Introducing Rare Diseases Hungary - Hungarian Federation of People with Rare and Congenital Diseases

Gábor Pogány Ph.D.
www.rirosz.hu

Huferdis

Charity Organisation

a member of

EURORDIS
Rare Diseases Europe

and

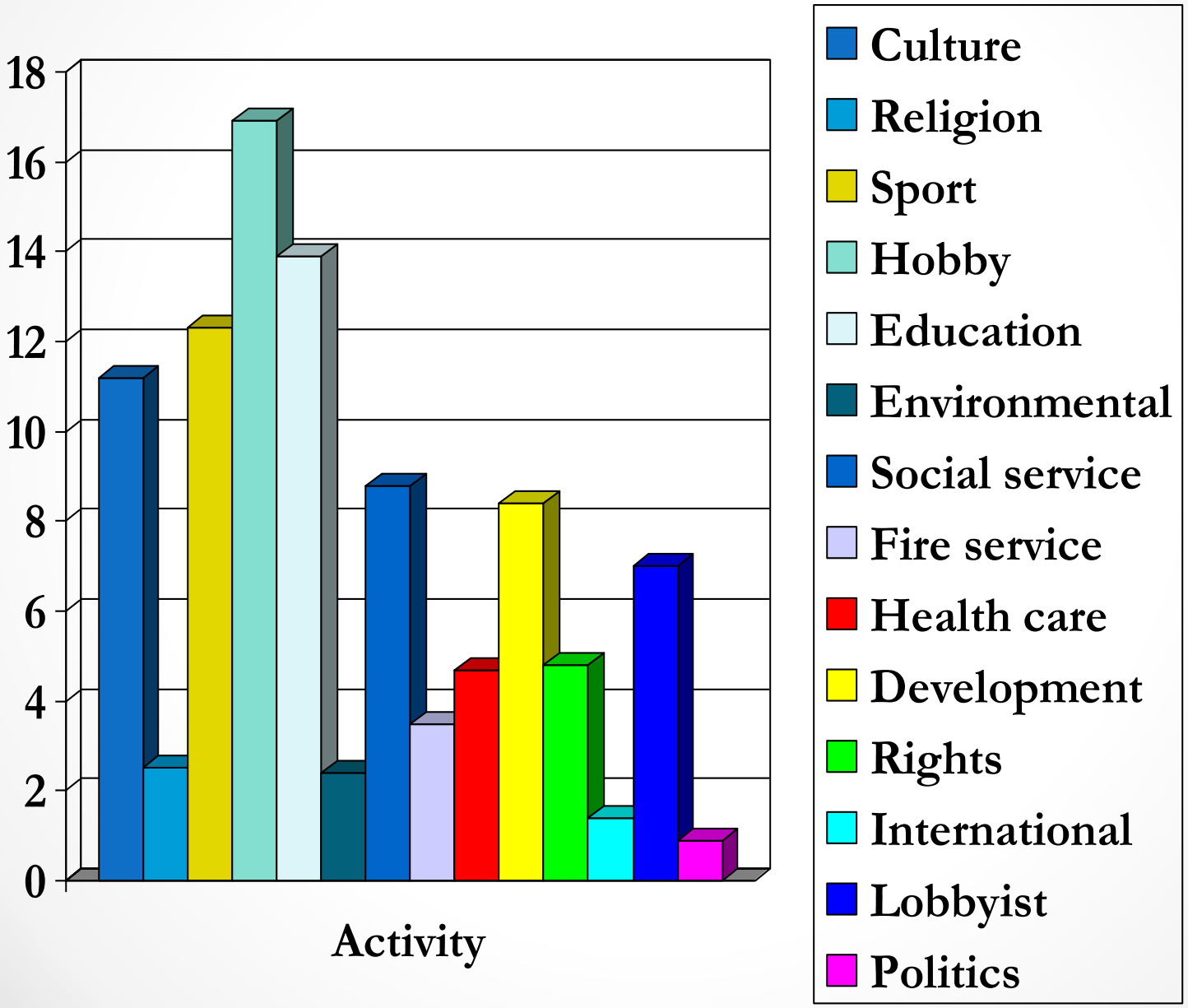
International Alliance of Patients' Organizations
A global voice for patients

