
The NGO Committee for Rare Diseases is a substantive committee established under the umbrella of the Conference of NGOs in Consultative Relationship with the United Nations (CoNGO).

The NGO Committee for Rare Diseases was initiated by the Agrenska Foundation and EURORDIS-Rare Diseases Europe, with the later integration of Rare Diseases International (RDI), with a view to bringing greater political recognition of the challenges of rare diseases at the global level. Its formation was approved by a vote of 27 CoNGO member organisations in April 2014, and its official inauguration at the United Nations took place on 11 November 2016 in New York.

Under the patronage of HRH The Grand Duchess of Luxembourg

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POLICY EVENT AT THE UNITED NATIONS

Second High Level Event of the NGO Committee for Rare Diseases

Conference Room 8 (GA-1B-CR08), United Nations, New York (NY 10017)
Morning Plenary: 9:45 to 13:30

KEYNOTE ADDRESSES
9:45-11:00

1. The NGO Committee for Rare Diseases: working on a common cause at the global level
   Mr. Anders Olauson, Chair of the NGO Committee for Rare Diseases; Chairman of Ågrenska Foundation

2. Remarks by the Host
   H.E. Mr. Sven Jürgenson, Ambassador and Permanent Representative, Permanent Mission of Estonia to the United Nations

3. Rare Diseases and the Sustainable Development Goals
   Ms. Marion Barthelemy, Director, Office of Intergovernmental Support and Coordination for Sustainable Development, Economic and Social Council (ECOSOC)

4. Human Rights of people living with a rare disease
   Mr. Andrew Gilmour, Assistant Secretary-General for Human Rights and Head of the Office of the High Commissioner for Human Rights in New York

5. Remarks by Chair of the UN Group of Friends of Universal Health Coverage
   H.E. Toshiya Hoshino, Ambassador and Deputy Permanent Representative, Permanent Mission of Japan to the United Nations

6. Universal Health Coverage and Rare Diseases knowledge improvement: a journey to leave no one behind
   Ms. Pilar Aparicio, Director General of Public Health, Quality and Innovation at the Ministry of Health, Consumption and Social Welfare of Spain

7. Universal Health Coverage for people living with a rare disease
   H.E. Vitavas Srivihok, Ambassador and Permanent Representative, Permanent Mission of Thailand to the United Nations

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 grouping rather than looking solely at each individual disease. As people living with a rare disease face a number of common needs and challenges, they demand a specific holistic strategy and are an example of traditionally disadvantaged social groups, with the added specificity of the ‘rarity’. This clearly demonstrate the added-value of collaborating across diseases/conditions, and across countries.

SESSION 2
11:00-12:00

RECOGNISING RARE DISEASES AS A POLICY PRIORITY: EMPOWERING PEOPLE LIVING WITH A RARE DISEASE

8. Raising awareness in society at the global level
   Ms. Avril Daly, Chief Executive Officer, Retina International, Vice-President of the Board of Directors, EURORDIS-Rare Diseases Europe

9. Giving existence to people living with a rare disease in health systems
   Ms. Ana Rath, Director, Orphanet

10. A need for visibility within support systems
    Mr. Lieven Bauwens, Secretary General, International Federation for Spina Bifida and Hydrocephalus; Member of Inception Executive Board NGO Committee for Rare Diseases

11. Inclusion of rare diseases in scientific research
    Dr. Irene Norstedt, Acting Director responsible for the Health Directorate within the Directorate-General for Research and Innovation, European Commission and Head of Unit, Innovative and Personalised Medicine Unit; Member of the International Rare Diseases Research Consortium (IRDiRC)
SESSION 3
12:15-13:30

UNIVERSAL HEALTH COVERAGE AND REDUCTION OF INEQUALITIES FOR INCLUSIVE DEVELOPMENT

This session aims to highlight 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. Persons affected often suffer from marginalisation and pauperisation due to limited or scattered expertise, lack of diagnosis or misdiagnosis, and disproportionate out-of-pocket spending on health. But in addition, people living with a rare disease are often lost in the system, having to visit different health, social and local services in a short period of time and interacting 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. Sub-session 3 will particularly highlight the importance of thinking beyond health systems solely and promoting measures that are multidisciplinary, holistic, continuous, person-centred and participative in nature. Such policies will significantly reduce the burden on everyday life, prevent discrimination and stigma, and contribute to the enjoyment of fundamental rights and to the fulfilment of the full potential of persons living with a rare disease as pledged by Member States under the UN 2030 SDGs agenda.

