

# Global Approaches for Rare Diseases and Orphan Products

## XII. Meeting Patient and Family Needs Across the Lifespan – Access to Health Care, Psychological, and Social Support Programs



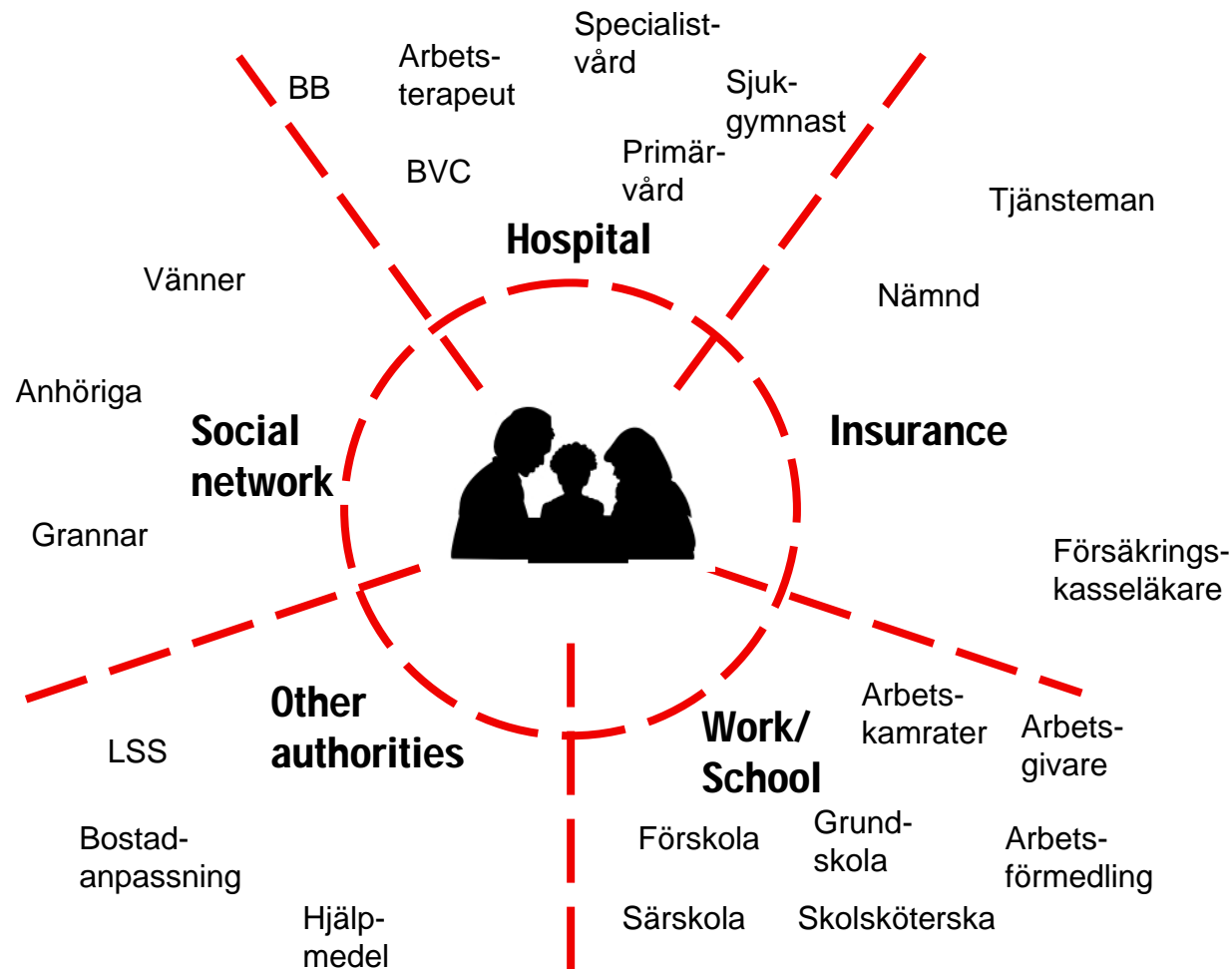
**Anders Olauson**  
AGRENSKA, Gothenburg  
EURORDIS, Paris  
EPF, Brussels

**Eesti Agrenska Foundation, Estonia**



Washington DC, May 21 2008

# When you have a rare disease > 40 contacts





- “In order to understand how it is to be a parent to a disabled child – you have to be a parent to a disabled child yourself!”

