



RARE
DISEASES
INTERNATIONAL

OVERVIEW

LEVERAGING GLOBAL POLICIES AND GLOBAL AGENCIES TO EXPLICITELY SUPPORT RARE DISEASES

Yann Le Cam

Chief Executive Officer of EURORDIS - Rare Diseases Europe

Member of the Council of Rare Diseases International

Member ExCo NGO Committee for Rare Diseases

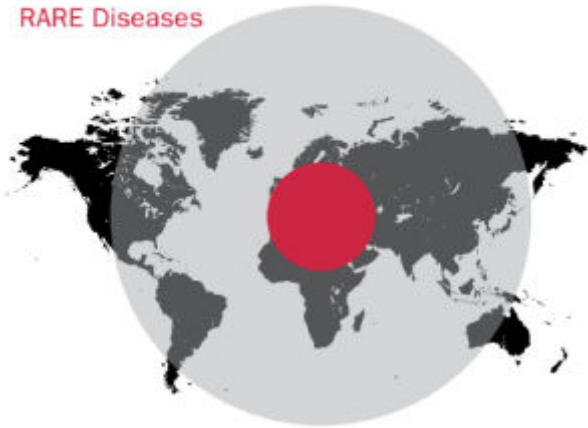
ECRD 2018 Vienna – May 11, 2018

Session 0601 - Global Policies and Agencies to Support Rare Diseases

GLOBAL FIGURES

300 MIO / 7'052 MIO

RARE Diseases



7,000 RARE DISEASES exist, with less than 500 FDA-approved treatments²
ONLY **5%** of RARE DISEASES have treatments.²

Patients with RARE DISEASES are frequently misdiagnosed or undiagnosed.

80% of RARE DISEASES ARE GENETICALLY BASED.²



8%

GLOBAL POPULATION
AFFECTED BY A
RARE DISEASE

3rd

WORLD'S MOST POPULOUS
COUNTRY CONSIDERING ALL THE
PEOPLE WITH RARE DISEASES

<5%

OF RARE
DISEASES HAVE A
TREATMENT

75%

OF THE PEOPLE AFFECTED
BY RARE DISEASES ARE
CHILDREN

RARITY CALLS FOR ACTION AT THE GLOBAL LEVEL

No one country, no one continent, can solve alone the problems posed by rare diseases

- Need to bring together a **critical mass** of patients and medical experts, scientists and public health authorities.
- Need to support:
 - Civil society empowerment at international level
 - International collaboration of researchers and experts
 - Companies acting at global level
 - Countries emerging to rare diseases



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AWARENESS & VISIBILITY

- ▶ People living with rare diseases still remain a **marginalised, vulnerable** and largely **invisible** population
- ▶ With little or no awareness and understanding of the needs of people living with rare diseases, they are often left unmet

Therefore,

- ▶ Need for greater **awareness** and **recognition** of rare diseases as a public health issue
- ▶ Greater **visibility** in healthcare systems (coding and classification – ICD 11)



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DEFINITION OF RARE DISEASES

Need to develop and implement definitions of rare diseases in many countries. This is a pre-condition for both national awareness, knowledge and international comparisons.

- **United States:** less than 7.5 in 10,000 people
(less than 200,000 individuals in the US population)
Population: 318 million
- **EU:** no more than 5 in 10,000 people
(less than 1 in 2,000 citizens)
Population: 507 million
- **Japan:** 1 in 10,000 people
Population: 127 million
- **Russia:** 1 in 10,000 people
Population: 144 million



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NEED FOR MORE ORPHAN DRUG REGULATION

- 1983: USA: The Orphan Drug Act (ODA)
(promoted by the National Organization for Rare Disorders (NORD))
 - 1991: SINGAPORE
 - 1993: JAPAN
 - 1997: AUSTRALIA
 - 1999: EU Regulation on Orphan Drugs
(EURORDIS strongly advocated for this Regulation)
- No OD regulation in most parts of the world



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OPPORTUNITIES TOWARDS GLOBAL POLICIES & STRATEGIES FOR RARE DISEASES

New opportunities arise from:

