living With a Rare Disease within this forum. **A key milestone in 2020 included the inclusion of the challenges of the rare disease community within the UHC2030’s «State of Commitment to UHC» synthesis, the first edition of an annual report monitoring action towards Universal Health Coverage in all UN Member states.**

The synthesis report noted that the COVID-19 pandemic had magnified inequities for vulnerable groups including Persons Living With a Rare Disease, and it also highlighted the need, not only for better coverage of health services but also for improved specialised health services, making particular mention of the rare disease community. This was the result of a concerted action by RDI and its Member Organisations, which jointly participated in the UHC2030 survey on national UHC context in September 2020.

Report available at:
https://www.uhc2030.org/fileadmin/uploads/uhc2030/Documents/Key_Issues/State_of_UHC/SoUHCC_synthesis_2020_final_web.pdf

Clara Hervas, as RDI's Public Affairs Manager, coordinated all advocacy actions related to the call for UHC for Persons Living with a Rare Disease. In 2020, she led the development of the 'UHC4RareDiseases' campaign with the RDI Advocacy Committee and she continued her role as designated focal person within UHC2030.

Call for a United Nations General Assembly (UNGA) Resolution on Addressing the Challenges of Persons Living with a Rare Disease and their Families



In 2020, **RDI set up the bases for the launch of an official advocacy campaign calling for the adoption of a UNGA Resolution on Addressing the Challenges of Persons Living with a Rare Disease and their families in 2021.** This strategy was led by Yann Le Cam, with the support of Clara Hervas and Flaminia Macchia, together with the RDI Advocacy Committee and RDI Council.

In this context, RDI led two processes in parallel:

1. Internal process:

In 2020, RDI managed the internal process for the elaboration of the messaging for the campaign and acted as the coordinator with its partners, the **NGO Committee for Rare Diseases and EURORDIS-Rare Diseases Europe:**

- a. With input from the RDI Advocacy Committee, RDI drafted the **'Key Asks from the Rare Disease Community'** that aimed to present in a concise way the arguments towards a UNGA Resolution and the types of actions that would be expected within it. This was developed taking into account the RDI Joint Declaration, the RDI position paper on UHC, the outputs from the two events organised at the UN Headquarters and a mapping and literature review of past UNGA Resolutions addressing issues around human rights, development, disability and vulnerable populations;
- b. **RDI coordinated the development of a proposal for a 'draft UNGA Resolution' from the rare disease community.** In consultation with the RDI Advocacy Committee, the RDI Council, the EURORDIS Board of Directors, and the NGO Committee for Rare Diseases Board finalized the proposal for a UNGA Resolution of a text in the same format as an official UNGA Resolution to be presented in confidentiality to interested sponsoring Member States in 2021.

2. Frontline negotiations

RDI together with two highly committed volunteers based in New York, Claudia Hirawat and Rasha Alnaibari, acting as RDI's liaison to the UN System, carried out a process of outreach to Permanent Missions to the UN for the creation of a supportive group of Member States that would take ownership of the UNGA Resolution and present it for adoption in 2021. **By the end of 2020, RDI was in touch with a core group of UN Member States from different regions of the world and was in a good place to obtain their official support by early 2021 and to announce this at the policy event organized to mark Rare Disease Day in February/March.**

GLOBAL INITIATIVES AND PROGRAMMES – SUPPORTING ACCESS TO DIAGNOSTICS, TREATMENTS AND CARE

MEMORANDUM OF UNDERSTANDING BETWEEN WHO AND RDI: 1ST YEAR OF EXECUTION

In December 2019, RDI and WHO signed a Memorandum of Understanding based on ambitious goals and long-term framework of collaboration that will contribute to the WHO 13th General Programme of Work.

In the first year of its implementation, activities under the Memorandum of Understanding (MoU) focused on a description framework to define rare diseases internationally and on laying the ground for the development of a global network of centres of excellence for rare diseases.

Throughout 2020, WHO and RDI have successfully collaborated to develop the following deliverables:

- A paper outlining the **Operational Description Framework for rare diseases** (WHO D0) that may inform the WHO and its Member States when considering national, regional and global decisions;
- A **Population Needs Assessment Study Report** (WHO D1) capturing the rare disease community needs from around the world and mapping the healthcare landscape maturity to address these needs;
- A **Concept and Methodological Model** for a Global Network for Rare Diseases (WHO D2) proposing how a global collaboration could be organised to ensure that every country and region can connect under a Global Network and be best structured to address the spectrum of needs from around the world.

