

21 February 2019



NGO COMMITTEE FOR
RARE DISEASES

United Nations, New York

RARE DISEASE DAY®

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)

Under the patronage of
HRH The Grand Duchess
of Luxembourg

KEYNOTE ADDRESSES

9:45-11:00

LEAVE NO PERSON
LIVING WITH A
RARE DISEASE
BEHIND

- 1 **The NGO Committee for Rare Diseases: working on a common cause at the global level**
Mr. Anders Olason, 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

SESSION 2

11:00-12:00

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

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**.

Co-Chairs:

- + **Mr. Terkel Andersen**, President of the Board of Directors, EURORDIS-Rare Diseases Europe
- + **Ms. Mandeep Dhaliwal**, Director of the HIV, Health and Development Group in the Bureau of Policy and Programme Support at United Nations Development Programme (UNDP)

- 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. Ruediger 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



SESSION 4

14:30-17:15

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** 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 Ezoe, 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, ProPacífico
- 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

Comfort Break

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

CLOSING SESSION

17:45-18:15

An event to mark the occasion of



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A Substantive Committee of
The Conference of NGOs in Consultative Relationship
with the United Nations

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.



www.agrenska.se



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