- UNITED NATIONS SDG 2030 AGENDA
- GLOBALISATION OF THE RARE DISEASE ECOSYSTEM
- GIANT LEAPS IN INFORMATION TECHNOLOGY
- ADVANCES IN MEDICAL SCIENCE & TRANSLATIONAL RESEARCH
- INTERNATIONAL COLLABORATION IN FUNDAMENTAL AND CLINICAL RESEARCH
- SPREADING OF RARE DISEASE POLICY AND LEGISLATION
- BUILDING A GLOBAL PATIENT MOVEMENT



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THE GLOBALISATION OF RARE DISEASES IS UNDERWAY

Research:

- IRDiRC: an International Rare Disease Research Consortium
- Increased budget of the US NIH or EU dedicated to RD Research
- Increasing international networking amongst scientists
- International platforms for rare disease registries
- Development of international platforms for RD patient data sharing, diagnostic, clinical research



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THE GLOBALISATION OF RARE DISEASES IS UNDERWAY

Policy:

- Rare disease policy, strategy and plans implemented nationally, having a 'snowball' effect in other countries
- Incentivizing policies for industry investment e.g. US Orphan Drug Act and EU Orphan Medicinal Products Regulation
- Increased collaboration between agencies (EMA, FDA); EU-WHO Data Sharing Pact
- Health Technology Assessment International



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH



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ACTORS IN THE RARE DISEASE ECO-SYSTEM

These developments are not happening in a vacuum ..more and more international actors are active in the field of rare diseases

► **IRDiRC** : International Rare Disease Research Consortium



► **IFPMA** : International Federation of Pharmaceutical Manufacturers & Associations



► **ORPHANET INTERNATIONAL** : ICD 11 & other International Classifications



► **ISHG**: International Federation of Human Genetics Societies

► **IAPO** : International Alliance of Patients' Organizations



RARE DISEASES INTERNATIONAL

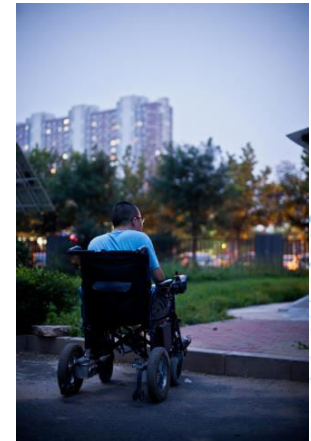
The Global Alliance of people living with a rare disease of all nationalities across all rare diseases.

- Mission:

«To be a strong common voice for rare disease patients across the world»

- Vision:

People living with a rare disease are facing similar challenges irrespective of where they live in the world and thus, share a common interest to address their comparable or specific needs



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GENERAL OBJECTIVES

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



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RDI LAUNCH EVENT MAY 28, 2015 MADRID, SPAIN



