



NGO COMMITTEE FOR  
**RARE DISEASES**

# Rare diseases – A Common Cause at the Global Level

Anders Olauson

Agrenska – Chairman

A Committee of **CoNGO**



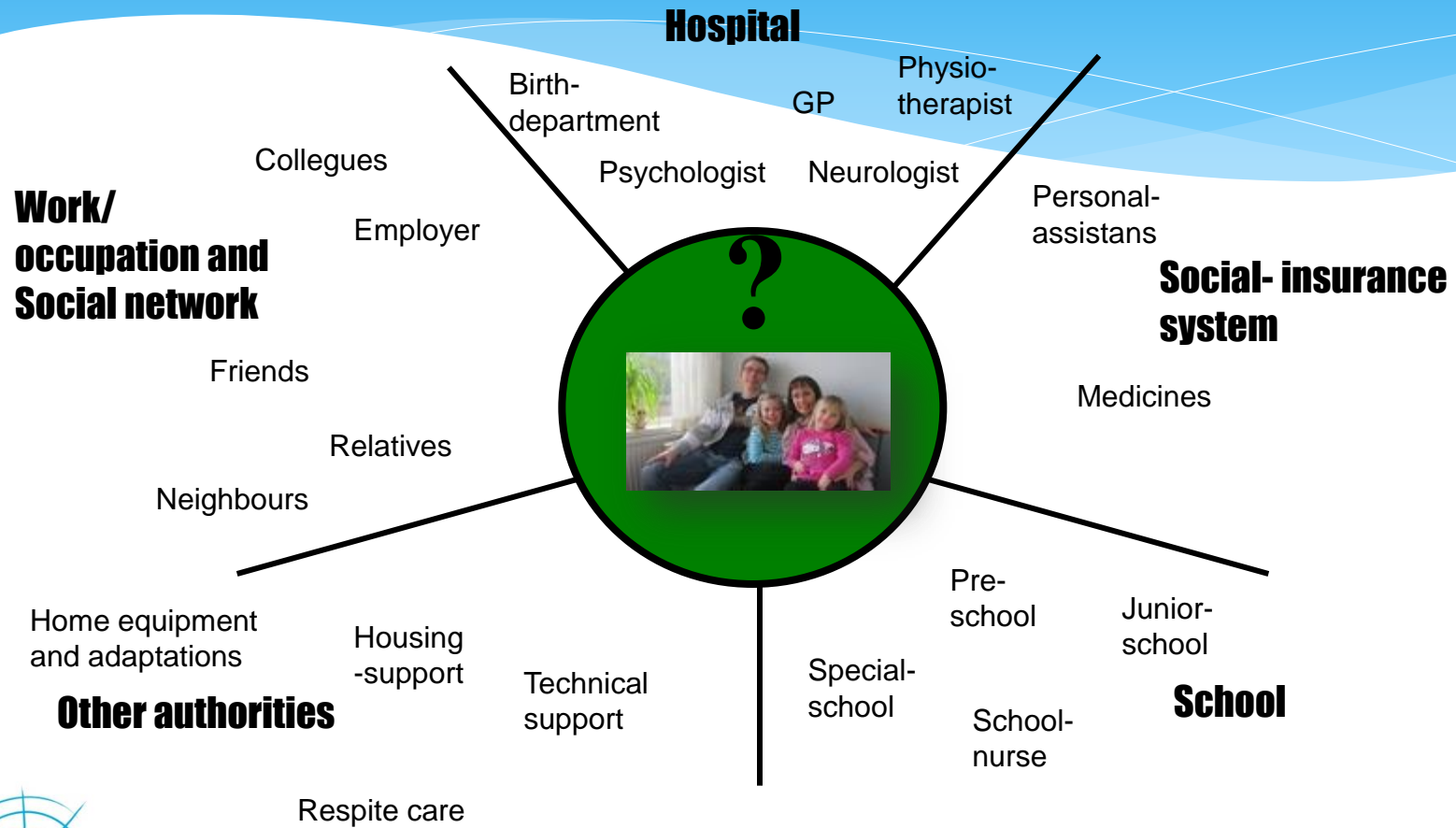
CoNGO  
The Conference of NGOs  
in Consultative Relationship  
with the United Nations