WHO D0 will be used as a basis of the next stage of the work whereby the WHO and RDI will collaborate to further elaborate an **operational description of rare diseases and key prevalence or incidence figures** that may serve as a common reference.

WHO D1 and WHO D2 will be used to inform the development of a Global Network of multidisciplinary specialised expert centres for rare diseases, from now on referred to – at WHO’s request - as the **WHO Global Network for Rare Diseases (WHO CGN4RD)**.



In relation to the WHO Collaborative Global Network, RDI has led the development of **five-years Work Programme** to coordinate the activities with the rare disease community to scope-out and develop, in a **stepwise approach, a pilot phase for the CGN4RD**. RDI drafted the planned activities in the Work Programme and Technical Research Plan, which were submitted in September and signed off by the WHO in October 2020.

RDI engaged with its Members through a series of focus groups to map the needs of the rare disease community, identifying both common and population-specific needs, as well as mapping the characteristics of healthcare systems. The focus groups were organised in WHO region and sub-regions. A total of 64 patient advocates based in 33 countries took part in the focus groups. RDI also undertook a desk review of regions, which were under-represented and conducted virtual interviews with clinical leads from these regions. The experiences and opinions captured in the focus groups were included in a series of regional reports. A review was completed of the needs across the six WHO region in order to inform the development of the first WHO deliverable: The Needs Assessment Study report.



RDI also completed additional technical research: a literature review in order to identify the evidence for centralisation of expertise and accreditation of expert centres, as well as the evidence base for added-value of networked care in the field of rare diseases. RDI also conducted a series of semi-structured interviews to identify the characteristics and content of existing networks, and capture the insights and opinions of key stakeholders on their added-value, impact and success factors of existing national, regional and global networks. Ten networks were selected to be included in the interviews process as well as interviews with key opinion leaders and RD leads from the rare disease community including representatives from the Key Opinion Leader, National Authority Lead, Digital Lead and Pharmaceutical Industry Lead. A total of 33 leaders were interviewed.

The findings, evidence and insights gain from the technical research were planned to be triangulated, to inform the design of WHO Collaborative Global Network for RD and its RD Hub Members, which is detailed in the second WHO deliverable – the Concept & Methodological Model.

The Concept & Methodological Model was co-produced through a series of focus groups with the RDI Members and refined based on the published evidence base and success factors and the methodology, characteristics, content and experiences gained from the existing Networks. The Model details a proposal for the structure and scope of the WHO CGN4RD and ensure that all potential Hub Members and patient populations from the different global regions are able to access and connect, given the diversity of the different healthcare systems across the WHO regions; and **supporting Universal Health Coverage for all and reducing health inequalities.**

In Q4 2020, RDI invited patient leaders and medical experts recommended by RDI Members to take part, in 2021, as volunteers in an international Panel of Experts. **Over 190 experts consented to take part in the Panel of Experts, representing 101 countries (51.8% of the WHO Member States).** The overall purpose of the Panel of Experts is to independently validate the needs identified and review the technical research findings and conclusions, so that they can be used to inform the design the WHO CGN4RD.

This RDI strategic initiative and flagship collaboration with the WHO was initiated and stewarded by Yann Le Cam, and led throughout 2020 by **Matt Bolz-Johnson**, RDI Programme Director for the Collaborative Global Network for Rare Diseases, with the support of **Concha Mayo**, RDI Programme Manager for the Collaborative Global Network for Rare Diseases, and **Veronica Lopez Gousset**, CGN4RD Concept and Sustainability Volunteer.

WHO ESSENTIAL LISTS

The RDI Global Meeting included a discussion on practical ways for patient organisations to use WHO frameworks and tools, now that medicinal products for rare disease are, and can be more, included in the WHO Essential Medicines List and WHO Essential in Vitro Diagnostics List.

Yann Le Cam introduced the WHO Essential Lists and their potential to support access to rare disease medical products and diagnostic tools around the world. Johan Prévot, Executive Director of the International Patient Organisation for Primary Immunodeficiencies ([IPOPI](#)), presented a case study to illustrate how and why patient groups can apply for inclusion in the WHO Essential and Priority Lists.

