



NGO COMMITTEE FOR  
**RARE DISEASES**

## Second High-Level Event

of the

**NGO Committee for Rare Diseases**



**RARE DISEASE DAY®**

### **Rare Disease Day**

### **Policy Event**

### **at the United Nations**

**Thursday 21 February 2019**

Conference Room 8 (GA-1B-CR08)

United Nations, New York (NY 10017)

#### **About rare diseases and Rare Disease Day**

There are over 6,000 identified rare diseases. Even though one disease may be rare, the number of people affected by rare diseases is extremely large, with an estimated 300 million worldwide. Rare diseases are often chronic, highly complex, progressive and severely disabling, frequently affecting life expectancy and generating specific care needs.

While rare diseases share the impacts and challenges of common severe conditions, because each affects very small numbers scattered across the globe, they are often misdiagnosed or undiagnosed and most persons affected are not referred to appropriate specialists. In addition, rare diseases receive little research attention and funding, resulting in limited knowledge about causes, natural progression, and effective intervention. In fact, fewer than 5% of diseases have any known treatment. Furthermore, rare diseases impact not only a person's health, but also their socio-economic status, family, education and labour opportunities. Difficulties such as poverty, unemployment, stigmatisation and social exclusion are in fact a daily reality for most people affected.

#### **A rare disease affects...**



HEALTH

FAMILY

EDUCATION

WORK

ECONOMY

SOCIETY

SCIENCE

These struggles are shared across many different diseases and geographies making rare diseases a global policy priority demanding common solutions within the Sustainable Development Goals Agenda. Persons living with a rare disease need to be seen as more than just patients or their disease, but as human beings with rights to health and well-being.

[Rare Disease Day](#) is an international initiative to raise awareness about rare diseases and their impact on people's lives. Over the past 10 years, Rare Disease Day has become an internationally recognised awareness-raising campaign, with events taking place in over 90 countries and regions, uniting millions of people living with a rare disease worldwide.

#### **About the NGO Committee for Rare Diseases**

This substantive committee, established under the umbrella of the Conference of NGOs in Consultative Relationship with the United Nations ([CoNGO](#)), aims to promote multi-stakeholder collaboration and actions for rare diseases within the United Nations system. For more detailed information on its activities, you may consult this [presentation](#).

The Committee acts as a forum of interested parties such as NGOs from the field of rare diseases and beyond; United Nations bodies and agencies; as well as individual experts. The governance of the Committee is led by the current members of the Inception Executive Board: [Ågrenska](#), [EURORDIS-Rare Diseases Europe](#), [International Alliance of Patients' Organizations](#), [International Alliance of Women](#), [International Federation for Spina Bifida and Hydrocephalus](#), [Word Federation of Hemophilia](#), and [Rare Diseases International](#) (RDI), the global alliance of people living with a rare disease of all nationalities across all rare diseases.



## About the Second High-Level Event of the NGO Committee for Rare Diseases

This event forms part of the broad strategy of the NGO Committee to work towards the achievement of the relevant Sustainable Development Goals that will contribute to better lives for the community of people living with a rare disease. It aims to present the progress achieved in the last 2 years within the human rights, health, and sustainable development agendas of the United Nations system as well as present and discuss a call towards a General Assembly Resolution on rare diseases.

The meeting will take the form of an all-day conference bringing together around 100 participants – from the international NGO community, UN agencies and national governments, academic and research institutions as well as the private sector – interested in collaborating towards the advancement of rare diseases as a global public health priority within the United Nations.

The call to integrate rare diseases within the UN Agenda was first issued at the first high-level event, which took place on November 11th 2016 at the United Nations in New York under the title 'Global Gathering for Rare Diseases'. You may refer to the [report](#) of this event. On that occasion, the link between the rare diseases community's goals and the UN SDGs 2030 Agenda was established and recognised by the main UN agencies and bodies (WHO, UNICEF, UNDP, ECOSOC), which opened the door to broader awareness of the issue and potential partnerships at global level.

### Preliminary Outline of the agenda

#### Morning Plenary: 10:00 to 13:30

##### 1. Keynote Addresses: Leave no person living with a rare disease behind

##### 2. Recognising rare diseases as a policy priority: Empowering people living with a rare disease and ensuring equality

*This session aims to give a clear overall view of the developments that the rare disease community has made in the last two decades (in public awareness, healthcare systems, support systems and in research), with the particular goal of demonstrating the need to consider rare diseases as a collective group in need of a specific holistic strategy.*

##### 3. Universal Health Coverage and reduction of inequalities for inclusive development

*This session aims to highlight public health policy priorities for persons living with a rare disease that are essential for the achievement of Universal Health Coverage (UHC) and efficient and equitable health care systems. It also aims to demonstrate the importance of looking beyond health when addressing the needs of persons living with a rare disease as they often have to visit different health, social and local services in a short period of time and interact with actors that work in silos. Throughout the sub-sessions, speakers will demonstrate the need for and the benefits arising from policies related to diagnostic, cross-border and cross-sector collaboration, and use of emerging technologies, as well as measures that are multidisciplinary, holistic, continuous, person-centred and participative in nature.*

#### Afternoon Plenary: 14:30 to 18:00

##### 4. Ensuring inclusion and participation of people living with a rare disease through national policies and strategies

*This session aims to showcase national strategies, case-studies and pilots that have been put in place in a number of Member States in different areas (such as research, access to health care and support systems) particularly when carried in collaboration with organisations of people living with a rare disease and/or with government support. These illustrate the benefit that comes to citizens when governments establish specific strategies for people living with a rare disease, as well as the progress towards inclusive and equitable societies these imply for Member States.*

##### 5. Closing Session: Call for action on rare diseases to the Members of the United Nations