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RDI LEGAL INCORPORATION

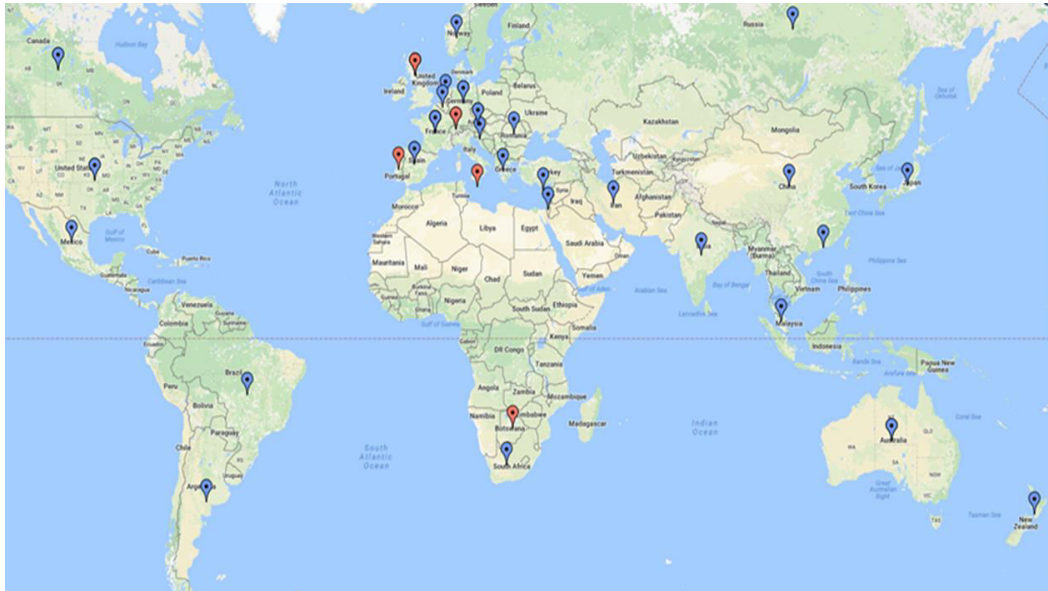
MAY 10, 2018

VIENNA, AUSTRIA



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RDI: NATIONAL AND SPECIFIC-DISEASE PATIENT ORGANISATIONS JOINING FORCES WORLDWIDE



Critical mass is reached with

53 Member Organisations

30 National Alliances

12 International Federations

4 Regional Networks

7 Associate Members

Members based in 34 countries, representing rare disease patients in more than 100 countries worldwide



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JOINT DECLARATION “RARE DISEASES: AN INTERNATIONAL PUBLIC HEALTH PRIORITY”

The patient movement worldwide has adopted the following twelve main recommendations to be addressed at international level as a matter of priority:

Visibility of Rare Diseases

1. Enhance visibility of rare diseases at an international level in terms of public awareness and within the healthcare system as a human rights issue and a public health priority.
2. Better classification and codification of rare diseases can support the process towards better recognition of rare diseases worldwide.
7. Facilitate universal access to high quality healthcare and treatments for rare diseases patients, including surgeries, special diets, transplants, and medical devices, common and orphan medicinal products.
8. Create policies on rare diseases that promote specific measures in regional / national / international strategies, including identification and support of specialised expert providers as well as their national and international networking.

Patient Empowerment

3. Support and empower patients and families to play an active role in shaping national health care provision that is appropriate to their needs.
4. Develop, gather, share and disseminate information on rare diseases in linguistically and culturally appropriate formats.
5. Promote international cooperation in the field of services to patients and families.
9. Promote recognition that rarity requires increased international cooperation and mobility of experts as well as of patients when expertise is not available locally.

Universal Access to Healthcare

6. Improve access to services that will facilitate informed decisions about prevention and screening that are legally permissible, and improve access to accurate and timely diagnoses.

Research

10. Coordinate worldwide research efforts on rare diseases through international and national research initiatives.
11. Build opportunities for effective networking of patient registries.

Cooperation in policy shaping at international level

12. Elaborate policies based on common values (equity, solidarity and Social Justice) that have a positive impact on the lives of rare disease patients.

