



NGO COMMITTEE FOR  
**RARE DISEASES**



*Report from the  
inauguration of the*

# **NGO COMMITTEE FOR RARE DISEASES**

An initiative by



In cooperation with



**RARE  
DISEASES  
INTERNATIONAL**



This event took place  
under the patronage of:

**Queen Silvia of Sweden**



And

**The Conference of NGOs in  
Consultative Relationship with  
the United Nations (CoNGO)**



at the United Nations  
Headquarters, New York





With the support of \_\_\_\_\_

**The Government of the Kingdom of Belgium**

**The Government of the Republic of France**

**The Government of the Republic of Malta**

**The Permanent Mission of Estonia to the United Nations**

**&**

**The Swedish Ministry of Health  
and Social Affairs**

The launch of the NGO Committee for Rare Diseases was also endorsed by a number of personalities through statements of support:

**Baroness Jane Campbell** – Independent  
Member of the UK House of Lords

**Philippe Douste-Blazy** – Under-Secretary-General of the UN, Special Adviser on Innovative Financing for Development in the United Nations, Chairman of UNITAID and Former French Minister of Health (1993-1995 and 2004-2005)

**Françoise Grossetête** – Member of the European Parliament and Vice-Chair of the Group of the European People's Party

**President Tarja Halonen** – Former President of Finland and Co-Chair of the WHO-OHCHR High Level Working Group for the Health and Human Rights of Women, Children and Adolescents

**Federico Mayor Zaragoza** – President of the Foundation Culture of Peace and Former Director-General of UNESCO (1987 – 1999)

**Marek Plura** – Member of the European Parliament

**Dainius Puras** – United Nations Special Rapporteur on the right of everyone to the enjoyment of the highest attainable standard of physical and mental health

**Miklós Szócska** – Former Minister of State for Health of Hungary, Director of the Health Services Management Training Centre of the Budapest Semmelweis University

# 1

## An initiative of civil society from within the United Nations system

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). Its goal is to promote rare diseases as a priority in global health, research, and social and medical care as part of the UN 2030 Agenda: the Sustainable Development Goals (SDGs). This Committee is an initiative of the Ågrenska Foundation and EURORDIS-Rare Diseases Europe in cooperation with Rare Diseases International (RDI).

The launch of the NGO Committee for Rare Diseases took place on 11 November 2016 at the United Nations headquarters in New York under the patronage of her majesty Queen Silvia of Sweden, with the active support and encouragement of several governments and with the engaged participation of a broad range of civil society actors.

This 'Global Gathering for Rare Diseases' brought together in New York 100 participants from around the world, and more than 1,600 people connected to the live webcast through the Committee's dedicated website.



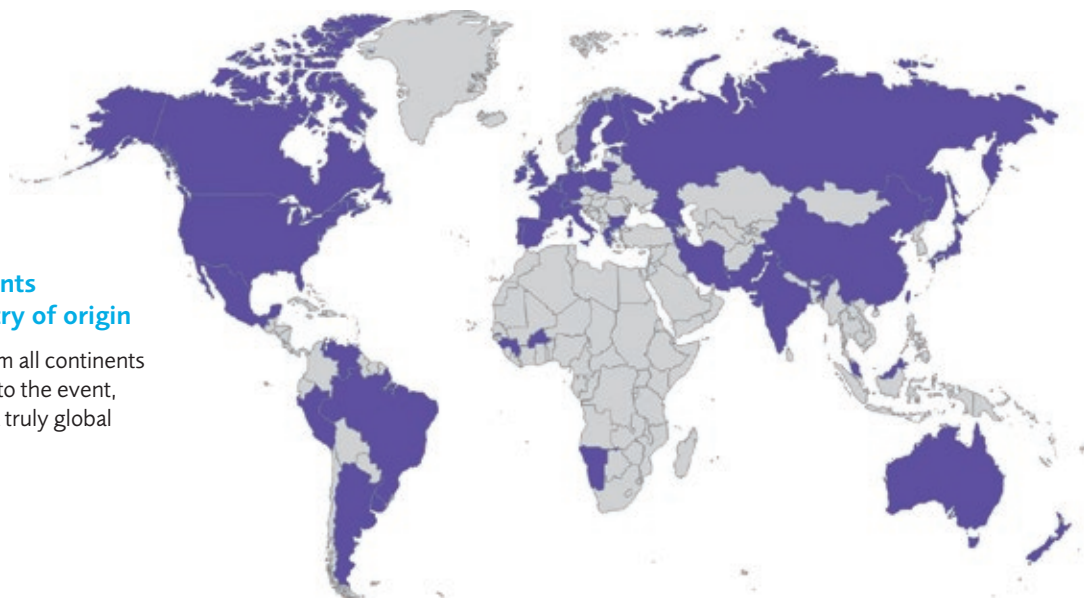
From left to right, Avril Daly (Retina International), Durhane Wong-Rieger (CORD), Yann Le Cam (EURORDIS), Cyril Ritchie (CoNGO), Anders Olauson (Ågrenska), Navid Hanif (ECOSOC), Abbey Meyers (NORD) and Terkel Andersen (EURORDIS)

### Participants



### Participants by country of origin

People from all continents signed up to the event, making it a truly global gathering.