Hungarian situation at the level of patient organisations:

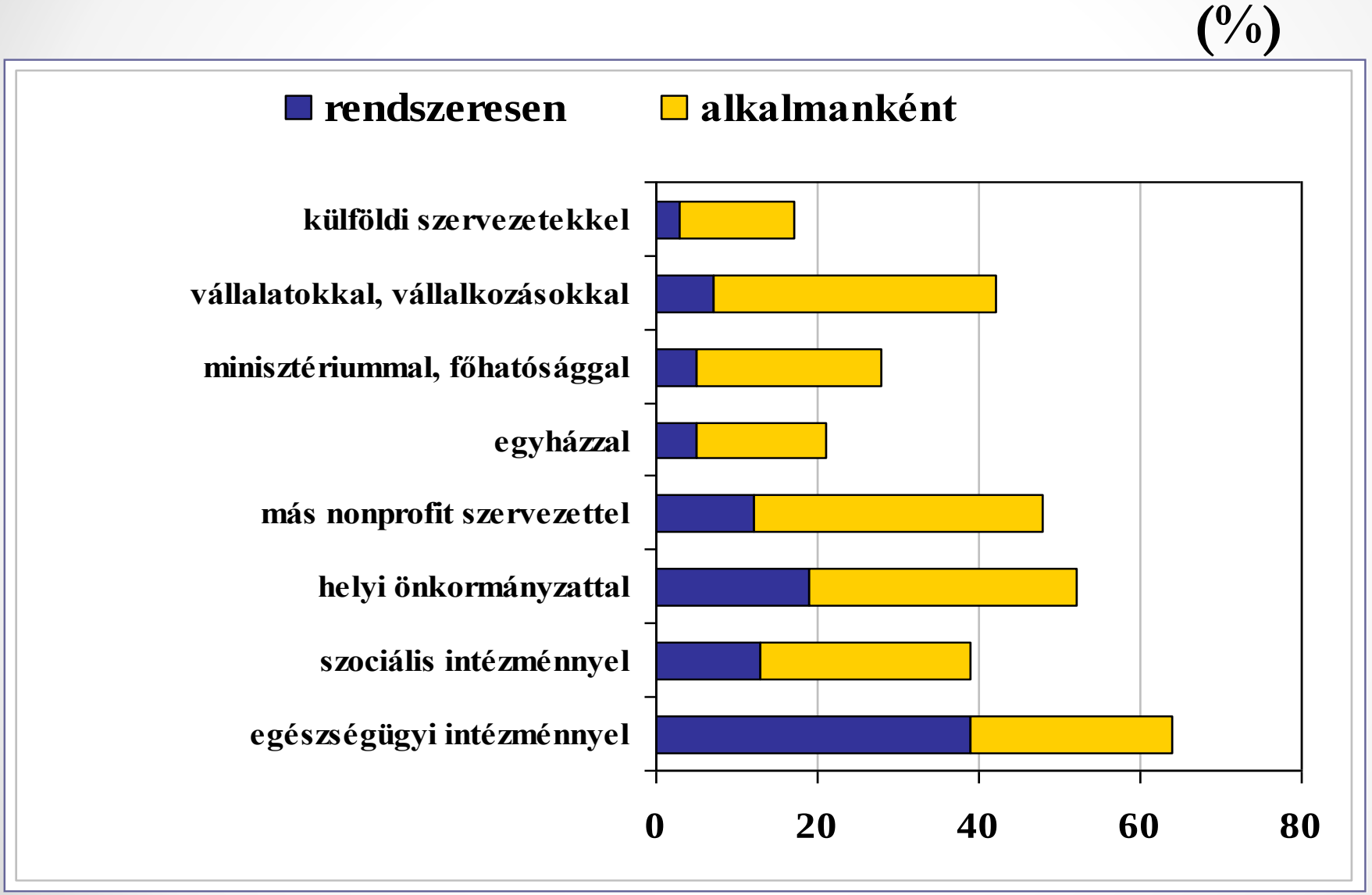
- Typical is the financial scarcity and uncertainty
- Combined with inappropriate infrastructural background and human resources!
- However, there are lots of unutilized possibility and capacity!
- And huge potential in the national and international collaboration!