SUPPORTING COUNTRIES EMERGING TO RARE DISEASES AND BUILDING CAPACITIES AMONGST PATIENT LEADERS OF THE RARE DISEASE COMMUNITY

CAPACITY BUILDING AND CONFERENCE SUPPORT

In 2020, RDI began designing a Capacity-Building Programme, which will be launched in 2021. RDI's Capacity Building Programme aims to address priorities identified by Members and to empower rare disease patient groups to engage meaningfully in global collaborations and shape RDI programmes and strategic direction.

In April, Members were asked to complete a comprehensive survey on their capacity building needs and international advocacy priorities. The survey included questions on training needs, preferred platforms and tools, as well as the advocacy priorities of each organisation. **Survey findings, ongoing discussions with patient organisations, and a review of best practices from patient organisations will inform a medium-term capacity building strategy and activities for the next three years starting from 2021.**

CONFERENCES AND FELLOWSHIPS IN 2020

Due to the travel restrictions adopted in response to the COVID-19 pandemic, all events supported by RDI in 2020 were hosted online.

RDI supported the following events:

- > **European Conference on Rare Diseases (ERCD)** – organised by EURORDIS on 14 – 15 May. RDI was an official partner to the ECRD conference. Recognised as the world's largest patient-led rare disease event, ECRD 2020 convened participants from 57 countries. 13 fellowships were offered to enable RDI Member Organisations to attend the virtual conference.

Durhanne Wong Reiger pre-recorded an ECRD conference session entitled "Getting our rights 'right': An international framework for rare diseases". Speakers for the session included Anders Olauson, Chair of the NGO Committee for Rare Diseases, Raquel Peck, Senior Advisor and Former CEO of World Hepatitis Alliance, and Todd Howland, Chief of the Development and Economic and Social Issues Branch, UN OHCHR

- > **China Conference on Rare Diseases** - organised by the China Alliance for Rare Disease (CHARD) on 24 – 25 October 2020.

Durhane Wong-Rieger introduced RDI and Matt Boltz-Johnson presented a discussion on the WHO CGN4RD. Both participated in a panel discussion entitled "What can China do for the rest of the world in rare diseases?"

- > **Conversatorio Poco Frecuente (Rare conversation)**, organised by Federación Argentina de Enfermedades Poco Frecuentes (FADEPOF) on 4 – 6 November 2020.

RDI Public Affair Manager Clara Hervas was invited to speak about international advocacy for rare diseases and the call for a UN General Assembly Resolution on PLWRD

- > **RareFest2020**, organised by Cambridge Rare Disease Network on 28 November 2020.

RDI supported the event as a Media Partner

- > **Transforming Rare Disease Across the Asia Pacific** organised by the Asia Pacific Alliance of Rare Disease Organisations (APARDO) on 10 December 2020

RDI (Matt Boltz-Johnson) led a discussion on the potential of WHO CGN4RD and international advocacy

- > **VIII Encuentro Iberoamericano de Enfermedades Raras** organised by Iberoamerican Alliance for Rare Diseases (ALIBER) on 29 – 31 October 2020.

RDI (Flaminia Macchia, Matt Boltz-Johnson, Concha Mayo, and Clara Hervás) contributed to a discussion on the WHO CGN4RD and UHC for rare disease advocacy

- > **World Orphan Drug Congress Europe** organised by Terrapinn on 2 – 5 November 2020.

RDI supported the event as a Media Partner

CSL Behring, Pfizer, and Roche supported this Programme in 2020.

RDI WEBINARS

- > An Introduction to the WHO CGN4RD on 27 April 2020
- > RDI Global Meeting on 19 May 2020
- > Addressing Rare Diseases through Universal Health Coverage on 11 December 2020

RDI PRESENCE IN THE INTERNATIONAL RARE DISEASE ECOSYSTEM

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), an important interface between the world's NGOs and the UN system. The Committee is one in forty CoNGO Committees, approved by the CoNGO General Assembly in 2014 and instigated by the CoNGO President in 2015. The Committee was then publicly launched in November 2016 at its First High Level Event at the United Nations.

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 the issues of relevance to Persons Living With a Rare Disease with the UN 2030 Sustainable Development Agenda: the Sustainable Development Goals (SDGs). The current goal of the Committee is to work with its NGO partners towards the adoption of a UN General Assembly Resolution on Persons Living with a Rare Disease.

