ADVOCATING FOR RARE DISEASES IN THE UNITED NATIONS SYSTEM

Yann Le Cam
Chief Executive Officer of EURORDIS - Rare Diseases Europe
Member of the Council of Rare Diseases International
Member of the Inception Executive of NGO Committee on Rare Diseases
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
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
THE 2030 SUSTAINABLE
DEVELOPMENT AGENDA & RDS

1. NO POVERTY
2. GOOD HEALTH AND WELL-BEING
3. QUALITY EDUCATION
4. GENDER EQUALITY
5. REDUCED INEQUALITIES
6. PARTNERSHIPS FOR THE GOALS
INTER LINKAGES BETWEEN HEALTH AND OTHER AREAS OF LIFE
SDGs & RARE DISEASES: SYNERGIES

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<th>SDGs</th>
<th>Rare Diseases Challenges</th>
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<tr>
<td><strong>1. No Poverty</strong></td>
<td>Vicious cycle of vulnerability and poverty due to exclusion from health care and education systems, as well as job markets</td>
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<td><strong>3. Good Health and Well-being</strong></td>
<td>Universal health coverage = more and better medicines, appropriate diagnosis and lifelong care and social support</td>
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<td><strong>4. Quality Education</strong></td>
<td>50% of rare diseases affect children who often face difficulties attending school due to inaccessibility of facilities and non-adapted teaching methods</td>
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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.
SDGs & RARE DISEASES: SYNERGIES

SDGs Rare Diseases Challenges

10 Reduced Inequalities

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

17 Partnerships for the Goals

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

Post-Submission action:

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


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
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 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 ←
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
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
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
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
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 people... who.int

8:41 AM - 28 Feb 2018

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