Distribution of NGO-s - Relative low organizational level

(%) (Hungarian Central Statistical Office, 2006)



Cooperation of patient ngo-s:



(Péntek E., 2008)

There is only one solution!

More effective, professional operation with strategic approach!

The best way to achieve it by the help of national and international cooperation!

- Utilize common experience,
- Best practices,
- Finantial support,
- Possibility of common political pressure!

Therefore HUFERDIS was formed!

- To reach a critical mass from the point of view of decision makers and the society as well
- Represent a maximum number of different rare disease patients
- Cooperation, concentration of resources, better division of labour
- Unity, moderated competition
- Increase influence on national policy makers and authorities
- Share experiences, information and best practices
- Spread knowledge on rare diseases
- Represent our country's RD patients on international level

What is HUFERDIS?

- Rare Diseases Hungary - The Hungarian Federation of People with Rare and Congenital Diseases - (HUFERDIS) was created on 05.05.2006 after several years of preparatory work.
- It is a network of 45 Hungarian patient organisations and several individuals active in the field of rare diseases.
- The federation is dedicated to helping people with rare diseases and assisting the organisations that serve them.

Difficulties organising Alliances 1.

1. **The lack of time and energy –we need crazy people!**

   - **You need to fight against your disease**
   - **To work for your career**
   - **To deal with your family life**
   - **If you still have time – you can organise your disease specific association**
   - **Then, in your „remaining" time – you can start your umbrella organisation and you will have your alliance!**
   - **Finally, utilizing your „left over" energy and time – you can work in an international organisation as well ☺.**

- **But it is worth all the efforts!**

Difficulties organising Alliances 2.

2. Lack of money, financial uncertainty
3. Lack of human resources: rare full time job colleagues, difficult to activate volunteers
4. Too much administration and bureaucracy
5. Lack of patient supportive social environment
6. Lack of NGO supportive social environment
7. Lack of political support
8. Difficulties in the cooperation among NGO-s – conflicts, dissension, competition
9. Problems within the supportive grant system – too big work, too much self contribution, not enough transparency, unfair, problem insensitive, not well targeted, etc.
10. Low member activity and participation
11. Social disadvantages, poverty
12. Conflicts with other services, professions

But we did it ☺!

Our member associations at the present:

- CML and GIST Patients Association
- Cri Du Chat Syndrome Association
- Living with Congenital Heart Problems Association
- David Little People Association
- Epidermolysis Bullosa Foundation (DebRA Hungary)
- Foundation for Fabry-diseases
- Gergely Alexandra Foundation (for Ewing-disease)
- Gyógyító Jószándék Foundation – for children living with Muscular Dystrophy
- Angelman Syndrome Association
- Club for Prader-Willi Syndrome
- Ataxia Hungary Foundation
- Hungarian Hemofília Association
- Hungarian Muscular Dystrophy Association
- Hungarian Mucopolisaccharidosis Association
- Hungarian Porphyria Association
- Hungarian VHL Association
- Association Williams Syndrome Association
- Hungarian Angio-Oedema Association
- Hungarian Hemochromatosis Association
- Hungarian PKU Association
- Martin Bell Club
- Misko Foundation
- Myelodysplasia Association
- Myasthenia Gravis Association
- Mitochondrial Patient's Club
- Narcolepsy club
- Neurofibromatosis Association
- National Cystic Fibrosis Association
- National Scleroderma Association
- Primer Immunodeficiency Association
- Retina Hungary Association
- Retinoblastoma Association
- Rett Syndrome Foundation
- Tuberous Sclerosis Association
- Deaf and Blind Association (for Usher syndrome patients)
- SMA Association
- Smith-Lemli-Opitz Syndrome Foundation
- Turner Syndrome Club
- Wolf-Hirschhorn syndrome Club
- Pulmonary hypertension Association

International Relationships

Our Federation became the official partner of EURORDIS and the Council of European Federations, representing Hungary in 2007. EURORDIS is the European umbrella federation of people living with rare diseases.

