

My lighthouse..

PATIENTS EXPECTATIONS FOR AND PARTICIPATION IN THE RESEARCH AGENDA

Anders Olauson
EPF President

21 January 2016
Brussels

“ A STRONG PATIENTS' VOICE TO DRIVE BETTER HEALTH IN EUROPE ”



Anders Olauson

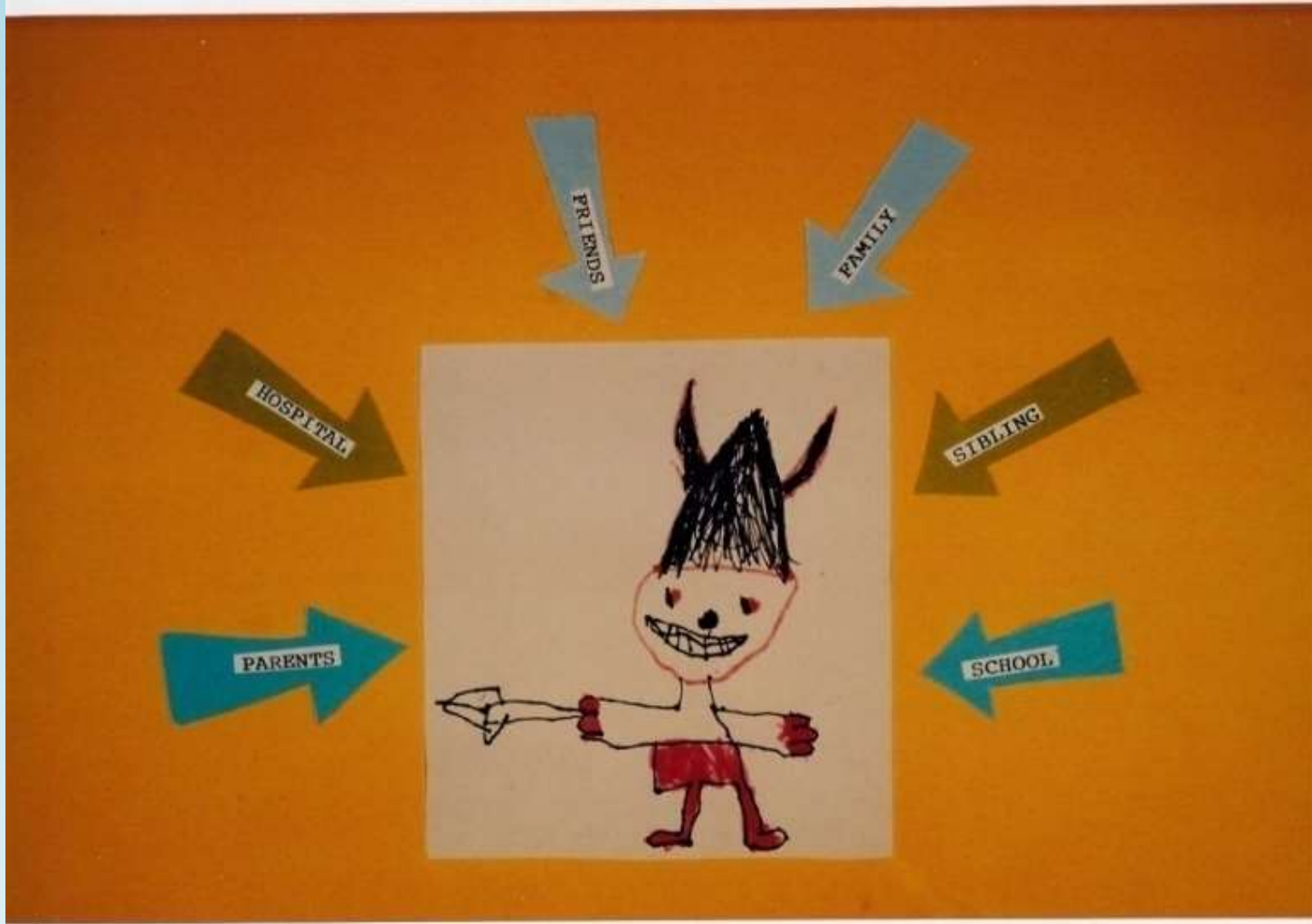
Honorary Chairman of the **European Patient Forum**,
Chairman of **Agrenska** Center in Sweden
Former president and board member of **eurordis**



SUPPORT TO RESEARCH FOR RARE DISEASES
EU AND OTHER SOURCES

BÉATRICE DE MONTLEAU
EURORDIS
1ST NOVEMBER 2015

eurordis.org



Roger,
My teacher!