Åke Martinsson, Sweden



# The Family Program



## Needs to;

- Invite the whole family
- Have a holistic approach
- No shorter than 4 days
- Address one and the same diagnose
- Be part of the (Re)-hability process
- Offer a tailor-made program
- Have 4 parallel program:
  - The child with the diagnose
  - Parents
  - Siblings
  - Family related Professionals
- Make a documentation afterwards that has focus on the whole family

# Stress and well-being among parents of children with rare diseases: a prospective intervention study: 2000 - 2002

The Agrenska Family program an habilitation intervension  
for families with children with rare diseases

Lotta Dellve & Lena Samuelsson

A Tallborn, L R-M Hallberg, A Fasth, M Gunnarsson, A Josefsson



Nordic School  
of Public Health



Ågrenska



Sahlgrenska University Hospital  
Göteborg University

# A mother told me....



- Before I came here I felt that I didn't had any influence of my life and situation, but after this program and even still after 6 month I'm in charge again...

**Before attending the family program 43 %  
of the mothers felt disconnected.**

**After the family program the number was  
less than 7 %!**

## Effects – families tell:



- Parents feel "normal" for the first time
- The family feel empowered, by meeting other in the same situation
- Parents get new knowledge, to take better care of their own life
- Children with the disorder meet other in the same situation
- Siblings meet other siblings
- Professionals meet each other



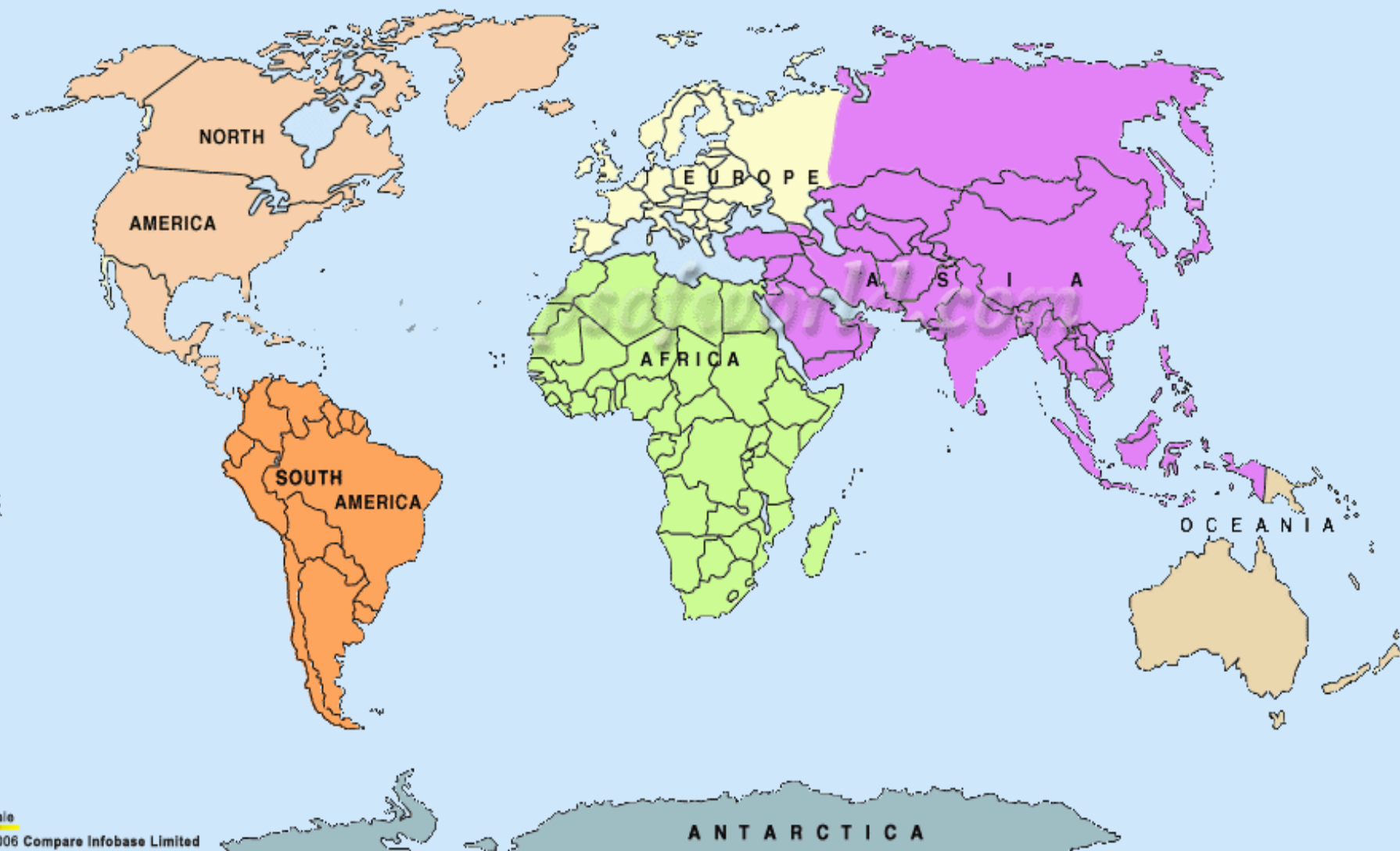
# Effects – scientific proof:



- **Reduced costs for healthcare**
- Attended family program - consumed medical care for 13.000 SEK/ year
- Attended ordinary program – consumed for 35.000 SEK/year

**45 Billions €**

# Continents of the WORLD



Map not to Scale  
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Washington DC, May 21 2008



## Agrenska Foundation is working under the patronage of Queen Silvia of Sweden

Queen Silvia inaugurates “the Queen Silvia Auditorium” at Agrenska Academy in 2001.

# Agrenska programs



- Family program
- Adult program
- Respite and summer service
- Educations
- ADHD counselor
- Family support unit
- Training program to get a job
- Staff who has a disability
- Eesti Agrenska
- Agrenska Friends
- AViA



# United Nations Nations Unies

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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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RV FACSLIN



# Comments from ICORD meeting in Brussels september 2007

## **The ICORD Network.**

- Yes, this is something we want to see developed to pursue items of interest to us all.
- Patient involvement in this network should build on partnership with professionals, academia and regulators to make progress. We should also provide challenges for governments and industry to improve their policies and actions.
- It should develop tools for countries and regions to assist their work e.g. guides and templates for best practice, goals, and ideas or models for groups to use.
- The network should build and maintain good communications among us all. And lead to good involvement of patient/family groups at all levels, including research, health policy, regulatory issues, and a role for us in priority setting.
- AViA website can be a tool we can use

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## The Agrenska Virtual International Academy

The Agrenska Academy is a virtual centre for information, research and the spreading of knowledge on rare diseases. This web site is still under working progress. This virtual gate to knowledge on rare disorders will be launched in spring 2006.

The main objectives of the Agrenska Academy are:

- To stimulate additional and deeper research on rare diseases - centred on the individual - that draws on the direct experience of the individuals and families afflicted.
- To initiate research on the value of the Family Program at Ågrenska, focusing on the societal values of such interventions.
- To ensure that the results from the research studio-projects and other important results from research institutes are widely disseminated to all important actors upon whom the individuals and their families are depending.
- To improve the welfare of patients with rare diseases and their families world-wide through better knowledge, research, care, information, education and awareness, ICORD society was formed on September 13 2007 in Brussels.

Within the Agrenska Academy a number of research studios are set up. They focus on different areas, but always with an holistic approach and presupposing lifelong consequences for the individual.

Scientific ideas, specific wishes and services could all be communicated at this site. It also offers a place for discussions and focused collaboration.



### News

» As a result of a need for an more global coordinating form for Rare Diseases ICORD was founded in 2007. ICORDs mission is to improve the welfare of patients with rare diseases and their families world-wide through better knowledge, research, care, information, education and awareness. ICORD is an International society for all individuals active in rare diseases and/or orphan drugs, including health care, research, academic, industry, patient organizations, regulatory authorities, health authorities, and public policy professionals.

» Under the control of the Swedish National Board of Health and Welfare there is an information centre for Rare Diseases. SmågruppsCentrum is a national information centre for rare diseases. The concept of rare diseases is defined in Sweden as "disorders or injuries resulting in extensive disability and affecting no more than one hundred individuals in one million inhabitants". The main mission of

