



RARE
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ADVOCATING FOR RARE DISEASES IN THE UNITED NATIONS SYSTEM

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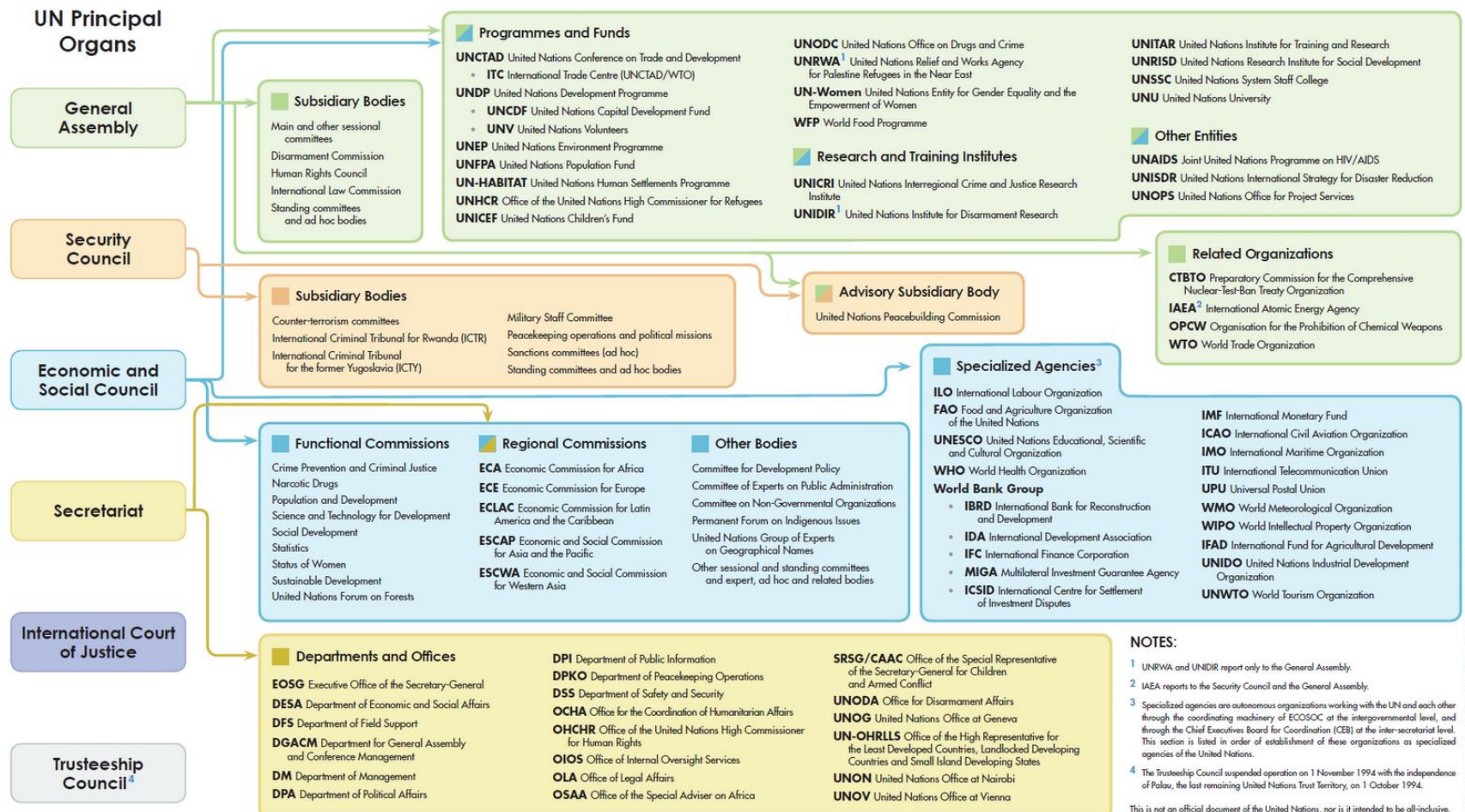
Member of the Council of Rare Diseases International

Member of the Inception Executive of NGO Committee on Rare Diseases

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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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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INTER LINKAGES BETWEEN HEALTH AND OTHER AREAS OF LIFE



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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
- ✓ Seek consultative status/ official relations with UN bodies and agencies (ECOSOC, WHO)



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ADVOCACY ACTIONS 2017- 2018

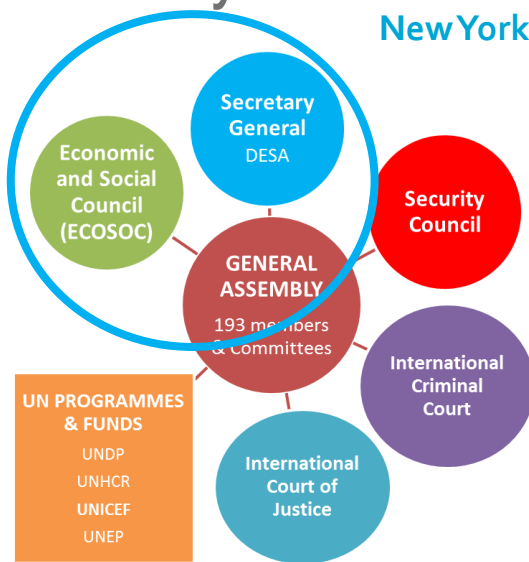
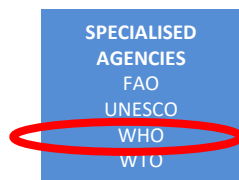
Where?