# Rare diseases as a global public health priority

# 2

There are over 6,000 identified rare diseases. Even though one disease may be rare, the number of people affected by rare diseases is huge. There is an estimated 30 million affected people in Europe alone, and an estimated 350 million affected individuals worldwide.

One of the main objectives of the inauguration of the NGO Committee for Rare Diseases was to establish rare diseases as a global health priority for the participants in the room and the audience watching the live stream online.

The testimonials from persons living with a rare disease and advocates during the meeting served to illustrate that rare diseases impact not only a person's health, but also issues around their socio-economic status, family, education and labour. Difficulties such as poverty, unemployment, stigmatisation and social exclusion are not distant concepts for people living with a rare disease. They are in fact a daily reality, and a direct consequence of their disease.

Gender is an extra layer of complexity – women and girls with rare diseases and their mothers, often suffer from multiple exclusions from health care systems, labour markets and so on.

As the struggles faced by people living with a rare disease are shared across many different diseases and geographies, these common challenges demand common solutions.

During panel one, a number of representatives from the rare disease community presented their strategies and vision for tackling rare diseases. The overwhelming message that came out of this session was that differences in values and priorities are not an obstacle to working together. In fact, this complexity and diversity can be turned into an advantage for impactful action from the grassroots to the global level.



Multiplicity of challenges faced by persons with a rare disease and their families.



From left to right, Yann Le Cam (EURORDIS), Abbey Meyers (NORD), Anders Olason (Ågrenska), Avril Daly (Retina International) and Terkel Andersen (EURORDIS)



Hassan Vahidnezhad and Leila Youssefian, visiting scholars at the Thomas Jefferson University and representatives of the Rare Diseases Foundation of Iran (RADOIR) with Gunilla Jaeger, Psychologist at Ågrenska



H.E. Mr. Marc Pecsteen de Buytswerve addressing the attendees at the inauguration reception at the offices of the Permanent Representation of Belgium to the UN

# 3

## The NGO Committee for Rare Diseases

An international rare diseases community is emerging, increasingly organised to take global action. A number of initiatives in various fields were presented at the meeting:

- » The International Rare Diseases Research Consortium (IRDiRC) is paving the way for future cures and treatments through the creation of a platform for cooperation in scientific research.
- » The International Federation of Pharmaceutical Manufacturers and Associations (IFPMA) is committed to finding solutions for rare diseases through the development of orphan medicinal products and has founded a Rare Diseases Working Group gathering 14 leading international pharmaceutical manufacturers.
- » OrphaNet has evolved into the primary international portal of reference for the inventory and classification of rare diseases.
- » The International Conference on Rare Diseases and Orphan Drugs (ICORD) has become the annual gathering point for rare diseases stakeholders, and is playing today a central role in advancing knowledge, research, care, information and education about rare diseases globally.

The last few decades have been characterised by the expansion of networks of patient and disability organisations. The testimonials from the ten representatives of the rare disease community during session six of Panel one indicated this growing trend towards global collaboration. Patient and disability organisations have



**To have access is not enough. Only through strong social policies can human rights be made usable for people, and the potential of people with rare diseases to contribute to society be fulfilled. NGOs have the power, the knowledge, the energy and ability needed to achieve that goal »**

Daniela Bas, Director, Division for Social Policy and Development (DSPD), UN Department of Economic and Social Affairs (DESA)



From left to right, Lauren Barredo (UN Sustainable Development Solutions Network), Migdalia Denis (Latin American Society for Pulmonary Hypertension) and John Forman (International Conference on Rare Diseases and Orphan Drugs).



played an essential role in breaking the isolation of persons with a rare disease transforming individual stories into collective action and fostering exchange. These organisations have been the true pioneers in the advancement of rare disease national strategies and the establishment of legal frameworks like the US Orphan Drug Act and the EU Orphan Medicinal Products Regulation.

As these networks of patient and disability organisations grow and in turn foster horizontal collaboration with other stakeholders, the need for a global platform increases. A platform that serves as a space to share ideas but also to bring these ideas to the leading force of global collaboration: the United Nations. This is the role of the NGO Committee for Rare Diseases.