# The reality



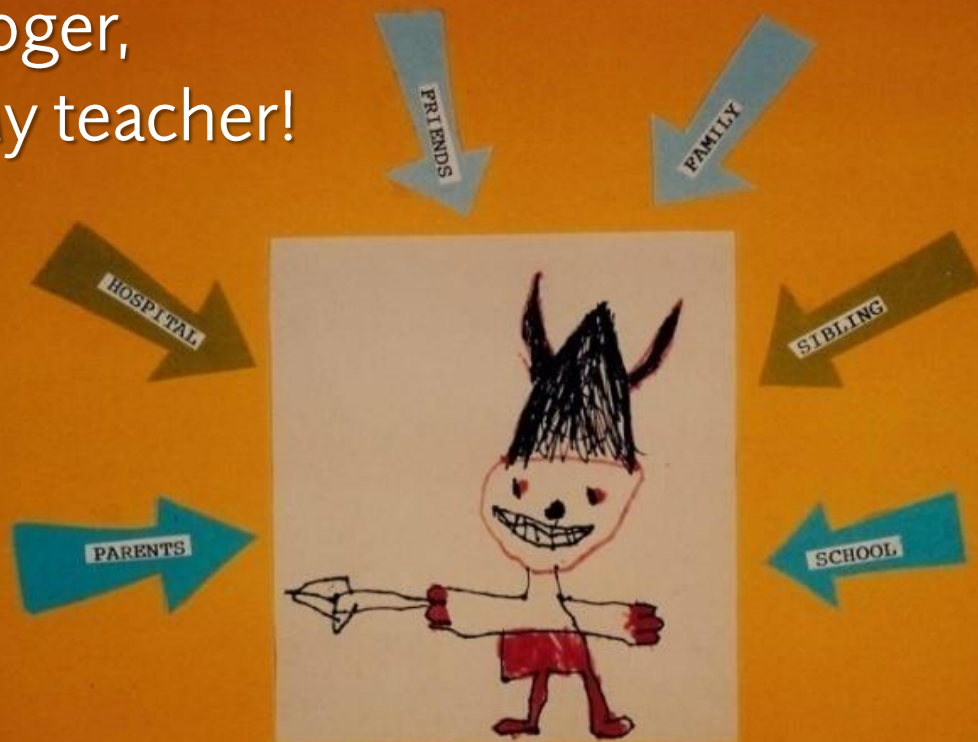
# The reality for a lot of families > 40 to 120 contacts





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Roger,  
My teacher!



Today's Ågrenska started by combine the following parties;

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





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



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



# 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 Nations Unies

NGO SECTION, DESA

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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](http://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.

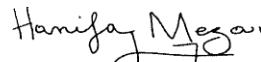
Please note, in particular, Parts II, IV, V and VII and the Council Resolution 1996/31, describing the procedures for carrying out your consultative relationship with the Council. Also, kindly note Part IX, paragraph 61c which requests that organizations in General and Special consultative status submit 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)**

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Sweden  
FAX: 46-31-911979

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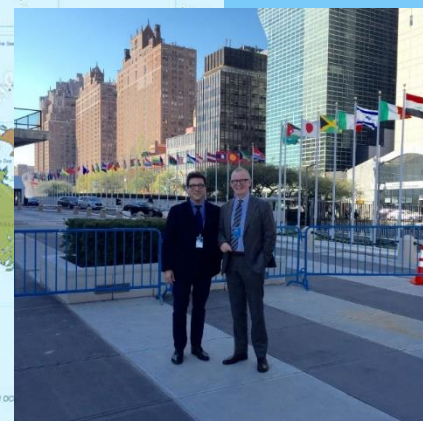






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# NGO Committee for Rare Diseases CfRD (UN, New York)



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# Common Challenges



# 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



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# 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.