Co-Chairs:
+ Dr. Nata Menabde, World Health Organisation NYC Office Director
+ Mr. Alain Weill, President of World Hemophilia Federation and member of board of NGO Committee for Rare Diseases

3.1 DIAGNOSIS AS A DOOR-OPENER

12 The extreme of ‘Leave no one behind’ – undiagnosed patients
Dr. William Gahl, Chair, Undiagnosed Diseases Network International (UDNI); Clinical Director, National Institutes of Health, National Human Genome Research Institute

13 New opportunities to improve the diagnosis of children living with a rare disease
Dr. Simon Kos, Chief Medical Officer and Senior Director, Worldwide Health, Microsoft & member of the Global Commission on ending the Diagnostic Odyssey of Children with Rare Diseases

3.2 LOCAL HEALTHCARE PROVISION AND GLOBAL NETWORKING

14 Rare Diseases as an opportunity for global collaboration
Dr. Ruudiger Krech, Director, Universal Health Coverage and Health Systems, World Health Organisation

15 Case-Study: European Reference Networks
Mr. Martin Seychell, Deputy Director-General for Health and Food Safety, DG SANTE, European Commission

3.3 HOLISTIC APPROACH: BRIDGING HEALTH AND SOCIAL CARE

16 The need to address the ‘Big 5’ from a lifelong perspective: healthcare, social care, school, insurance and labour
Mr. Robert Hejdenberg, Chief Executive Officer, Ågrenska Foundation

Lunch Break 13:30 to 14:30

Buffet to be served by the stairs behind the left side of the Vienna Café
This session aims to showcase national strategies, case-studies and pilots put in place in a number of Member States and carried in collaboration with organisations of people living with a rare disease and/or with government support. These illustrate the benefits that ensue when specific national strategies for rare diseases are established and how they contribute towards inclusive and equitable societies.

Co-Chairs:
+ Ms. Daniela Bas, Director of Division for Inclusive Social Development, UN Department of Economic and Social Affairs (DESA), United Nations Secretariat
+ Ms. Durhane Wong-Rieger, President and CEO, Canadian Organization for Rare Disorders (CORD); Chair of the Council, Rare Diseases International (RDI)

4.1 NATIONAL COMPREHENSIVE STRATEGIES

17 Nan-Byo Strategy – Japan
Mr. Toshi Eoze, Counsellor, Permanent Mission of Japan to the United Nations

18 National plan for Rare diseases - France

19 Implementing a national rare disease plan starting with national registry – Colombia
Mr. Germán Escobar Morales, Director of Health, ProPacifico

20 Establishing an APEC Rare Disease Registry Network: QUT and a framework for collaboration
Prof. Matthew Bellgard, Director of eResearch, Division of Research and Innovation, Queensland University of Technology and Chair of APEC LSIF Rare Disease Network

4.2 INTEGRATED APPROACH BETWEEN RESEARCH AND CARE; AND BETWEEN MEDICAL AND SOCIAL CARE

21 Networks for applying research to diagnosis and care – Canada
Dr. Kym Boycott, Professor of Pediatrics, University of Ottawa; Care4Rare

22 Rare Diseases Clinical Research Networks – United States of America
Dr. Marshall Summar, Director, Rare Disease Institute at Children’s National, Washington, D.C; Chairman of Board of Directors of the National Organization for Rare Disorders (NORD), USA

23 Resource centres for rare diseases – Romania
Ms. Dorica Dan, President, RONARD (Romanian National Alliance for Rare Diseases); Member of Board, EURORDIS-Rare Diseases Europe

4.3 COUNTRIES EMERGING TO THE CHALLENGES OF RARE DISEASES: FROM THE GRASSROOTS LEVEL TO THE POLICY LEVEL

24 Building a grassroots approach for persons living with a rare disease – Iran
Dr. Hamid. R. Edraki, Managing Director, Rare Diseases Foundation of Iran (RADOIR)

25 The case of rare diseases in China
Dr. Shuyang Zhang, Vice President of Peking Union Medical College Hospital (PUMCH), Director of Clinical Pharmacology Research Center, PUMCH

26 The case of rare diseases in Brazil
Mr. Ricardo Monteiro, Minister-Counsellor, Permanent Mission of Brazil to the United Nations

27 Integration of rare diseases in the national health system - Philippines
Ms. Cynthia Magdaraog, President of the Philippine Society for Orphan Disorders, Inc. (PSOD)

28 Strategies for rare diseases within government’s commitment to healthcare - South Africa
Ms. Kelly du Plessis, Chief Executive Officer, Rare Diseases South Africa

29 Regional collaboration for care of people living with a rare disease – Kuwait
Mr. Tareq Albanai, Counsellor at Permanent Mission of the State of Kuwait to the United Nations

30 Call for action on rare diseases to the Members of the United Nations
Mr. Yann Le Cam, Chief Executive Officer, EURORDIS-Rare Diseases Europe; Member of Council and Chair of Advocacy Committee, Rare Diseases International (RDI)

31 Closing
Mr. Anders Olason, Chair of the NGO Committee for Rare Diseases; Chairman of Ågrenska Foundation

32 Closing Keynote Addresses
Official video statement from Mrs. Tamara Vucic, Spouse of the President of the Republic of Serbia.
Official video statement from Mrs. Michelle Muscat, Spouse of the Prime Minister of the Republic of Malta.
Mr. Anders Nordström, Ambassador Global Health, Swedish Ministry of Foreign Affairs
H.E. Mrs. Lana Z. Nusseibeh, Ambassador and Permanent Representative, Permanent Mission of the United Arab Emirates to the United Nations
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