As one of the forty substantive Committees of the Conference of NGOs in Consultative Relationship with the United Nations (CoNGO), the NGO Committee is now an integral part of the principal interface between the world's NGOs and the United Nations system. It is through the input and expertise of NGOs that national governments and United Nations agencies can improve their output and generate policies that truly respond to needs at the grassroots level. By having a seat at the table, the NGO Committee for Rare Diseases can now empower its local members, providing the link between the bottom-up and the top-down approach and formalising alliances.

Its main mission is thus to:

- » Bring visibility and understanding about rare diseases to the United Nations.
- » Act as an advocacy platform for the issue of rare diseases, creating a space for collaboration.



From left to right, Rachel Yang (Chinese Organization for Rare Disorders), Yukiko Nishimura (Advocacy Service for Rare and Intractable Diseases' stakeholders in Japan) and Ramaiah Muthyala, Founder of the Indian Organization for Rare Diseases (IORD)

- » Collect and share accurate information about rare diseases and associated challenges.
- » Elevate rare diseases as a priority within global public health, research, medical care, social care and social integration.



**Our experience in hemophilia shows how the support of the United Nations can help make a difference to reduce stigma, gain support from governments and encourage better regulatory cooperation »**

Alain Weill, President of the Board, World Federation of Hemophilia (WFH)



# 4

## Rare diseases are relevant to the Sustainable Development Goals of the UN 2030 Agenda



**The Sustainable Development Goals are inclusive. If they are not met for everyone equally, then they are not met »**

Lauren Barredo, Manager, Thematic Group on Health, UN Sustainable Development Solutions Network (SDSN)



The timing of the new NGO Committee for Rare Diseases is perfectly aligned with the kick-off of the United Nations 2030 Agenda: the Sustainable Development Goals (SDGs). This universal agenda is comprised of 17 goals, a number of which are in synergy with the goals of the rare disease community .

As Navid Hanif, Director of the Office for ECOSOC Support and Coordination, said during the meeting: 'Development is a mirage without health'. The UN Millennium Development Goals (2000-2015) already included three health-specific targets and paved the way for future international collaboration. However, these goals were narrow in scope and did not highlight the connection that health has with so many other aspects (e.g. economic development, social integration, gender issues and education).



**Development is a mirage without health »**


Navid Hanif, United Nations Economic and Social Council (ECOSOC)

The post-2015 agenda was therefore expanded into a universal project. Not only is each specific SDG broader in scope, but they are to be viewed in a holistic way, interdependent on one and another and to be achieved by both developing and developed nations. As proven by NGOs' testimonials during the inauguration, this interdisciplinary perspective has already formed the basis of rare disease advocacy strategies, as the well-being of those affected depends not only on access to health, but also social care, non-discrimination and opportunities to study, work and contribute to society.

The coherence between the rare diseases community's goals and the SDGs was confirmed by members of United Nations agencies at the inauguration. The bearing principle of the Agenda, "Leave no one behind", which should be upheld throughout the implementation, was mentioned by several speakers. Vidhya Ganesh of UNICEF said that although the prioritisation of rare diseases would be challenging as part of the operational work of the agency, they would support it in their advocacy efforts to ensure that their mandate is fulfilled and that 'no child is left behind'.

Similarly, Nata Menabde, Executive Director of the World Health Organisation Office at the United Nations, said that the WHO was pushing for a new agenda, transforming itself into a more collaborative partner and focusing on the goal of universal health coverage. In words that echoed those of UNDP Director Helen Clark at the 11th ICORD meeting, Nata Menabde said: 'If we are serious about leaving no one behind, then we cannot leave behind people who have rare diseases just because they are few'. She declared the openness of the WHO to collaborate with the NGO Committee in the future.



 H.E. Prime Minister Joseph Muscat declares the government of Malta's intention to prioritise rare diseases during their term of the Presidency of the European Council



**If we are serious about leaving no one behind, then we cannot leave behind people who have rare diseases just because they are few »**

Nata Menabde, World Health Organisation (WHO)

The overall outcome of Panel two was the need to take a rights approach, where persons with rare diseases need to be seen as more than just patients or their disease, but as human beings with rights to health and well-being. As Tenu Avafia, Policy Adviser on HIV, Health and Development Practice at the United Nations Development Programme (UNDP) said in his concluding note 'this must be framed around the chapeau of human rights as they are enshrined in most countries' constitutions'.

People living with a rare disease must be given the chance to fulfil social functions and contribute to a common welfare. As Marek Plura, Member of the European Parliament, said: 'it is not possible to misuse the potential of citizens'. In line with this view, Daniela Bas, Director of the Division for Social Policy and Development (DSPD) advocated for the need to move from a medical model to a social model. In conclusion, a place at the table for rare diseases was confirmed and a number of potential initiatives proposed.