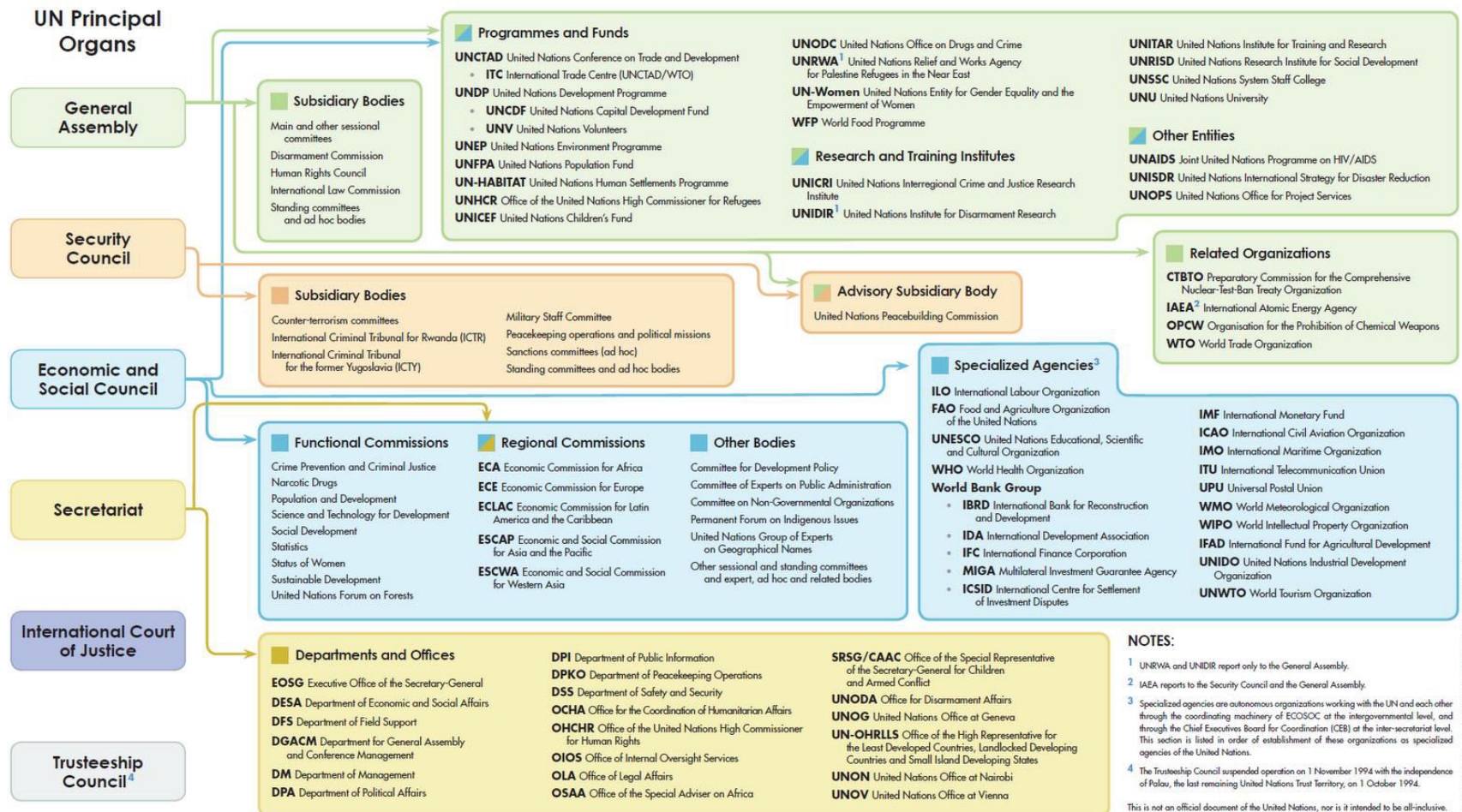


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THE UNITED NATIONS SYSTEM



The United Nations System



STRATEGY TO GAIN RECOGNITION IN THE UN SYSTEM

- Raise RD awareness as a collective health & social issue. Get visibility. Foster understanding.
- Contribute to relevant UN policy – leverage effect on Member States + create background for future resolution
- Be present in the Human Rights sphere
- Be present in the Disability sphere
- Include RD in SDG 2030 Agenda “leave no one behind”
- Universal Health Coverage: “Health for all” (access, equity, health system strengthening)
- Be present in the Access to medicines/ Fair pricing/ IP debate
- Incursion in the NCD agenda – chronic diseases with co-morbidities; protocols of care



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INTER LINKAGES BETWEEN HEALTH AND OTHER AREAS OF LIFE



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THE 2030 SUSTAINABLE DEVELOPMENT AGENDA & RDS

- Provide international policy framework for rare disease policy
- Universal & inclusive: « Leave no one behind »
- Opportunity to address rare diseases from a holistic perspective
- New definition of health: more than the mere absence of disease: Health & well-being
- SDG address the social, economic, and environmental determinants of health
- SDGs recognise that health challenges can no longer be addressed by the health sector acting alone.
- There is a clear alignment between a number of SDGs and Rare Diseases beyond HEALTH



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THE 2030 SUSTAINABLE DEVELOPMENT AGENDA & RDS



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SDGs & RARE DISEASES: SYNERGIES

SDGs

Rare Diseases Challenges



- Vicious cycle of vulnerability and poverty due to exclusion from health care and education systems, as well as job markets



- Universal health coverage = more and better medicines, appropriate diagnosis and lifelong care and social support



- 50% of rare diseases affect children who often face difficulties attending school due to inaccessibility of facilities and non-adapted teaching methods



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SDGs & RARE DISEASES: SYNERGIES

SDGs



Rare Diseases Challenges

- Gender equality = Recognising and valuing unpaid care and domestic work that many mothers of children (including when they are grown adults) with rare diseases take on
- Persons with rare diseases and disabilities are often marginalised from job markets due to lack of accessible facilities, flexible working hours and adapted roles.
- Need to invest in research & development of therapies, health technologies and diagnostic tools
- Accessibility to infrastructure is key to be included in society