Ministers of Health 1989 - 2015



Commissioners for DG Sanco/Sante in the EC from 2005 - 2015



Carlos Moedas
Commissioner for Research,
Science and Innovation



Agrenska – a centre for people with disabilities, their families and professionals



Queen Silvia Patron of Agrenska Sw.
Mrs. Ilves, Patron of Eesti Agrenska

Today's Ågrenska started by combine the following parties;

- Hospital,
- School,
- Social,
- Patients
- the Agrenska Foundation



Agrenska programs 1989 - 2016

- **Family program** – focus on rare diseases, since 1989 >5 000 families
- **Adult program** – focus on rare diseases
- **Respite care** - all kinds of disabilities
- **Staff with disabilities** – day centre
- **Personal assistance**
- **ADHD consultant**
- **Courses for professionals**
- **Research**
- **Conferences**
- **Projects/development**

Why patients view, -Some reflections after more than 26 year's of experience



1. Too few professionals have knowledge of rare diseases and their impact on individual and family
2. Efforts incomplete or wrong
3. Long time before diagnosis set - needless suffering
4. Charged to the wrong parts of the system
5. Society's efforts are not coordinated - system competence is lacking in many of society - must be a carrier of knowledge
6. Need to discuss opportunities instead of obstacles
7. Must always explain and "defend" their difficulties / disability due to rarity and ambient ignorance
8. Need different stages of life to gain knowledge and meet others in the same situation





UNITED NATIONS
 1 UN Plaza, Room 5600, New York, NY 10017
 Tel: (212) 963-2000 Fax: (212) 963-2001
 www.un.org/development/desa

21 July 2013

Dear participants,

We would like to welcome you to the Economic and Social Council at its collective session on 4 July 2013, devoted to the Special Representative's mission to the organization "Agenda for Sustainable Development: Goal 11. Access to Transport".

The organization may send designated official representatives to the United Nations - in the United Nations Headquarters in New York and the United Nations Offices in Geneva and Vienna - along the time in our work with you. An annual United Nations Designated representatives meet with us here present at the designated time. Studies to carry the higher priorities of your organization will allow your organization to represent effectively and faithfully the provisions for the sustainable development.

Please note, in particular, Parts E, IV, V and VI and the Council Resolution 1986(II), describing the provisions for carrying out your collaborative relationship with the Council. Additionally note that EC, paragraph 41, which requests that organizations in General and Special conferences review subject questionnaire reports on their activities for the first year period, in 2013/2014 to the Committee in 2015. You will be advised of the conditions for submitting your report in due season. Moreover, we request that you maintain detailed records of your activities.

The United Nations issues a calendar of meetings and conferences, which can be obtained by your representatives at the United Nations sites. Every year, you will receive from this office the "Calendar of United Nations meetings open to participation by an official representative in NGOs or non-State entities". This calendar will be published in the calendar for submitting your report in due season. Moreover, we request that you maintain detailed records of your activities.

Finally, should you wish to enhance your contact with the United Nations as your kind friend, please see the following website: "NGOs in Special Collaborative Status with the Economic and Social Council of the United Nations". The United Nations website may not be used, unless expressed approval has been granted by the Legal Office of the United Nations. This is neither granted the necessary use, nor for any other materials describing your organization.

We look forward to a productive relationship with your organization and its representatives.

Sincerely yours,
 Jennifer Morgan
 Deputy Secretary, Field
 NGO Section / DESA

Agenda for Sustainable Development: Goal 11. Access to Transport



PATIENTS & PARTICIPATION @
OUR VOICE
 FOR A HEALTHIER EUROPE
 22-25 MAY 2014

WATCH THIS VIDEO: @PATIENTSFORUM



United Nations  Nations Unies

NGO SECTION, DESA
1 UN Plaza, Room DC1-1477, New York, NY 10017
tel: (212) 963-8652 / fax: (212) 963-9248
www.un.org/esa/coordination/ngo
e-mail: desangosection@un.org

011081

21 July 2005

Dear sir/madam:

We would like to inform you that the Economic and Social Council, at its substantive session on July 2005, decided to grant Special consultative status to the organization "Agrenska Foundation (Grosshandlare Axel H. Agrens Donationsfond)".