# A call for global action on rare diseases to achieve the 2030 Agenda

The 17 SDGs will not be achieved without the activism of civil society. Navid Hanif, Director of the Office for ECOSOC Support and Coordination, pointed out that while governments implement policy, they more often than not need the guidance, expertise and real-life evidence coming from patient organisations and NGOs to produce sound policies. For rare diseases, the most important role for these groups is two-fold:

- » to press on governments to include rare diseases in their policies and in their budgets; and
- » to take a system-strengthening approach by advocating to the global community as a whole on the issues people living with a rare disease face.

A key success factor for the creation of this enabling environment is alignment between stakeholders. Several members of UN agencies gave examples of success stories coming from the common engagement of NGOs, particularly in the field of health. For instance, non-communicable diseases were put to the fore following a six-year campaign and are now integral to all United Nations agencies' programmes. Similarly, the International Diabetes Federation (IDF) led a campaign in 2006 and secured a UN resolution (61/225) after six months only, affirming diabetes as a major global health threat. In the case of rare diseases, Malta Ambassador, H.E. Carmelo Inguanez, welcomed the use of a UN resolution as a tool to encourage further action worldwide.

In order to do this, there are various fora and initiatives available to rare disease NGOs that are in consultative relationship with the United Nations, including those groups that are members of the NGO Committee for Rare Diseases:

- » **The High-Level Political Forum (HLPF):** this is United Nations' central platform for the follow-up and review of the 2030 Agenda for Sustainable Development and the SDGs. The HLPF includes voluntary country reviews, thematic reviews on progress of SDGs as well as a range of side events that can be organised by major stakeholders like civil society.
- » **ECOSOC Partnerships Forum:** Every year ECOSOC and other UN entities collaborate to bring together leaders from government, the private and non-profit sectors and civil society to discuss multi-stakeholder partnerships.
- » **The World Health Assembly:** This is the decision-making body of the WHO and determines the policies of the organisation, appoints the Director-General and approves the proposed programme budget. There are also a number of side-events organised on the same days.



**I truly believe that the time for rare diseases is now and we stand committed to assist in any way we can at UNDP »**

Tenu Avafia, Human Rights, Law and Treatment Access, HIV, Health and Development Group, United Nations Development Programme (UNDP)



- » **Official International days:** These can be a good opportunity for strong communication and advocacy campaigns such as Official WHO health days.
- » **The Commission for Social Development (CSocD):** This is the key UN body advising ECOSOC on social policies of a general character and, in particular, on all matters in the social field not covered by the specialised inter-governmental agencies.
- » **The UN Convention on the Rights of People with Disabilities (CRPD):** This is intended as a human rights instrument with an explicit social development dimension. As it adopts a broad categorization of persons with disabilities it is possible for the rare diseases community to employ this instrument in its advocacy where relevant and hold accountable those countries that are signatories.



Hawa Dramé, Founder of Fondation Internationale Tierno et Miriam (FITIMA) and Ramaiah Muthyala, Founder of the Indian Organization for Rare Diseases (IORD)



» **UN General Assembly Resolutions:** These are voted on by all member states of the UN in the General Assembly. They usually require a simple majority to pass. These are generally non-binding towards member states but they may act as soft law. Rare Diseases advocates can try to collaborate with governments to push for a resolution on Rare Diseases (particularly on Rare Disease Day as it was done for Diabetes).

The opportunities for global action and global advocacy can be facilitated through closer collaboration with UN agencies and the introduction of rare diseases into their own programmes and advocacy.

The end of the inauguration saw the presentation of the NGO Committee Founding Act, demonstrating the consensus of event participants that rare diseases are a global healthy priority.



**I am moved by what I have heard today - testimonies of great breadth and depth, from all corners of the globe, from people whose knowledge is grounded in daily - and often dramatic - reality »**

Cyril Ritchie, President of CoNGO 2011-2018

## 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 repertoried 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.

*[Handwritten signatures of participants]*

 **NGO COMMITTEE FOR RARE DISEASES**

# The synergies between the UN Sustainable Development Goals and rare diseases

Sustainable Development Goals	Rare Disease Challenges
<b>Goal 1</b> End Poverty in all its forms everywhere	Persons with rare diseases are often trapped in a vicious cycle of vulnerability and poverty due to exclusion from health care and education systems, as well as job markets
<b>Goal 3</b> Ensure healthy lives and promote well-being for all at all ages	Achieving universal health coverage implies attending to the needs of persons living with a rare disease who need more and better medicines, appropriate diagnosis and lifelong care and social support
<b>Goal 4</b> Ensure inclusive and equitable quality education and promote lifelong learning opportunities for all	50% of rare diseases affect children who often face difficulties attending school due to inaccessibility of facilities and non-adapted teaching methods
<b>Goal 5</b> Achieve gender equality and empower all women and girls	Recognising and valuing unpaid care and domestic work that many mothers of children (including when they are grown adults) with rare diseases take on will be a step towards greater gender equality