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SDGs & RARE DISEASES: SYNERGIES

SDGs

Rare Diseases Challenges



- Marginalised and invisible population suffering from discrimination in the health, labour and governance fields



- The rare disease community is increasingly interconnected, with a myriad of networks of patient advocates, regulators, research & industry



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SDGs & RARE DISEASES: OPPORTUNITIES

- ▶ “Rare diseases have a health & social impact – so they need to be considered and they have a space in the United Nations agenda”

Daniela Bas, DSPD division, DESA



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UNIVERSAL HEALTH COVERAGE: WHAT ABOUT RARE DISEASES?

One of the key commitments under Goal 3, “ensuring healthy lives for all”, is to achieve universal health coverage and provide “access to safe, effective, quality, and affordable essential medicines and vaccines for all”.

The research on and development of health technologies is an important element of universal health coverage.

‘No country can claim to have achieved universal healthcare if it has not adequately and equitably met the needs of those with rare diseases’



Helen Clark, Administrator of the UN Development Programme until April 2017



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HOW TO INFLUENCE THE UN?

- ✓ Through contribution to existing platforms:
 - ✓ Respond to Consultations. Submit contributions to relevant reports and studies
 - ✓ Generate Positions Papers, White Papers, Reflection Papers; ad hoc Statement
- ✓ Through physical presence:
 - ✓ Organise Side Events and Satellite Meetings. Make oral statement at other's events
 - ✓ Advocate Member States to give us political support/ sponsor : Permanent Mission in Geneva and New York +Ministries of Health/ Foreign Affairs
 - ✓ Develop relations with technical staff in relevant institutions



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HOW TO INFLUENCE THE UN?

- ✓ Seek consultative status/ official relations with UN bodies and agencies (ECOSOC, WHO); some members have eg WFH, ISBH, TIF, AGRENSKA ,RADOIR; other members applying; RDI applying
- ✓ Through the NGO Committee for Rare Diseases : bringing together all relevant actors; using capacity to contribute
- ✓ Through collaborative framework: eg WHO, UNICEF,
- ✓ Working toward specific resolutions or inclusion in relevant resolutions and policies
 - ✓ A technical resolutions at WHO
 - ✓ A broader political resolution at UN



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ADVOCACY ACTIONS 2017-2018

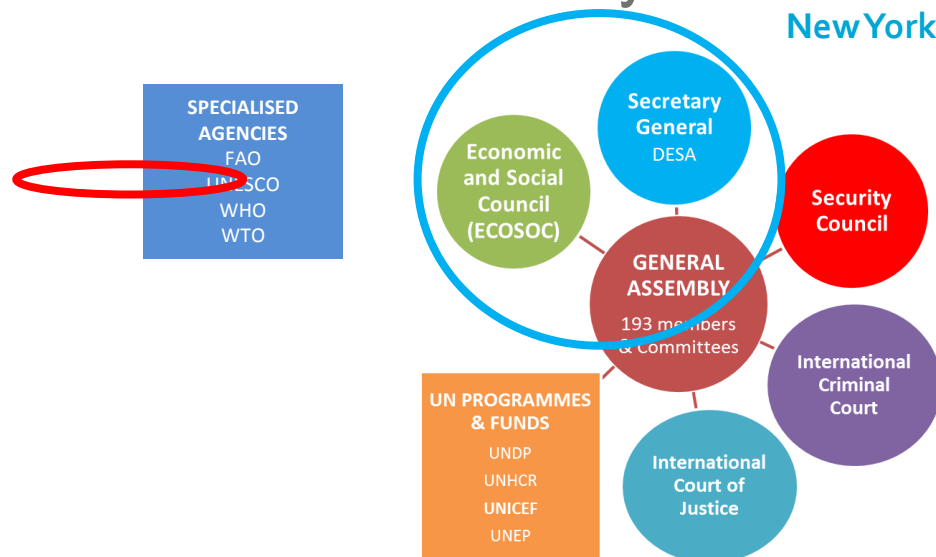
Where?

