HOPES AND CHALLENGES OF PEOPLE LIVING WITH A RARE DISEASE WITHIN UNIVERSAL HEALTH COVERAGE

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RARE DISEASES

OVER 6000 distinct rare diseases

Each one affects fewer than 1 IN 2000 PEOPLE

Affects approx. 5% of the population in the course of their lives

NO CURE for the vast majority of diseases and few treatments available

All together, an estimated 25/30 MILLION PEOPLE are living with a rare disease in Europe and 300 MILLION worldwide
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 countries emerging to rare diseases and promote **international collaboration** between researchers, medical experts and other stakeholders worldwide

• **Civil society empowerment** at international level
THE CHALLENGES

Most rare diseases are chronic, progressive, degenerative, disabling and frequently life-threatening

• Patients and experts are few, geographically scattered and often isolated
• Patients are undiagnosed, misdiagnosed or wait years for a diagnosis. Highly heterogeneous diseases
• Reliable information is scarce
• Fragmented research, data and expertise. Lack of treatments: difficulties to access adequate care
• High social impact and marginalisation within society at large and within healthcare systems designed for common diseases
• Heavy psychosocial burden
• Commonalities of issues and critical mass enables common policies across diseases
• New opportunities in 21st century
• Scientific advances and translational research
• New technologies: digital, AI, 3D, nano...
• Global networking
• Translate into more and earlier diagnosis; better care and innovative treatments for patients; improved patient health outcomes and quality of life
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.

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.

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.

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.

9. Promote recognition that rarity requires increased international cooperation and mobility of experts as well as of patients when expertise is not available locally.

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.
The Global Gathering for Rare Diseases

Inaugurating the NGO Committee for Rare Diseases

11 November 2016
United Nations, New York
2016: LAUNCH OF NGO COMMITTEE FOR RARE DISEASES AT THE UNITED NATIONS IN NEW YORK

November 11, 2016
A MILESTONE IN THE JOURNEY TO PUT RARE DISEASES IN THE GLOBAL AGENDA

Founding Act
« Rare Diseases and the UN Sustainable Development Goals »
New York City, Friday 11 November 2016

We, the undersigned participants to the inaugural « Global Gathering for Rare Diseases », proclaim and endorse the following statements:

- Each of the 6,000 reported 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.

The NGO Committee for Rare Diseases will catalyse all efforts towards the delivery of the SDGs in support of rare diseases, and towards the recognition and integration of rare diseases in all relevant future global policy and initiatives of the United Nations and its agencies.
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<th>SDGs</th>
<th>Rare Disease Challenges</th>
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<tr>
<td>No Poverty</td>
<td>Persons with a rare disease are often trapped in a vicious cycle of vulnerability and</td>
<td>Decent Work and Economic</td>
<td>Persons with a rare disease and disabilities are often marginalised from job markets</td>
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<td>poverty due to exclusion from health care and education systems, as well as job</td>
<td>Growth</td>
<td>due to lack of accessible facilities, flexible working hours and adapted roles</td>
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<td>Good Health and</td>
<td>Persons with a rare disease need more and better medicines, appropriate diagnosis and</td>
<td>Industry, Innovation and</td>
<td>There is a need to invest in research &amp; development of therapies, health technologies</td>
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<td>Well-being</td>
<td>lifelong care and social support</td>
<td>Infrastructure</td>
<td>and diagnostic tools</td>
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<td>Quality Education</td>
<td>50% of rare diseases affect children who often face difficulties attending school due</td>
<td>Reduced Inequalities</td>
<td>Accessibility to infrastructure is key to be included in society</td>
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<td>Gender Equality</td>
<td>to inaccessibility of facilities and non-adapted teaching methods</td>
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<td>Persons with a rare disease tend to remain a marginalised population suffering from</td>
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<td>Gender equality means recognising and valuing unpaid care and domestic work that many</td>
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<td>discrimination in the health, labour and governance fields</td>
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<td>mothers of children with rare diseases (including when they are grown adults) take on</td>
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<td>The rare disease community is increasingly interconnected, with a myriad of networks</td>
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<td>of patient advocates, regulators, research &amp; industry</td>
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2017: 1ST RARE DISEASE POLICY EVENT IN GENEVA

THE RIGHT TO HEALTH: THE RARE DISEASE PERSPECTIVE
Friday, February 10, 2017
#rdiGeneve
2019: RARE DISEASE DAY POLICY EVENT AT THE UNITED NATIONS

• Second High-Level Event of the NGO Committee for Rare Diseases, 21 February 2019, United Nations Headquarters, New York
2019: RARE DISEASE DAY POLICY EVENT AT THE UNITED NATIONS
The Rare Disease Community Position

RDI Position Paper released in April 2019

- RD and UHC movements share important commonalities in spirit and vision
- UHC shall never be fully attained nor realised if persons living with rare diseases are left behind and their needs left unmet.
- The time to integrate rare diseases in the reflection on, and practice of, Universal Health Coverage is now.
International Universal Health Coverage Day
12 December 2018, United Nations Headquarters, New York

RDI was invited to participate in a Discussion Panel organised by WHO and Group of Friends of UHC (Japan, Brazil, France, Ghana, Hungary, South Africa, Thailand) at United Nations in New York. RDI was the only civil society organisation represented in the panel.

Durhane Wong-Rieger, Chair of the Council of RDI addressed dignitaries and delegates.
KEY ASKS FROM THE UHC MOVEMENT FOR THE UN HIGH-LEVEL MEETING ON UHC

• ASK 1: Ensure political leadership beyond health – Commit to achieve UHC for healthy lives and wellbeing for all at all stages, as a social contract.

• ASK 2: Leave no one behind – Pursue equity in access to quality health services with financial protection.

• ASK 3: Regulate and legislate – Create a strong, enabling regulatory and legal environment responsive to people’s needs.

• ASK 4: Uphold quality of care – Build quality health systems that people and communities trust.

• ASK 5: Invest more, invest better – Sustain public financing and harmonize health investments.

• ASK 6: Move together – Establish multi-stakeholder mechanisms for engaging the whole of society for a healthier world.
THANK YOU!