The organization may now designate official representatives to the United Nations – to the United Nations Headquarters in New York and the United Nations offices in Geneva and Vienna using the form on our web site: www.un.org/esa/coordination/ngo. Designated representatives must pick up their passes in person at the designated site. Needless to say, the regular presence of your organization will allow your organization to implement effectively and fruitfully the provisions for this consultative relationship.

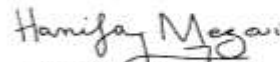
Please note, in particular, Parts II, IV, V and VII and the Council Resolution 1996/31, describing the procedures for carrying out your consultative relationship with the Council. Also, kindly note Part IX, paragraph 61c which requests that organizations in General and Special consultative status submit quadrennial reports on their activities for the four-year period in 2005-2008 to the Committee in 2009. You will be advised of the modalities for completing your report in due course. Meanwhile, we suggest that you maintain detailed records of your activities.

The United Nations issues a calendar of meetings and conferences, which can be obtained by your representatives at the United Nations sites. Every year, you will receive from this office the "Calendar of United Nations meetings open to participation by or of special interest to NGOs in consultative status". The latest copy of the calendar and other NGO-related information can also be found on the NGO Section's homepage.

Finally, should you wish to indicate your status with the United Nations on your letterhead, please use the following wording: "*NGO in Special Consultative Status with the Economic and Social Council of the United Nations*". The United Nations emblem may not be used, unless expressed approval has been granted by the Legal Office of the United Nations. This is neither granted for stationery use, nor for any printed materials describing your organization.

We look forward to a productive relationship with your organization and its representatives.

Sincerely yours,

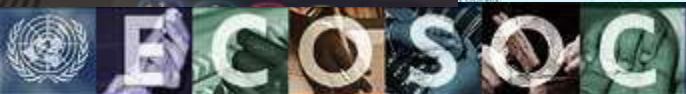

Hanifa Mezoui, Chief
NGO Section / DESA

Agrenska Foundation (Grosshandlare Axel H. Agrens Donationsfond)

Box 2058
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Sweden
FAX: 46-31-911979

RV FALSA

NGO Committee for Rare Diseases CfRD (UN, New York)



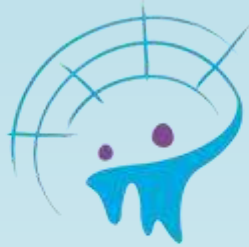
NGO Committee for Rare Diseases (UN, NY)