- General Assembly (Third Committee on Social, Cultural & Humanitarian)
- Conference of States on Convention on the Rights of People with Disabilities
- Human Rights Council
- WHO World Health Assembly

New York

Geneva

Geneva



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NGO COMMITTEE FOR RARE DISEASES

- Committee under the umbrella of Conference of NGOs in Consultative Relationship with the United Nations (CoNGO)
- **Goal:** promote rare diseases as a priority in global health, research, and social and medical care as part of the UN 2030 Agenda: the Sustainable Development Goals (SDGs).
- **Who is involved?**
 - Initiative by Ågrenska Foundation & EURORDIS – Rare Diseases Europe in cooperation with RDI



NGO COMMITTEE FOR
RARE DISEASES



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NGO COMMITTEE FOR RARE DISEASES



NGO COMMITTEE FOR
RARE DISEASES

■ Who is involved?

- Initiative by Ågrenska Foundation & EURORDIS – Rare Diseases Europe in cooperation with RDI
- IAPO, IFSBH, WFH, IWF
- Supported by 34 NGOs from CoNGO
- Forum for interested parties (NGOs, ECOSOC members, UN bodies, individual experts)



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NGO COMMITTEE FOR
RARE DISEASES

11 November 2016

United Nations, New York

The Global Gathering for Rare Diseases

INAUGURATING THE NGO COMMITTEE
FOR RARE DISEASES



LAUNCH OF NGO COMMITTEE FOR RARE DISEASES AT UN HEADQUARTERS IN NEW YORK



NOVEMBER 11, 2016



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Founding Act

New York City, Friday 11 November 2016

Each of the 6,000 repertoried rare diseases affects a very small population locally. **All together, however, they represent an international public health issue.**

The 350 million persons living with a rare disease worldwide are facing **common challenges across diseases and across borders** that affect all parts of their lives at once.

No one country, no one continent alone can solve the problems posed by rare diseases.

Experience shows that actions are possible and effective. **Common national policies and international collaboration can address these challenges.**

Scientific, medical, technological, social opportunities will be high in the next 20 years. **Patients, NGOs and other stakeholders are committed and aligned for partnership.**

Rare diseases fit within the objectives from several UN SDGs  and can significantly contribute to their achievement.



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THE RIGHT TO HEALTH: THE RARE DISEASE PERSPECTIVE

Friday, February 10, 2017

#rdiGeneve



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In partnership and
with the support of:

**BLACKSWAN[®]
FOUNDATION**

BLACKSWAN FOUNDATION FOR RESEARCH IN RARE DISEASES
FONDATION POUR LA RECHERCHE EN MALADIES RARES
FUNDATION FÜR DIE RECHTUNG DER SELTENEN KRAUKHEITEN
FUNDACIÓ PER A LA RECERCA EN MALADIES RARES



FIRST RARE DISEASE POLICY EVENT IN GENEVA



Panelists from WHO, UNDP, IFPMA, OECD, IRDiRC, Orphanet, BLACKSWAN Foundation, patient groups and academic institutions



UN Special Rapporteur on the Right to Health



Co-Chair of UN High-Level Panel on Access to Medicines



FIRST INTERNATIONAL RECOMMENDATION FOR UNDIAGNOSED DISEASE PATIENTS

INTERNATIONAL JOINT RECOMMENDATIONS

TO ADDRESS SPECIFIC NEEDS OF

UNDIAGNOSED RARE DISEASE PATIENTS

October 2016

On behalf of patients living with undiagnosed and rare diseases across Europe, North America, Australia and Japan; SWAN UK (the support group run by Genetic Alliance UK), the Wilhelm Foundation, EURORDIS (Rare Diseases Europe), Rare Voices Australia (RVA), the Canadian Organization for Rare Disorders (CORD), the Advocacy Service for Rare and Intractable Diseases' stakeholders in Japan (ASrid) and the National Organization for Rare Disorders (NORD) jointly submit the following list of recommendations to address the specific needs of patients without a diagnosis. We urge all stakeholders to recognise undiagnosed patients as a specific population within the rare disease community.