In 2008 we were admitted by the IAPO (International Alliance of Patients' Organisations).

What is rare disease?

- A rare disease is a disease affecting less than 1 in 2000 citizens
- There are between 6,000 and 8,000 rare diseases
- Rare diseases are often chronic, progressive, degenerative, and often life-threatening
- Rare diseases are disabling: the quality of life of patients is often compromised by the lack or loss of autonomy
- High level of pain and suffering for the patient and his/her family
- No existing effective cure
- 75% of rare diseases affect children
- 30% of rare disease patients die before the age of 5
- 80% of rare diseases have identified genetic origins.
- Other rare diseases are the result of infections (bacterial or viral) , allergies and environmental causes, or are degenerative and proliferative.
- Information is missing
- Research is insufficient

Our Goals

- to increase the chance of rare disease patients for equal opportunities to promote their social reintegration.
- to help and coordinate the Hungarian rare disease patient groups.
- to raise public rare disease awareness and to advocate rare diseases as a public health issue.
- to organise common programmes to enhance of the lives of all persons affected by rare disorders. (conferences, patient support, social, welfare and educational services, disseminate information concerning treatment, and care)

Our Board Memebers:

President: Dr. Pogány Gábor pogany@rirosz.hu

Vice-president: Károly Fogarassy fogarassy@rirosz.hu

Vice-president: Süliné Dr. Vargha Helga vhl@rirosz.hu

Our achievements so far

- Organization of Conferences on Rare Diseases, along with press-campaigns.
- Organization of Rare Disease Days in Hungary
- Establishing an interactive, dynamic homepage, with web-based information service: www.rirosz.hu
- Publication of common books about rare diseases where all member associations had their own chapters. Their organisations and diseases were introduced, the latter also improved by sections written by field experts.
- Strategy Partner of the government National Plan for Rare Diseases Preparing Workgroup
- Launching international projects and researches

Some programmes of HUFERDIS:

- Europlan I-II (Joint Action) project (http://europlan.rirosz.hu)
- Polka project (http://polka.rirosz.hu)
- Burqol-RD project (www.burqol-rd.com)
- EurordisCare2 project
- Rare Disease Day (http://ritkanap.rirosz.hu)
- Communication protocol of bad news (http://protokoll.rirosz.hu)
- Special family camps
- Uni-Versum Habilitation Centre (http://ritkanap.rirosz.hu/az-uni-versum-koezpont)

We organise Rare Diseases Day with EURORDIS and the other European Federations

Annually, at the last weekend of February

www.rarediseaseday.org ritkanap.rirosz.hu

The goals:

- Make awareness about rare diseases and their affects about the patients life.
- Make the domestic service system better.
- Gather funds for (Re)Habilitation Centre for Rare Diseases (like Frambu in Norway

A rare day for very special people.

Uni-Versum Center

A National Habilitation, Development and Social Service Centre for Rare Diseases in order to help rare diseases patients' social integration.

- Make up for a multidisciplinary service centre upon on Scandinavian good practices where the different developing methods and possibilities could be collected.
- Beside early developing, here you could get life-long support, care and service.
- After getting the diagnosis the families could move in the centre for a while and they could be informed and they could get advices about conducting their life.