- 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



New York



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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 fulfilment. 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 and vision statements (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 (Accessed 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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BRINGING CLOSER DISABILITY AND RARE DISEASES?

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

Post-Submission action:

- 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 Persons with Disabilities in New York

Factsheet

Most People Living with a Rare Disease are Living with Disability

"Disability is an umbrella term, covering impairments, activity limitations, and participation restrictions. An impairment is a problem in body function or structure, an activity limitation is a difficulty encountered by an individual in executing a task or action, while a participation restriction is a problem experienced by an individual in involvement in life situations" [1, WHO].

"Disability is a universal human experience, in the sense that everyone can be placed on a continuum of functioning and either currently experiences or is vulnerable to experiencing disability over the course of their lives. This understanding of disability is the key to mainstreaming disability within the public discourse" [2].

Key policy references:

- The Commission Expert Group on Rare Diseases [Recommendations to support the integration of rare diseases into social services and policies](#), adopted unanimously by all Member States in 2016 [3], state that "rare diseases are often chronic, highly complex, severely disabling and frequently affect life expectancy" and recommend that "rare disease specificities should be integrated into national systems assessing a person's level of functioning, in line with the United Nations Convention on the Rights of Persons with Disabilities.
- The European Rare Disease Task Force, in 2008 [4], underlined that rare diseases "all together account for a big part of the early-life deaths and life-long disabilities in the European population".

Results of European survey on the social impact of rare diseases:

The survey "Assessing care and daily life: The balancing act of the rare disease community" [5a] shows that rare diseases severely affect the everyday life of people living with a rare disease and their careers.

Rare diseases are disabling, limiting several Activities of Daily Living (ADLs)



- Rare diseases disabling aspects are often invisible and may vary in time due to the fluctuation of symptoms.

"The difficulty lies in the impossibility of carrying a routine (...). The problem arises when one day you appear completely healthy, the next day you are sick, and two days later you appear completely normal again. Many people find it difficult to understand the disease and the process, and the absence of that entails". Female, Spain

- 50% of people living with a rare disease have uncovered needs regarding the access to disability benefits:
 - 28% of respondents expressed that they have access to disability benefits but not enough to cover their needs;

Submission by the



to the United Nations Special Rapporteur on the Rights of People with Disabilities for the study on "the right of persons with disabilities to the highest attainable standard of health" to be presented at the 73rd session of the General Assembly (October 2018).

Word count: 3,484

On behalf of the NGO Committee for Rare Diseases¹, a substantive committee established under the umbrella of the Conference of NGOs in Consultative Relationship with the United Nations (CONGO) to share knowledge about rare diseases (RDs) and raise their visibility on the political stage globally, we welcome the opportunity to provide input from the RD perspective on the right of persons with disabilities to the highest attainable standard of health.

The governance of the NGO Committee for Rare Diseases is led by the current members of the Reception Executive Board: [Aegionia](#), [EURODIS](#), [Rare Diseases Europe](#), [International Alliance of Patients' Organizations](#), [International Alliance of Women](#), [International Federation for Gene Ethics and Hydrocephalus](#), [World Federation of Hemophilia](#), the global patient voice being represented through [Rare Diseases International](#) (RDI), the global alliance of people living with a RD of all nationalities across all RDs.

Most people living with a rare disease are living with disability.

The WHO's definition of "disability", which reflects into the International Classification of Functioning and Disability, states that as an umbrella term, it covers "impairments (problems in body function or structure), activity limitations (difficulties in executing a task or action), and participation restrictions (problems experienced by an individual in involvement in life situations)".

Disability is a universal human experience in which everyone can be placed in a continuum of functioning and can be vulnerable to experiencing disability over the course of their lives². This rethinking of disability is the key to mainstreaming disability within the public discourse.

RDs are often chronic, highly complex, progressive and severely disabling, frequently affecting life expectancy and generating specific care needs³. Therefore, in line with the three WHO core principles of disability it is possible to explicitly highlight how RDs are highly disabling and thus most people living with RDs are also living with disability.

¹ For further information, please refer to www.ngocommitteeforrare.org

www.ngocommitteeforrare.org/secretariat

www.ngocommitteeforrare.org/secretariat/secretariat-board/ for details of the Committee's members and of our Reception Executive Board formed in Nov. 2017.

www.ngocommitteeforrare.org



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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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