## Inauguration Programme

Morning Plenary: 10:00 to 13:30

### KEYNOTE ADDRESSES

10:00-11:00

### PANEL DEBATE 1

11:00-13:30

### THE GLOBAL STATE OF PLAY OF RARE DISEASES

Moderator:  
**Mr Yann Le Cam**,  
Chief Executive,  
EURORDIS-Rare  
Diseases Europe;  
Council Member,  
Rare Diseases  
International (RDI)

- 1 The United Nations and Civil Society: CoNGO and CoNGO Committees**  
**Mr Cyril Ritchie**, President of CoNGO 2011-2018
- 2 Public Health in the Mandate of the United Nations Economic and Social Council**  
**Mr Navid Hanif**, Director, Office for ECOSOC Support and Coordination, UN Department of Economic and Social Affairs (DESA)
- 3 Rare Diseases: A Common Cause at the Global Level**  
**Mr Anders Olauson**, Chairman, Ågrenska
- 4 How It All Started: The Contribution of the NGO Community to Advancing the Cause of Rare Diseases**  
+ **Ms Abbey S. Meyers**, President Emeritus, National Organization for Rare Disorders (NORD)  
+ **Mr Terkel Andersen**, President of the Board of Officers, EURORDIS-Rare Diseases Europe; Chairman of the Board, Danish Hemophilia Society
- 5 A Call for Global Action Coming from Civil Society**  
**Mr Yann Le Cam**, Chief Executive, EURORDIS-Rare Diseases Europe; Council Member, Rare Diseases International (RDI)
- 6 Tackling Rare Diseases Internationally Through National and Regional Strategies**  
+ **Europe** ▶ **Ms Avril Daly**, Vice-President of the Board of Officers, EURORDIS-Rare Diseases Europe; Chief Executive, Retina International  
+ **North America** ▶ **Mr Paul Melmeyer**, Associate Director of Public Policy, National Organization for Rare Disorders (NORD)  
+ **Latin America** ▶ **Ms Migdalia Denis**, Founder, Venezuelan Society for Pulmonary Hypertension; Governing Board Member, International Alliance of Patients' Organisations (IAPO)  
+ **Russia** ▶ **Ms Irina V. Myasnikova**, President, Interregional Public Organisation « Help for Patients with Cystic Fibrosis »; Co-Chair, All-Russia Patients Union  
+ **Middle East and Central Asia** ▶ **Mr Sirous Eftekhari**, International Affairs, Rare Diseases Foundation of Iran (RADOIR)  
+ **Africa** ▶ **Ms Hawa Dramé**, Founder, Fondation International Tierno et Mariam (FITIMA), Burkina Faso and Guinea  
+ **Japan** ▶ **Ms Yukiko Nishimura**, President, ASrid (Japanese Advocacy Service for Rare and Intractable Diseases)  
+ **China** ▶ **Ms Rachel Yang**, Director of International Affairs, Chinese Organization for Rare Disorders (CORD)  
+ **Malaysia** ▶ **Ir Lee Yee Seng**, President, Malaysia Lysosomal Diseases Association  
+ **Australia** ▶ **Ms Megan Fookes**, Founder, Rare Voices Australia; Council Member, Rare Diseases International
- 7 Tackling Rare Diseases Internationally Through Bottom-Up Diagnosis and Care Strategies**  
+ **Mr Lieven Bauwens**, Secretary-General, International Federation for Spina Bifida and Hydrocephalus (IF)  
+ **Mr Alain Weill**, President of the Board, World Federation of Hemophilia (WFH)  
+ *in conversation with* **Ms Vidhya Ganesh**, Deputy Director, Programme Division, United Nations Children's Fund (UNICEF)

**Goal 8**  
Promote sustained, inclusive and sustainable economic growth, full and productive employment and decent work for all

Persons with rare diseases and disabilities are often marginalised from job markets due to a lack of accessible facilities, flexible working hours and adapted roles.