UNI-VERSUM

EUROPLAN PROJECT

- EUROPLAN is an initiative to support National Strategies and Plans for Rare Diseases in all EU Member States.
- **EUROPLAN: a joint action for National Strategies and Plans for Rare Diseases in all EU Member states**
- That is, information on the different steps and priority areas of interventions in the field of rare diseases. The project is funded by the European Commission (DG SANCO). Basically, it aims at providing a tool box of methodology, best practices, case studies and indicators to help each EU Member State define their national plan for rare diseases. EUROPLAN includes 25 countries and EURORDIS as a main partner representing patients.
- We already participated in the 1st and 2nd round of the project organising 4 conferences and writing a Report about the Hungarian situation in 2010.

http://europlan.rirosz.hu

http://www.eurordis.org/content/europlan-project

POLKA project

- Strategies and plans for rare diseases are currently being developed by the European Union and many of its Member States. EURORDIS, the voice of all rare disease patients in Europe, believes that patient input into this process is of the utmost importance. EURORDIS' newest project, Polka[1], launched in September 2008, has been set up to respond to this objective.
- The project will facilitate the consultation of the European rare disease community at large, with the aim of building consensus on preferred public health policy scenarios for rare diseases.
- Patient debates on five to seven carefully chosen, rare disease public health policy themes will be launched using a variety of methods such as the Play Decide game or Delphi[2]-like methods.
- The game was developed in 2004 and launched in 2006 by a consortium of four science centres and museums[3], with the support of Ecsite, the European Network of Science Centres and Museums, and NEF, the New Economics Foundation.
- Moderators in various Member States will organise debates with up to eight patient representatives per debate. In total, the project should facilitate between 600 and 1000 discussions across 27 countries, in 21 languages, with a minimum of 80 participants per country!

http://www.eurordis.org/content/polka-patients-consensus-preferred-policy-scenarii-rare-disease

http://polka.rirosz.hu/

„How to tell the Diagnosis?" protocol

- Getting diagnosis is very difficult and hectic mainly for Rare Diseas patients. There are many late and miss diagnosis and often when they get right one, the circumstances and the content of telling the diagnosis are inadequate.
- Therefore a workgroup of patients and experts worked on to collect experiences and good pracices about the suitable method.
- Our protocol was published at our 2nd Europlan Conference in Budapest.

Here you can find details: http://protokoll.rirosz.hu/

Burquol-RD

- BURQOL-RD is a 3 year project under the 2nd Programme of Community Action in the Field of Public Health, that commenced in April 2010 and is promoted by the DG Sanco.
- The main aim of BURQOL-RD is to generate a model to quantify the socio-economic costs and Health Related Quality of Life (HRQOL) , of both patients and caregivers, for up to 10 rare diseases in different European countries.
- This model will be adaptable and sufficiently sensitive to capture the differences in the distinct Health and Social Care Systems in the EU Member States. Our hope is that, the information generated by the BURQOL-RD consortium will help to:
- Design future policies in the area of rare diseases, which will ultimately have positive benefits for EU citizens health, both that of patients and of their caregivers. Readily transfer the protocols established to other RD and to other countries.
  Compare the availability and access to specific health resources for specific RD in each country.
- Explore the potential relationships between HRQL and access to healthcare resources.

http://burqol-rd.com/

Results on the policy area

- A decree of the Ministry of Health established the National Rare Disease Centre (NRDC) in Hungary in 2008 as a part of the National Centre for Healthcare Audit and Inspection (OSZMK). It is reorganized within the National Institute for Health Development.
- The National Rare Disease Research Coordination Centre was created in the University of Pécs, in 2009.
- The Expert Committee on Rare Diseases was formed in 2009.
- The development of Hungarian National Plan / Strategy for rare diseases started in 2010 initiated by the EUROPLAN programme of HUFERDIS.
- A National Coordinator of the Hungarian RD National Plan was officially nominated in 2011. The National Plan Organising Committee formed, and started to develop the National Plan with his leadership. The Plan was signed by the minister in the October of 2013.

Thank you for your attention!

www.rirosz.hu, mentoov.rirosz.hu, www.nemzetibetegforum.hu

Rare Diseases Hungary

e-mail: pogany@rirosz.hu
Cím: H-1089, Budapest, Orczy út 2.
Tel: (36-1) 326-7492, Fax: (36-1) 438-0739

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HAPO
HUNGARIAN ALLIANCE OF PATIENT ORGANIZATIONS

NBF