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The 5 Recommendations to address specific needs of undiagnosed rare disease patients

1. **Undiagnosed Rare Disease Patients should be recognised as a distinct population** with specific unmet needs by national authorities to enable development of personalised health and social care. Although some undiagnosed diseases are common, the vast majority are rare. Hence, in this paper we refer to undiagnosed patients as "undiagnosed rare disease patients".
2. **National sustainable programmes** dedicated specifically for undiagnosed diseases should be developed and supported by appropriate authorities in each country to enable rapid and equitable access to diagnosis and social support.
3. **Knowledge and Information sharing should be structured and coordinated** at national and international levels to optimise use of existing resources and facilitate access for all undiagnosed rare disease patients.
4. **Patients should be equally involved** with other stakeholders in the governance of undiagnosed diseases programmes and international networks to adequately address the priorities of undiagnosed rare disease patients and contribute to improved healthcare.
5. **Ethical and responsible international data sharing should be promoted** through existing initiatives to support diagnosis, increase clinical collaboration, facilitate research, and accelerate treatment of undiagnosed and rare conditions.

- SWAN UK
- the Wilhelm Foundation
- EURORDIS (Rare Diseases Europe)
- Rare Voices Australia (RVA)
- Canadian Organization for Rare Disorders (CORD), the Advocacy Service for Rare and Intractable Diseases' stakeholders in Japan (ASrid)
- National Organization for Rare Disorders (NORD)



THE RIGHT TO HEALTH: INTRODUCING THE RARE DISEASES PERSPECTIVE

Report commissioned to the Office of the High Commissioner for Human Rights (OHCHR) through [Resolution 35/23](#) of the Human Rights Council (June 2017) asking to collect and present “contributions of the right to health framework to the effective implementation and achievement of the health-related SDGs, identifying best practices, challenges and obstacles thereto”

Report drafted by office of Deputy High Commissioner
Ms. Kate Gilmore, Australia, 2015)



To be presented at the June HRC 2018 (38th session)



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THE RIGHT TO HEALTH: INTRODUCING THE RARE DISEASES PERSPECTIVE

- ▶ Title of contribution: *The Right to Health in Rare Diseases: A Practical Contribution to Implementing and Achieving the SDGs*

- ▶ Submission by NGO Committee for RDs -
Deadline: Friday 9th, 2018
- ▶ Ad hoc Group: led by Jean-Louis Roux and Agrenska

Post-Submission action:

- ▶ Statement or side event at Human Rights 38th session in Geneva – 18 June to 6 July 2018

Submission by the



to the United Nations High Commissioner for Human Rights
for your report to the Human Rights Council at its 38th session on "contributions of the right to health framework to the effective implementation and achievement of the health-related Sustainable Development Goals"

The "Right to Health" in Rare Diseases:

A Practical Contribution to Implementing and Achieving the Sustainable Development Goals

1 On behalf of the NGO Committee for Rare Diseases¹, a substantive committee established under the
2 umbrella of the Conference of NGOs in Consultative Relationship with the United Nations (CoNGO) to share
3 knowledge about rare diseases and raise their visibility on the political stage globally, we welcome the
4 opportunity to provide input on the relationship between rare diseases and the Sustainable Development
5 Goals in a human rights-based framework.
6 Resolution 35/23, adopted by the Human Rights Council on 23 June 2017, enshrines "the right of
7 everyone to the enjoyment of the highest attainable standard of physical and mental health" not only
8 as a central component of the 2030 Agenda for Sustainable Development, but also as an essential
9 instrument for its actual implementation.
10 Health intersects in a structural way several of the Sustainable Development Goals, well beyond SDG
11 3 only. To highlight but a few, poor health can very frequently compromise access to "quality education"
12 (SDG 4) and to "decent work" (SDG 8), thus opening up the risk of a vicious and self-sustaining spiral
13 leading to inequalities (SDG 10), exclusion and discrimination, particularly against women and girls (SDG 5),
14 and ultimately poverty (SDG 1). In other words, the highest attainable standard of physical and mental
15 health represents the foundational bedrock without which an individual cannot be genuinely enabled
16 to set forth on any pursuit of life, be it towards personal, intellectual, economic or social fulfillment. Only
17 by upholding human rights "to health and through health", will the delivery of the SDGs indeed "leave no one
18 behind".
19 Such considerations are of a particularly acute relevance in the field of rare diseases, which gather
20 well over 6,000 conditions, often of genetic origin but also correlated to other types of infectious or
21 environmental agents, and which affect small to ultra-small populations of patients (typically fewer than 1 in
22 2,000 individuals). Most rare diseases are chronic, progressive, degenerative, disabling and frequently life-
23 threatening.