**Goal 9**  
Build resilient infrastructure, promote inclusive and sustainable industrialization and foster innovation

There is a need to invest in research and development of therapies, health technologies and diagnostic tools for persons with rare diseases  
Accessibility to infrastructure is key for persons with rare diseases in order to be included in society

**Goal 10**  
Reduce inequality within and among countries

People living with a rare disease tend to remain a marginalised and invisible population suffering from discrimination in the health, labour and governance fields

**Goal 17**  
Revitalise the global partnership for sustainable development

The rare disease community is increasingly interconnected, with a myriad of networks of patient advocates, regulators, research and industry  
The NGO Committee for Rare Diseases embodies this goal as multi-stakeholder partnerships are enshrined in its by-laws

## PANEL DEBATE 2

14:30-17:30

### THE WAY FORWARD: ACTING GLOBALLY FROM WITHIN THE UN SDGs

Moderator:  
**Ms Durhane Wong-Rieger**, President and CEO, Canadian Organization for Rare Disorders (CORD); Council Member, Rare Diseases International (RDI); Former Chair of the Governing Board, International Alliance of Patients' Organisations (IAPO)

## CLOSING SESSION

17:30-18:00

### 8 How to Further Carry the Patients' Voice at the Global Level to Deliver Change?

**Ms Durhane Wong-Rieger**, President and CEO, Canadian Organization for Rare Disorders (CORD); Council Member, Rare Diseases International (RDI); Former Governing Board Chair, International Alliance of Patients' Organisations (IAPO)

### 9 The UN Sustainable Development Goals (SDGs): Implications for Health in General and Rare Diseases in Particular

- + **Ms Lauren Barredo**, Manager, Thematic Group on Health, UN Sustainable Development Solutions Network (SDSN)
- + **Dr Nata Menabde**, Executive Director, WHO Office at the United Nations, New York
- + *in conversation with* **Mr Jean-Louis Roux**, Public Affairs Director, European and International Advocacy, EURORDIS-Rare Diseases Europe

### 10 Bringing the SDGs to Life: Spotlight Examples of Where the Rare Disease Community Can Make a Contribution

- + **Disability and Gender** ▶ **Ms Maria Montefusco**, Secretary of the Council of Nordic Cooperation on Disability, Nordic Center of Welfare and Social Issues *in conversation with* **Dr Gustavo Gonzalez-Canali**, Senior Advisor, UN Coordination Division, UN WOMEN
- + **Education** ▶ **Ms Gunilla Jaeger**, Psychologist, Ågrenska
- + **Employment, Inclusion and Poverty** ▶ Video testimonial by **Mr Marek Plura**, Member of the European Parliament, European Parliament's Committee on Employment and Social Affairs; Vice-President, Disability Intergroup at the European Parliament *in conversation with* **Ms Daniela Bas**, Director, Division for Social Policy and Development (DSPD), UN Department of Economic and Social Affairs (DESA)
- + **A Holistic Perspective** ▶ **Mr Tenu Avafia**, Human Rights, Law and Treatment Access, HIV, Health and Development Group, United Nations Development Programme (UNDP)

### 11 Avenues for Global Action: Mobilising all stakeholders in the rare disease community worldwide on the road to 2030

- + **IRDiRC: How Can International Collaboration Accelerate Research Advancements?** **Dr Christopher P. Austin**, Director, National Center for Advancing Translational Sciences (NCATS); Chair, International Rare Disease Research Consortium (IRDiRC)
- + **IFPMA: How Can the Pharma and Biotech Industry Help Translate Scientific Opportunities into Accessible and Affordable Treatments?** **Dr Philip Vickers**, Global Head of Research and Development, Shire on behalf of the International Federation of Pharmaceutical Manufacturers and Associations (IFPMA)
- + **OrphaNet: How Can International Classification of Rare Diseases, International Encyclopedias and Resource Listings Enhance Research and Clinical Excellence?** **Dr Ana Rath**, Director, INSERM US14-OrphaNet
- + **ICORD: How Can A Cycle of Multi-Stakeholder Conferences Stimulate Dialogue and Actions in Different Parts of the World?** **Mr John Forman**, Past-President, International Conference on Rare Diseases and Orphan Drugs (ICORD)

### 12 Introducing the NGO Committee for Rare Diseases and its Founding Act « Rare Diseases and the UN Sustainable Development Goals » **Mr Anders Olason**, Chairman, Ågrenska

### 13 Closing Keynote Addresses

with an official statement from **H.E. Prime Minister Joseph Muscat and the Government of the Republic of Malta** on the eve of the Maltese Presidency of the Council of the European Union in 2017