RDI is a Member of the Inception Executive Board of the NGO Committee for Rare Diseases. The Chair of the Committee is **Anders Olauson**, Founder and Chair of the Agrenska Centre for Rare Diseases. The Vice-Chair of the Committee is Yann Le Cam, CEO of EURORDIS-Rare Diseases Europe. The other Members of the Executive Board are the World Federation of Haemophilia, International Federation for Spina Bifida and Hydrocephalus, International Alliance of Patients' Organisations, and the International Alliance of Women. RDI's Chair, Durhane Wong-Rieger, is RDI's representative within the Executive Board of the NGO Committee for Rare Diseases.

INTERNATIONAL RARE DISEASE RESEARCH CONSORTIUM (IRDiRC)



IRDiRC, the International Rare Disease Research Consortium, was officially launched in 2011, with the vision to enable all persons living with a rare disease to receive an accurate diagnosis, care, and available therapy within one year of coming to medical attention. IRDiRC experience in the past decade has shown that the international rare diseases research community is eager to share knowledge and experience, and work collaboratively across borders in order to bring diagnoses and therapies to patients.

The revised set of goals for the decade 2017 – 2027 is to accelerate progress towards its ambitious vision and push the limits of what is currently possible in the longer term, with rare disease patients' lives in mind. The three specific goals are the following ones:

- **Goal 1:** All patients coming to medical attention with a suspected rare disease will be diagnosed within one year if their disorder is known in the medical literature; all currently un-diagnosable individuals will enter a globally coordinated diagnostic and research pipeline;
- **Goal 2:** 1000 new therapies for rare diseases will be approved, the majority of which will focus on diseases without approved options;
- **Goal 3:** Methodologies will be developed to assess the impact of diagnoses and therapies on rare disease patients.

IRDiRC is governed through a Consortium Assembly, an Operating Committee, three Constituent Committees and three Scientific Committees, aided by ad hoc Task Forces. The Scientific Secretariat, together with the Office of the IRDiRC Chair, Lucia Monaco, provides organisational and communications support and manages IRDiRC actions on strategic projects and activities.

In 2020, RDI took part to a series of meetings within the following IRDiRC structures:

- RDI is represented in the **Consortium Assembly**, IRDiRC main governing body. In 2020, there were three meetings of the Consortium Assembly: 18 March, 19 July, and 20 October.
- RDI is represented in the **Patient Advocates Constituent Committee (PACC)**, which aims to address issues that apply to all members and to the patient community at large, highlight points in the development process where patient involvement is crucial, and measure the impact of patient involvement. The PACC is composed of 15 umbrella patient groups of which **13 are Member Organisations of RDI and RDI is itself represented by Durhane Wong Rieger, as Chair of the PACC.**
- RDI is represented, via Durhane Wong Rieger being Chair of the PACC, in the **Operating Committee**, which consists of the Chair and Vice Chair of the Consortium Assembly, the Chairs and Vice Chairs of the Constituent and Scientific Committees, and the Scientific Secretariat. The Operating Committee meets regularly to prepare and advance IRDiRC activities, process information, and enable a more effective management of the consortium as a whole.

- **The Working Group on Rare Diseases Treatment Access**, co-chaired by Durhane Wong Rieger and Bill Gahl, was launched in 2020 with the following objectives: 1. To create a list of standard-of-care products for rare diseases and make the list available to countries throughout the world. The list should be updated periodically; and 2. To identify the barriers to accessing rare disease drugs, particularly in low-and-middle income populations. In 2020, the Working Group has been launched and discussions were kicked-off via teleconferences; **the existing orphan drugs and rare disease drug lists were mapped (compiled List of Essential Rare Disease Medicines) and a White Paper on Rare Disease Treatment Access was elaborated and is ready for publication.**
- Durhane Wong Rieger also participated in the **Chrysalis Task Force** whose overarching goal is to identify key criteria that would make rare diseases research more attractive to industry for research and development. The proposed task force would serve as a critical link between industry, funders, and advocates in accomplishing the stated goal.
- In 2020, RDI was also involved in the preparation of the **IRDIRC Conference & RE(ACT) Congress** (held in January 2021): Preparation of conference sessions with venue change and session redesign from in-person to virtual meeting.

GLOBAL COMMISSION TO END THE DIAGNOSTIC ODYSSEY FOR CHILDREN WITH A RARE DISEASE



The Global Commission to End the Diagnostic Odyssey for Children with a Rare Disease, created by EURORDIS, Microsoft, and Takeda in 2018, is a multi-disciplinary group of experts committed to shorten the time to diagnosis by building a road map to improve diagnostic pathways for children with a rare disease.