¹ For further information, please refer to www.associationrare.org. Please see www.associationrare.org for the list of members and of our mission. Executive Board meeting 11 Nov. 2017.

² Report of the High-Level Working Group on the Health and Human Rights of Women, Children and Adolescents (2017), "Leading the Realization of Human Rights to Health and through Health", Joint World Health Organization/Available at: www.who.int/publications-detail/leading-the-realization-of-human-rights-to-health-and-through-health, processed 1 Feb. 2018.



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BRINGING CLOSER DISABILITY AND RARE DISEASES?

Call for contributions to the **Report of the UN Rapporteur on the Rights of People with Disabilities** (Ms. Catalina Devandas) on 'the right of persons with disabilities to the highest attainable standard of health'.



Every year the Special Rapporteurs must report to the Human Rights Council (HRC) and to the General Assembly (GA).

Report will be presented at General Assembly 73rd session - Third Committee – October 2018)

Deadline for contributions: March 30, 2018 .

Submitted by NGO CfRD with mention of all members including RDI

Ad Hoc Group: Led by Annette Dumas and Raquel Castro Social Policy Unit EURORDIS and Agrenska



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Angle: Most people living with a rare disease are living with disability + often multiple-disabilities + health challenges for PLWRD

- Special Rapporteur expert group meeting to inform the report (15 & 16 May, Geneva)
- Statement at Conference of States parties to Convention on the Rights of People with disabilities in New York – 12 to 14 June 2018



FITTING RARE DISEASES IN THE WHO STRATEGY 2019-2023

- ✓ RDI Response to Public Consultation on *13th General Programme of Work (2019-2023)* – October 2017
- ✓ RDI Contribution to Draft GPW 13 – November 2017

No *direct* reference to Rare Diseases

Strategic priorities of WHO:

- Prevent, detect, and respond to epidemics
- Emergencies: provide health services and strengthen health systems
- **Help countries to achieve universal health coverage**
- **Lead on health related SDGs ← “leave no one behind”**
- **Provide the world’s platform for collective decision-making in health ←**



**World Health
Organization**



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FITTING RARE DISEASES IN THE WHO STRATEGY 2019-2023

Within Strategic priority 1: health coverage - *one billion more people with health coverage*

► Universal Health Coverage

- Access to affordable, quality-assured health services
- Quality assurance of products through effective regulation, HTA and Fair Pricing
- Pro equity position: include vulnerable incl. Disability and Rare



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FITTING RARE DISEASES IN THE WHO STRATEGY 2019-2023

Within Strategic Priority 3: Health priorities - *one billion lives improved*

► NCD Agenda

- Improving lives despite prevalence
- Include genetic and inherited in NCDs, not only reducing environmental risk factors

► Children's health

- Reduce Under 5 mortality rates by including rare congenital diseases
- Newborn Screening



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FITTING RARE DISEASES IN THE WHO STRATEGY 2019-2023

Within Strategic Priority 3: Health priorities - *one billion lives improved*

► Data Collection

- Collect, pool, share FAIR data
- Coding & Classification (ICD-11)
- Patient registration (link to health care services)

► Fostering Access & Innovation

- Incentives
- WHO Leadership role / Catalyst for development of low cost medicines



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DRAFT FRAMEWORK OF COLLABORATION WITH WHO

Proposal includes:

- Awareness: *Rare Disease Day* Official WHO Day
 - Visibility: Coding & Classification (ICD-11)
 - Consensus on Definition
 - Networking of Clinical expertise
 - Expand healthcare pathways and guidelines
 - Access to medicines
 - Access to medical devices and assistive technology
 - National Plans and Strategies for RD
- Next step: submit proposal formally (after RDI Membership Meeting) + Discussion meeting at WHO



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Tedros Adhanom Ghebreyesus ✓

@DrTedros

Following



We are working for a world where no one is left behind. On [#RareDiseaseDay](#), 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



Statement for Rare Disease Day

The vision of the Sustainable Development Goals is a world in which no one is left behind, including people who suffer from rare diseases. Just because a disease affects a small number of pe...
[who.int](#)

8:41 AM - 28 Feb 2018

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