# The organisers of the inauguration of the NGO Committee for Rare Diseases would like to give their special thanks to:

<b>Terkel Andersen</b>	President of the Board of Officers, EURORDIS	<b>Ted FitzGerald</b>	Director of Global Research Services, FHI 360
<b>Christopher Austin</b>	Director, NCATS; IRDiRC	<b>John Forman</b>	Past-President, International Conference on Rare Diseases and Orphan Drugs (ICORD)
<b>Tenu Avafia</b>	Policy advisor on Human Rights, Law and Treatment Access at the HIV, Health and Development Group, United Nations Development Programme (UNDP)	<b>Megan Fookes</b>	Co-Founder, Rare Voices Australia
<b>Frédéric Badey</b>	Senior Director International Public Affairs Coordination, Sanofi	<b>Michele Galen</b>	Independent strategic advisor
<b>Lauren Barredo</b>	Manager at Thematic Group on Health, UN Sustainable Development Solutions Network (SDSN)	<b>Vidhya Ganesh</b>	Deputy Director of Programme Division, UNICEF
<b>Fabienne Bartoli</b>	Social Affairs, Permanent mission of France to the United Nations in New York	<b>Jean-Pierre Gaspard</b>	Chief Executive, AFM-Telethon
<b>Daniela Bas</b>	Director, Division for Social policy and Development, United Nations Department for Economic and Social Affairs (DESA)	<b>Gustavo Gonzalez-Canali</b>	Senior Advisor at UN Coordination Division, UN Women
<b>Lieven Bauwens</b>	Secretary-General, The International Federation for Spina Bifida and Hydrocephalus (IF)	<b>Jaimie Grant</b>	Communications Officer, International Disability Alliance
<b>Muriel Beach</b>	Representative to UN, ILC: Global Alliance	<b>Steve Groft</b>	Senior Advisor to Director, NCATS/NIH/USA
<b>Paula Brito e Costa</b>	Chair, Raríssimas; President of the Board of Directors, FEDRA; Vice-President, ALIBER	<b>Rae Gurewitsch</b>	On site UN Representative, Hadassah
<b>William Buttigieg</b>	Advisory Board Member and Representative, The National Alliance for Rare Diseases Support - Malta,	<b>Navid Hanif</b>	Director of Office for ECOSOC Support and Coordination, United Nations Department of Economic and Social Affairs (DESA)
<b>Emmanuel Chantelot</b>	Executive Director, Government Relations & Policy for Europe, Middle-East & Africa, Celgene	<b>Marc Hanauer</b>	Deputy director; INSERM OrphaNet
<b>Kathleen Coolidge</b>	Director of Patient Advocacy, Sanofi Genzyme	<b>Matthew Harold</b>	International Public Affairs Lead for Rare Disease, Pfizer, Inc.
<b>Marcelo Costa</b>	Diplomat, Brazilian Mission to the UN	<b>Robert Hejdenberg</b>	President, Agrenska
<b>Thomas Croce</b>	Head of Global Patient Advocacy, Shire	<b>Anja Helm</b>	Manager of Relations with Patient Organisations, EURORDIS
<b>Michael Cronin</b>	Main Representative to the UN-New York, International Federation of Social Workers	<b>Clara Hervás</b>	Public Affairs Junior Manager, EURORDIS
<b>Susan Crowley</b>	Delegate, Global Alliance for Women's Health	<b>Claudia Hirawat</b>	Chair of International Circle of Ambassadors, EURORDIS
<b>Avril Daly</b>	Vice-President of the Board of officers, EURORDIS	<b>BJ Holtgrewe</b>	Photographer
<b>Mariana Darvenne</b>	Adviser, Brazilian Mission to the UN	<b>Carmelo Inguañez</b>	Permanent Representative, Permanent Mission of Malta to the UN
<b>Migdalia Denis</b>	President, Latin American Society for Pulmonary Hypertension	<b>Gunilla Jaeger</b>	Chief of operations, Agrenska
<b>Hawa Dramé</b>	Founder, Fondation Internationale Tierno et Miriam (FITIMA)	<b>Dirk Jakobi</b>	Second Secretary, Permanent Mission of Germany to the UN
<b>Florence Duguet</b>	Delegate to international Actions, AFM-Téléthon	<b>Alireza Javaheri</b>	Counsellor on UNFPA, UNICEF, UN Women, SRHR, HIV/AIDS, Health, Permanent Mission of Sweden to the UN
<b>Kesinee Dulyarat</b>	UN Representative, Pan Pacific and South East Asia Women's Association	<b>Richard Jordan</b>	Director of UN Operations, The Royal Academy of Science International Trust
<b>Jos Even</b>	Representative, Luxembourg Association for Neuromuscular and Rare Diseases (ALAN)	<b>Margaretha Jones-Eggenberger</b>	UN Representative, International Humanist and Ethical Union
		<b>Sven Jürgenson</b>	Permanent Representative, Permanent Mission of Estonia to the United Nations
		<b>Elizabeth Katz</b>	Publisher, Neurology Reviews
		<b>Lisa Kearns</b>	Research associate at the Division of Medical Ethics, NYU School of Medicine