The three co-chairs of the Global Commission are Gregory Moore (Corporate Vice President, Microsoft Health), Wolfram Nothaft (Chief Medical Officer, Takeda), and Yann Le Cam (Chief Executive Officer, EURORDIS-Rare Diseases Europe).

In 2020, EURORDIS facilitated and supported greater participation and visibility of RDI in the Global Commission Secretariat and work streams. ARDI, together with RDI Members, actively supported the vision, mission and activities of the Global Commission.

The overall objective of RDI's involvement in the Global Commission is to promote greater integration of the patient voices in countries outside Europe and the USA in the Global Commission's activities and ensure a more international perspective.

RDI is directly represented and involved in the activities of two Working Groups:

1. The **Empowerment & Awareness Working Group** (Flaminia Macchia is the Secretariat Lead) - Broaden awareness about the need to reduce time to diagnosis and empower key stakeholders to accelerate diagnosis;
2. The **Policy & Advocacy Working Group** (Simone Boselli is the Secretariat Lead) - Advocate for local and global policy change to build an enabling environment for rare disease diagnosis.

In 2020, RDI was involved in the development of the **Patient Empowerment & Awareness Campaign (Rare Navigator)** that will be further piloted in low and middle-income countries. RDI also closely worked with the Global Commission and the EURORDIS Rare Barometer Team to expand the geographical coverage of the Rare Barometer in countries outside of Europe, starting with six countries for a pilot phase at international. RDI was also involved in setting the priorities for 2021, based on further expansion of the international scope of the Global Commission activities.

APEC RARE DISEASE NETWORK



RDI was invited to present at the Policy Dialogue on Developing a New Strategic Plan for Life Sciences Innovation, hosted by the Asia-Pacific Economic Cooperation (APEC) Life Sciences Innovation Forum (LSIF) on 17 September 2020.

The event was a unique opportunity for participants to engage in open dialogue with government, industry, academia, and civil society on the future of biomedical innovation in the Asia Pacific. Durhane Wong-Rieger, in her capacity as RDI Chair and Patient Lead Advisor of the APEC Rare Disease Framework, gave an overview of the role patient organisations can play in improving access to transformative therapies. The discussion explored barriers to access as well as the potential for structured cooperation between developers, payers and patients.

On 30 October and 2 November, RDI participated in panel discussions on the impact of the COVID-19 crisis on PLWRD in Chile and the Asia Pacific at the APEC Virtual Consultation on COVID-19 & Rare Diseases in Chile.

APARDO President and RDI Board Member, Ritu Jain hosted two workshops with the objective of empowering patient organisations to advocate for rare diseases using the APEC Rare Disease Framework and Universal Health Coverage. These workshops were supported by APARDO, RDI, APEC, and the CORD and took place online on 16 November and 23 November 2020.

On 1 – 2 December 2020, Durhane Wong-Rieger was part of the APEC Virtual Workshop on Facilitated Regulatory Pathways organised in partnership with the APEC Harmonization Center and the APEC Regulatory Harmonization Steering Committee. The multi-stakeholder workshop aimed to facilitate the sharing of perspectives on the use of facilitated regulatory pathways.

GLOBAL STATE OF THE ART RESOURCES



The funding mechanism for the previously existing **Report on the State of the Art of Rare Disease activities in Europe** was discontinued in 2020. Yann Le Cam created an opportunity for EURORDIS-Rare Diseases Europe to re-think the model of such a valuable resource and to elevate it to the global level with RDI. Yann Le Cam and Flaminia Macchia, with the support of colleagues at EURORDIS and together with the RDI Council, defined the conceptual international framework and execution, as well as re-shaped the information collection and validation, the report contents, geographic scope, and distribution model. The ambition is to create a public good for policy-makers, rare disease stakeholders, and RDI Members, while promoting national strategies on rare diseases.

In 2020, RDI led a Survey, with the support of Ellen Coleman, Chief Executive and President of VOZ Advisors, to explore whether private sector funding would be available to fund an updated resource. Approximately 15 pharma/biotech companies expressed interest to support an enhanced version of the current report.

In 2020, RDI also developed a concept model, together with EURORDIS and Orphanet, for the enhanced version of the Global State of the Art Resource that will be the basis for further discussion within RDI Membership, and other stakeholders in 2021 to finalise the feasibility.