# Why the United Nations?



World Health  
Organization



 **UN  
WOMEN**

## 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.



**unicef** 





# Why the United Nations?

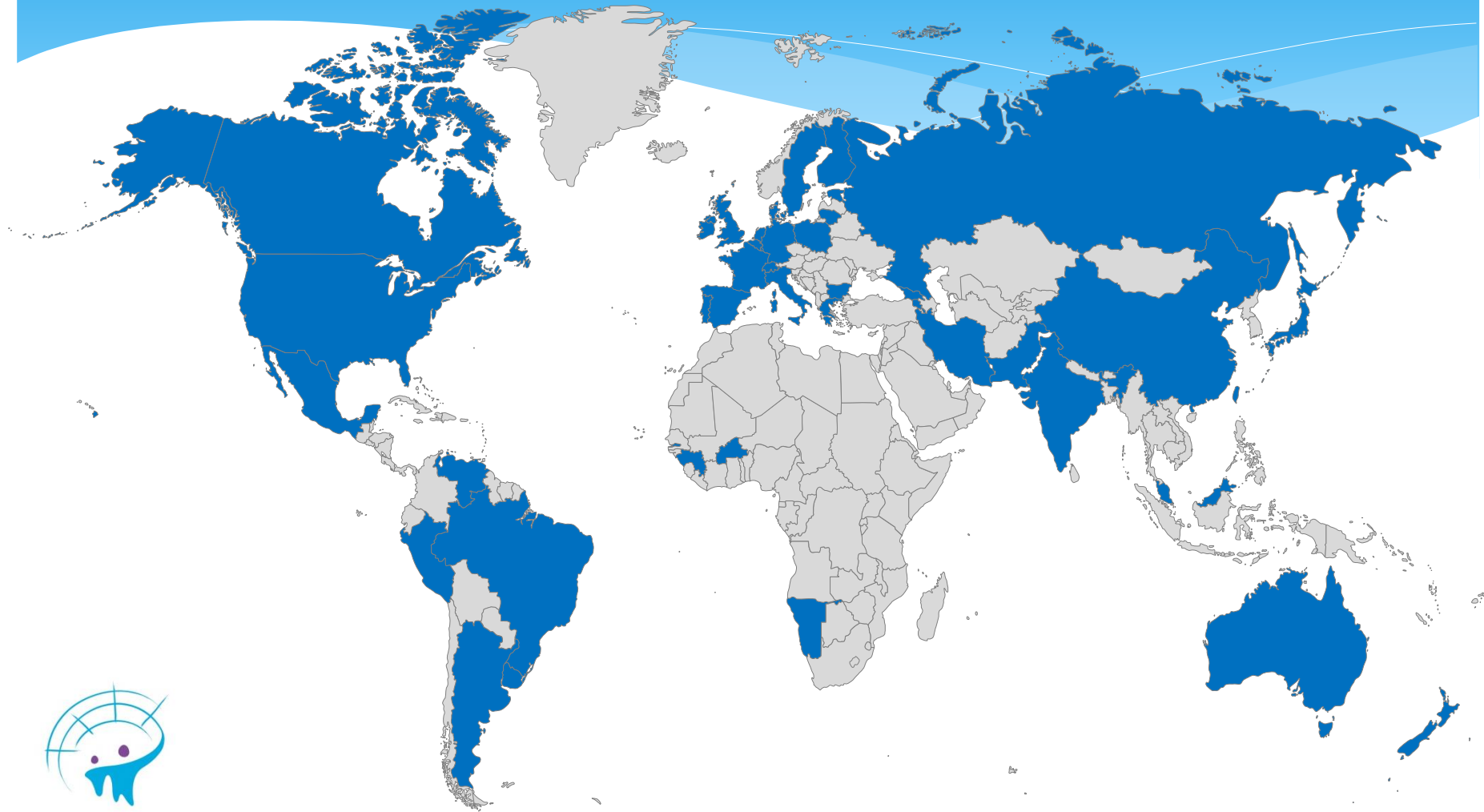
... at the right time

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



# A Global Gathering



THANK YOU VERY  
MUCH