<b>Nancy Kelly</b>	Executive Director, Health Volunteers Overseas
<b>Katherine Klein</b>	UN Volunteer, ICC
<b>Bruce Knotts</b>	Director, Unitarian Universalist UN Office
<b>Mark Krueger</b>	President, MK&A
<b>Yann Le Cam</b>	Chief Executive Officer, EURORDIS
<b>Yee Seng Lee</b>	President, Malaysia Lysosomal Diseases Association
<b>Bette Levy</b>	Main UN Representative, Soroptimist International
<b>Michele Lipucci Di Paola</b>	President, AVL T (Associazione Veneta Lotta alla Talassemia )
<b>Veronica Lopez</b>	Strategic Associate, MK&A
<b>Flaminia Macchia</b>	Director of Government Affairs and Public Policy, Vertex
<b>Paul Melmeyer</b>	Associate Director of Public policy, National Organization for Rare Disorders (NORD)
<b>Nata Menabde</b>	Executive Director of WHO Office at the United Nations, New York
<b>Abbey Meyers</b>	President Emeritus, National Organization for Rare Disorders (NORD)
<b>Irina Myasnikova</b>	Co-Chairman, Russian Patients Union; President, Help to Cystic Fibrosis Patients; CEO, Russian Association for Rare Diseases
<b>Maria Montefusco</b>	Project Manager, Council of Nordic Cooperation on Disability
<b>Padmini Murthy</b>	NGO Representative to the United Nations, Medical Women's International Association
<b>Ramaiah Muthyala</b>	Founder, Indian Organization for Rare Diseases, (IORD); Member of council, Rare Diseases International (RDI)
<b>Yukiko Nishimura</b>	President, Advocacy Service for Rare and Intractable Diseases' stakeholders in Japan (ASrid)
<b>Kunihiro Nishimura</b>	Vice-President, Advocacy Service for Rare and Intractable Diseases' stakeholders in Japan (ASrid)
<b>Ann Nordgren</b>	Professor, Karolinska Institutet (Sweden)
<b>Anders Olason</b>	Chairman, Agrenska
<b>Francesc Palau</b>	Director, Pediatric Institute for Rare Diseases – IPER; Group Leader U732 – Neurogenetics and Molecular Medicine, CIBER on Rare Diseases – CIBERER
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<b>Laurie Shestack Phipps</b>	Advisor on Gender, Health and Social Issues, ECOSOC Section, U.S. Mission to the United Nations
<b>Terje Raadik</b>	First Secretary, Mission of Estonia to the UN

<b>Ana Rath</b>	Director, INSERM OrphaNet
<b>Cyril Ritchie</b>	President, the Conference of NGOs in Consultative Relationship with the United Nations (CoNGO)
<b>Shane Robinson</b>	Co-Executive Director, Elhers-Danlos National Foundation (EDNF)
<b>Jean-Louis Roux</b>	Public Affairs Director, European and International Advocacy, EURORDIS
<b>Alasan Senghore</b>	Head of Delegation and Permanent Observer, International Federation of Red Cross and Red Crescent Societies (IFRC)
<b>Fatima Scipione</b>	Senior Director of Patient Advocacy, Takeda Oncology
<b>Tiana Toumayan Smilow</b>	Main UN Representative, NGO Armenian International Women's Association
<b>Philip Spoerri</b>	Permanent Observer and Head of Delegation, International Committee of the Red Cross (ICRC)
<b>Paloma Tejada</b>	Rare Diseases International Senior Manager, EURORDIS/RDI (Rare Diseases International)
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<b>Min Chieh Tseng</b>	President, Taiwan Foundation for Rare Disorders (TFRD)
<b>Jeffrey T. Tseng</b>	Patient Representative, Taiwan Foundation for Rare Disorders (TFRD)
<b>Hassan Vahidnezhad</b>	Visiting Scholar scientist and research assistant , Department of Dermatology and Cutaneous Biology, Thomas Jefferson University; Representative of Rare disease foundation of Iran (RADOIR)
<b>Philip Jonathan Vickers</b>	Global Head of Research and Development, Shire (on behalf of International Federation of Pharmaceuticals Manufacturers and Associations, IFPMA)
<b>Inger Wästberg</b>	Former Director General Swedish government
<b>Alain Weill</b>	President of the Board, World Federation of Hemophilia
<b>Durhane Wong-Rieger</b>	President and CEO, Canadian Organization for Rare Disorders (CORD)
<b>Rachel Yang</b>	Director of International Affairs, Chinese Organization for Rare Disorders (CORD)
<b>Leila Youssefian</b>	Visiting scholar Scientific and research assistant at the Department of Dermatology and Cutaneous Biology, Thomas Jefferson University, Philadelphia, PA, USA



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