NGO COMMITTEE FOR
RARE DISEASES



Common Challenges

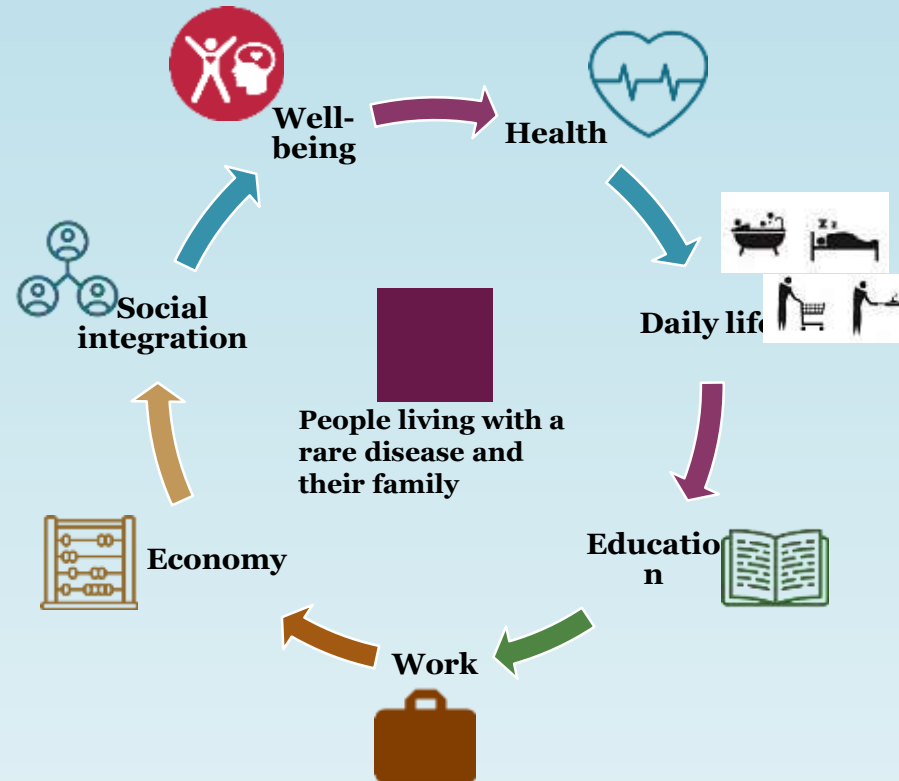


NGO COMMITTEE FOR RARE DISEASES

Ågrenska blev medlem i ECOSOC 2005 med special consultative status. Vilket är unikt.



A rare disease can affect many spheres at once



Serious unmet health and social needs affect individual's - and their families' - well-being, autonomy and fundamental human rights

Hurdles in Care Provision

- People living with a rare disease need to be followed simultaneously and continuously **by a set health, social and support services**;
- These are often **managed by different authorities/providers** and there is a lack of communication and coordination between them;
- **Care systems are fragmented** and extremely difficult to navigate for patients and families;
- Professionals **lack knowledge** on RDs and tend to be reluctant to treat patients due to the complexity of their disease;
- In most cases, the management and coordination of care has to be done by patients and families, which **places a heavy burden on family life.**

Common Solutions



NGO COMMITTEE FOR
RARE DISEASES



The need for global action against rare diseases

No one country, no one continent, can solve alone the problems posed by rare diseases.

- Numbers of people living with rare diseases as well as medical and other experts, even at a continental level, are too low and geographically scattered to reach the point of good health and well-being for every person living with a rare disease
- A critical mass of people living with rare diseases as well as other experts and public health authorities must be brought together internationally
- People living with rare diseases still remain a marginalised, vulnerable and largely invisible population within healthcare and social systems
- There is a need to develop and implement definitions of rare diseases in many countries. This is a pre-condition for both national awareness, knowledge and international comparisons.
- With little or no awareness and understanding of the needs of people living with rare diseases, they are often left unmet



The globalisation of rare diseases is gearing up...

- **Research:**

- IRDiRC: an International Rare Disease Research Consortium
- International platforms for rare disease registries

- **Patients** are increasingly organised across borders.

- **Policy:**

- Rare disease policy, strategy and plans implemented nationally, having a 'snowball' effect in other countries
- Incentivizing policies for industry investment e.g. US Orphan Drug Act and EU Orphan Medicinal Products Regulation
- Increased collaboration between agencies (EMA, FDA) + EU-WHO Data Sharing Pact



More needs to be done

There is an urgent need for a [global platform](#):

- To [share the scarce knowledge](#) that we already have: it is applicable, valid and useful for all socio-economic and cultural contexts.
- To explore what more could be done or developed to advance knowledge of rare diseases at a global level.
- To [connect rare disease stakeholders](#) across borders and diseases.
- To [create synergies with other stakeholders](#) to mutually exchange knowledge and expertise.
- For [recognition and attention at the UN level](#), where rare diseases remain an area little explored, with great social and economic impact.

Common Goals:

Why is the
United Nations
the right
platform?



NGO COMMITTEE FOR
RARE DISEASES



Why the United Nations?



The right place...

For decades, the UN has been a driver of a **strong public health agenda, social development, inclusion, and progression, developments in science, technology and innovation, and human rights for all.**



Why the United Nations? ... at the right time

In November the UN adopted its **2030 Agenda for Sustainable Development**, including the adoption of the 17 **Sustainable Development Goals (SDGs)**.

There is a clear alignment of a number of goals with rare diseases, e.g;:





GOAL 1: END POVERTY IN ALL ITS FORMS EVERYWHERE

1.3 - Implement nationally appropriate **social protection systems and measures** for all, including floors, and by 2030 achieve substantial coverage of the poor and the **vulnerable**.