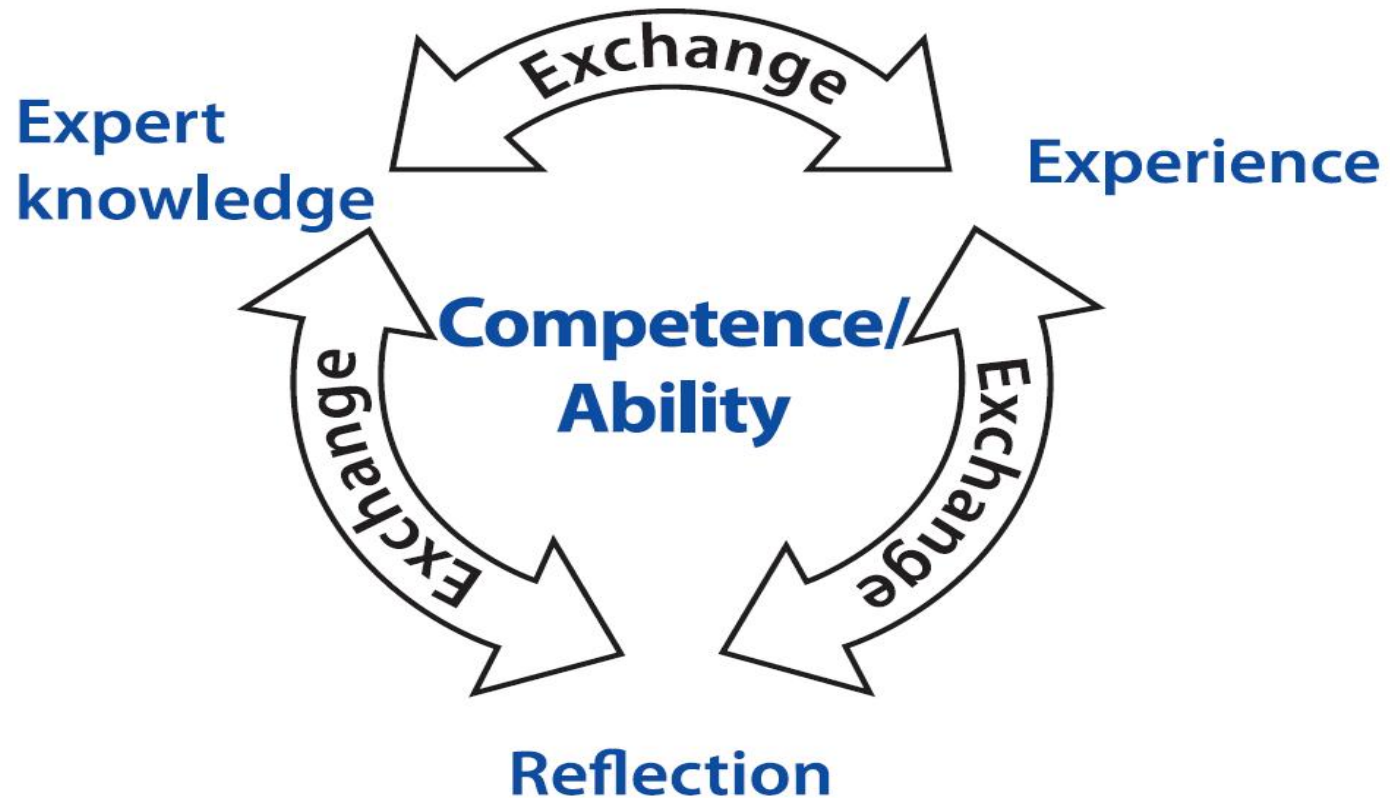


- Virtual centre for research
- Stimulate further and deeper research regarding rare diseases
- Based upon experiences from Agrenska programs
- Holistic perspective
- International cross boarder cooperation's
- Scientific council

# Educational consequences

People with syndrome diagnoses often represent educational challenges

# Empowerment





# Broad observation instrument

- shows data for groups, variation within groups, differences between groups
- reliability considered good  
professor Erland Hjelmquist and docent Bengt Jansson,  
Göteborg University
- theoretical validation work remains

# We want to map

- motor ability
- perception
- social behaviour
- emotional behaviour
- ADL
- language and speech
- learning strategies
- writing, reading and mathematics

# Diagnoses in our database



- Prader Willi (13)
- Angelman (12)
- Asperger (33)
- Tourette (20)
- Achondroplasi (12)
- Cornelia de Lange (6)
- Silver Russel (6)
- Fragile X (30)
- Congenital Dystrophia Myotonica (14)
- Ehlers Danlos (10)
- Marfan (11)
- Turner (10)
- Charge (5)
- Möbius (2)
- Treacher Collins (2)
- 22q11 deletion/ CATCH 22 (20)
- PKU/ Phenylketonuria (9)
- Apert (1)
- Galactosemia (4)
- Klinefelter (6)
- Neurofibromatosis (11)
- Noonan (10)
- Osteogenesis imperfecta (2)
- Rubenstein Taybi (8)
- Sotos (11)
- Sturge Weber (4)
- Tuberos sclerosis (14)
- William (17)
- Albinism (9)
- Becker (7)
- Crouzon (7)
- LMBB (9)
- Cri du chat (10)
- CDG – Congenital Disorders of Glycosylation (7)
- Hereditary spastic paraplegia (9)



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News

» Newstext will be here

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