LUONG. MUCOPOLYSACCHARIDOSES. VIETNAM

ANNEX 1

FULL LIST OF RDI MEMBER ORGANISATIONS AS OF DECEMBER 2020

1. ACHSE – Allianz Chronischer Seltener Erkrankungen
2. AELIP – Asociación Internacional de Familiares y Afectados de Lipodistrofias
3. ALAPA – Alianza Argentina de Pacientes
4. ALIBER – Alianza Iberoamericana de Enfermedades Raras, Huérfanas o Poco Frecuentes
5. Alliance Maladies Rares
6. APARDO – Asia Pacific Alliance of Rare Disease Organisations
7. AORD – Arabic Organisation for Rare Diseases
8. APCDG – Associação Portuguesa CDG e outras doenças Metabólicas Raras
9. Association ANNA
10. ASrid – Advocacy Service for Rare and Intractable Diseases
11. FEBER – Associação Brasileira de Enfermidades Raras
12. BLACKSWAN Foundation
13. BORDIS – Botswana Organisation for Rare Diseases
14. CHARD – China Alliance for Rare Disease
15. Child & Youth Care, Zimbabwe
16. CMTC-OVM – Cutis Marmorata Telangiectatica Congenita and Other Vascular Malformations
17. CORD – Canadian Organization for Rare Disorders
18. CORD – Chinese Organization for Rare Disorders
19. CLI – Cutis Laxa International
20. CARD – Cyprus Alliance for Rare Disorders
21. DEBRA International – Dystrophic Epidermolysis Bullosa Research Association
22. EAT – Federation of Esophageal Atresia and Tracheo-Esophageal Fistula Support Groups
23. ESPERANTRA – Esperantra Pacientes con Enfermedades Crónicas no Transmisibles
24. EURORDIS – European Organisation for Rare Diseases
25. FECOER – Federación Colombiana de Enfermedades Raras
26. FADEPOF – Federación Argentina de Enfermedades Poco Frecuentes
27. FEDER – Federación Española de Enfermedades Raras
28. FEMEXER – Federación Mexicana de Enfermedades Raras
29. FEDERG – Federation of European Patient Groups affected by Rare/Genetic Kidney Diseases
30. FIN – Fabry International Network
31. Findacure
32. GASCDO – Global Alliance of Sickle Cell Disease Organisations
33. GAA – Genetic Alliance Australia
34. GSNV – Genetic Support Network of Victoria
35. GlobalSkin – International Alliance of Dermatology Patients Organizations
36. Greek Alliance for Rare Diseases
37. HypoPARA Norge
38. IADPO / GlobalSkin - International Alliance of Dermatology Patient Organizations
39. ICF – Illness Challenge Foundation

40. IFSBH - International Federation for Spina Bifida and Hydrocephalus
41. IGA – International Gaucher Alliance
42. IORD – Indian Organization for Rare Diseases
43. IPOPI – International Patient Organisation for Primary Immunodeficiencies
44. IPWSO – International Prader-Willi Syndrome Organisation
45. INPDA – International Niemann-Pick Disease Alliance
46. Instituto Vidas Raras
47. Japan Patient Association
48. LSPH – Latin Society of Pulmonary Hypertension
49. LePAF – Leukemia Patient Advocates Foundation
50. MRDS - Malaysian Rare Disorders Society
51. MCT8-AHDS Foundation
52. Naevus Global
53. National Alliance for Rare Diseases Support - Malta
54. Nicolaiides Baraitser Syndrome (NCBRS) Worldwide Foundation
55. NORBS - National Organisation for Rare Diseases of Serbia
56. NORD -National Organization for Rare Disorders
57. ORDI - Organization for Rare Diseases India
58. Pancyprian Federation of Patients' Associations and Friends
59. PSOD – Philippine Society for Orphan Disorders
60. Pro Rare Austria
61. RADOIR - Rare Disease Foundation of Iran
62. Rare Diseases Croatia
63. RDGI – Rare Diseases Ghana Initiative
64. RDHK – Rare Disease Hong Kong
65. RDSA – Rare Diseases South Africa
66. Rare Diseases Sweden / Riksförbundet Sällsynta Diagnoser
67. Rare Disorders Kenya
68. RDNZ – Rare Disorders NZ
69. RVA – Rare Voices Australia
70. RI – Retina International
71. RONARD – Romanian National Alliance for Rare Diseases
72. Russian Patients Association
73. TIF – Thalassaemia International Federation
74. The Ehlers-Danlos Society
75. WAPO – World Alliance of Pituitary Organizations
76. WFH – World Federation of Hemophilia



POMPE DISEASE. INDIA



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