1.4 - By 2030, ensure that all men and women, in particular the poor and the **vulnerable, have equal rights to economic resources, as well as access to basic services**, ownership and control over land and other forms of property, inheritance, natural resources, appropriate new technology and financial services, including microfinance

- Exclusion from health and care systems
- Lacking social protection
- Inaccessible educational environment
- Lower employment rates
- Absence from work due to inaccessible labour markets for people living with rare diseases and family members
- Expensive treatments, special resources and adaptations
- Inability to live in dignity and autonomy with no income and costly adaptations to be autonomous



GOAL 3: ENSURE HEALTHY LIVES AND PROMOTE WELL-BEING FOR ALL AT ALL AGES

SDG Targets:

- **3.2** - By 2030, **end preventable deaths of newborns and children under 5 years of age**, with all countries aiming to reduce neonatal mortality to at least as low as 12 per 1,000 live births and under-5 mortality to at least as low as 25 per 1,000 live births
- **3.4** - By 2030, **reduce by one third premature mortality from non-communicable diseases** through prevention and treatment and promote mental health and well-being
- **3.8** - **Achieve universal health coverage**, including financial risk protection, **access to quality essential health-care services and access to safe, effective, quality and affordable essential medicines and vaccines** for all
- **3.b** - **Support the research and development of vaccines and medicines for the communicable and non-communicable diseases**
- Poor understanding of rare diseases and their needs as patients and experts are few and geographically scattered.
- Slow or misdiagnosis
- Highly complex, often degenerative and disabling
- Life expectancy is often severely affected
- Only 5% of rare diseases have an adequate treatment
- Unaffordable and inaccessible treatments
- Profound psychological burden
- Preventable deaths through newborn screening
- Lack of incentives to boost research and development of rare disease treatments.



GOAL 4: ENSURE INCLUSIVE AND EQUITABLE QUALITY EDUCATION AND PROMOTE LIFELONG LEARNING OPPORTUNITIES FOR ALL

SDG Targets:

- **4.2** - By 2030, ensure that all girls and boys have access to **quality early childhood development, care** and pre-primary education so that they are ready for primary education
- **4.5** - By 2030, **eliminate gender disparities in education** and ensure equal access to all levels of education and vocational training for the vulnerable, **including persons with disabilities**, indigenous peoples and children in vulnerable situations
- **4.a** - Build and upgrade **education facilities that are child, disability and gender sensitive** and provide safe, non-violent, inclusive and effective learning environments for all
- *50% of rare diseases affect children*
- *Difficulties to attend school and university and to develop in a quality manner due to rare diseases:*
 - *Negative impact on health*
 - *Frequent medical appointments*
 - *Inaccessibility of facilities*
 - *Inaccessible teaching methods*
- *Ineffective translation of medical information to teachers and educators who do not understand what the rare disease is, nor the impact it has and the needs it creates, limiting quality education*



GOAL 5: ACHIEVE GENDER EQUALITY AND EMPOWER ALL WOMEN AND GIRLS

SDG Targets:

- **5.1** - End all forms of discrimination against all women and girls everywhere
 - **5.c** - Adopt and strengthen sound policies and enforceable legislation for the promotion of gender equality and the empowerment of all women and girls at all levels
- **Primary carer role** for people living with rare diseases is mostly **assumed by the mother** ([ENSERio](#))
 - Mothers of children with a rare disability reported **high parental stress and high physical and emotional strain** ([Delve et al, 2006](#))
 - **Pain is considered less seriously in women than in men** ([EURORDIS Care](#)) e.g. Elhers Danlos, CF
 - **Women face more rejection in looking for a diagnosis** ([EURORDIS Care](#)), meaning diagnosis takes longer and treatment is delayed e.g. Crohns disease, Cystic Fibrosis
 - **Gender equality and women in science**



GOAL 10: REDUCE INEQUALITY WITHIN AND AMONG COUNTRIES

SDG Targets:

- **10.2** - By 2030, empower and promote the social, economic and political inclusion of all, irrespective of age, sex, disability, race, ethnicity, origin, religion or economic or other status
- **10.3** - Ensure equal opportunity and reduce inequalities of outcome, including by eliminating discriminatory laws, policies and practices and promoting appropriate legislation, policies and action in this regard
- *Health inequalities and disparities between regions in acknowledging rare diseases*
- *Invisibility of rare diseases within health care systems of LMIC*
- *Inequality in accessing health care services, and inequality of treatment and integration compared to more common diseases*
- *Worse quality of life and experience higher losses in terms of medical care and social and economic activities compared to more common diseases (Van Nispen 2003)*
- *Inequality in provisions of and price of treatments.*
- *Discrimination in the labour market*



GOAL 17: REVITALIZE THE GLOBAL PARTNERSHIP FOR SUSTAINABLE DEVELOPMENT

Capacity building

- *17.9 - Enhance international support for implementing effective and targeted capacity-building in developing countries to support national plans to implement all the sustainable development goals, including through North-South, South-South and triangular cooperation*

Multi-stakeholder partnerships

- *17.16 - Enhance the global partnership for sustainable development, complemented by multi-stakeholder partnerships that mobilize and share knowledge, expertise, technology and financial resources, to support the achievement of the sustainable development goals in all countries, in particular developing countries*
- *17.17 - Encourage and promote effective public, public-private and civil society partnerships, building on the experience and resourcing strategies of partnerships*

- *Rarity requires global collaboration*
- *Multistakeholder partnerships are necessary: public, private and civil society groups*
- *Patients and patient groups to be seen as partners*
- *The role of science, technology and innovation as an enabler of the post-2015 development agenda: rare diseases research drives innovation often beneficial to larger population groups.*

The NGO Committee for Rare Diseases



NGO COMMITTEE FOR
RARE DISEASES



- *Leave no one behind*

Introducing the Committee



NGO COMMITTEE FOR
RARE DISEASES

Mission

Promoting rare diseases on the global level

Vision

An inclusive, multi-stakeholder ecosystem to share knowledge and expertise about rare diseases and to increase global visibility of rare diseases

Introducing the Committee

Objectives:

- To **increase visibility** of rare diseases at the global level
- To **extend and share knowledge** about rare diseases and their unmet needs
- To **connect** NGOs interested in rare diseases and their partners within a global platform
- To **promote international, multi-stakeholder collaboration and actions** for rare diseases
- To **align** rare diseases as a global priority in public health, research and medical and social care policies

What will the Committee do?

Collect and share up-to-date information and research on the global dimensions of rare diseases

Develop and present reports, recommendations for actions and positions on rare diseases and associated issues

Host dedicated events to share information and to raise the profile of people living with rare diseases worldwide

Highlight current or potential opportunities for **improved and more integrated collaborations between all stakeholders**

Create synergies with other CoNGO committees and with NGOs in ECOSOC

Encourage all relevant UN bodies to **better reflect aspects related to rare diseases into their mission and actions**

Potential deliverables

- Two working meetings a year – one in Geneva (Spring), one in New York (Autumn)
- A Rare Diseases Atlas: capturing the landscape of rare disease information, policy and initiatives around the world.
- Short annual reports on specific issues of relevance to rare diseases
- Publications, media articles

- **... and more broadly, direct input into relevant policy initiatives or resolutions to be discussed within the UN system, at various levels (ECOSOC, WHO, UNESCO, IMF etc.)**

Introducing the Committee: Composition and Members



Our current timeline

October 2015



**Accepted
as a
CoNGO
Committee**

March 2016



**By-laws
approved by
CoNGO
president**

April 2016



**Establishing
contacts
within
UN and
member
states**

11, November 2016



**Inaugural
meeting**

SAVE THE DATE!



The Global Gathering for Rare Diseases: Inaugurating the NGO Committee for Rare Diseases (CfRD)

Friday 11 November 2016

Conference Room 8 (GA-1B-CR08), United Nations

New York, NY 10017, USA

Under the patronage of:

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

With the support of:

The Swedish Ministry of Health and Social Affairs

The Permanent Mission of Estonia

The French Minister of Social Affairs and Health (tbc)

The French Minister of Foreign Affairs and International Development (tbc)



NGO COMMITTEE FOR
RARE DISEASES



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

For information and registration:

www.ngocommitteerarediseases.org

TACK SÅ MYCKET



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