



The European Conference on Rare Diseases ●●●●
The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Patient involvement and empowerment through the NPRD (Eastern Europe)

Dorica Dan - RONARD



Alianța Națională pentru Bolii Rare România



Questions to be answered

1. Which were the main steps in designing the National Plans for Rare Diseases in EE countries?
2. Who initiated the National Plans?
3. How do Patient Organizations advocate to adopt the Plans in EE? Do these actions empower POs?



The European Conference on Rare Diseases



The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



EURORDIS
Rare Diseases Europe

Involvement, integrity and impact

- The act or fact of participating, sharing and mobilizing resources for long-term impact projects.
- PO have a strong sense of social responsibility and a determination to bring about positive change.
- *....."And if you give... you will surely be rewarded"*



The European Conference on Rare Diseases 
The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Bulgaria

- **On 1 January 2009** the National plan for rare diseases (genetic diseases, congenital malformations and non-hereditary diseases) was **officially launched**.
- The strategy builds on the foundations laid down in the draft for rare diseases and orphan drugs program which was prepared by BAPES and proposed to the Bulgarian Ministry of health on 11 November 2004.



The European Conference on Rare Diseases 

The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



EURORDIS
Rare Diseases Europe

Bulgaria...

- The national plan includes **9 priorities**
- The total budget for implementation of the program activities is 11 294 515 Eur.



The European Conference on Rare Diseases 

The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Romania

- On 9th of August 2007, RPWA has established the RONARD – and organized the first working groups on a National Plan for RD;
- 1-2 November 2007, organized the National Conference on RD – *From evaluation of the needs to establish priorities in RD*;
- 29 February 2008– signed a partnership agreement with MoH – to finalize and implement a NPRD with 6 priorities;
- June 2008 – **the first national program on RD started**;



The European Conference on Rare Diseases 

The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Romania..

At the end of 2008, RPWA initiated a **partnership with MoH** and applied for a grant from **Innovation Norway** in partnership with **Ministry of Health Romania**

- ***to improve the quality of life for people affected by rare diseases in Romania through a comprehensive and accessible network of facilities and resources as set forth by the National Plan for Rare Diseases.***
- ***Budget for national program in 2010: 170.000.000 lei = 41.463.414 eur***
- Continuous changes at political level slowed down the progress.



The European Conference on Rare Diseases 

The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Hungary

- Several **improvements** in **2009**, but still before designing the Plan:
 - the National Centre of Surveillance for Congenital Anomalies and Rare Diseases has been formed (responsible for the development of a national plan).
 - The National Institute of Research Coordination for Rare Diseases was created at the University of Pécs.
 - Hungarian Rare Diseases Task Force was created by the Hungarian Ministry of Health to promote the implementation of EU recommendations.



The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow
The European Conference on Rare Diseases ●●●●



Hungary...

- Up to this point mainly the HUFERDIS and the enthusiastic professionals pushed it, **because there are four running national health plans, and the limited health budgets and resources are not enough for a new one.**
- Several good services are available in the forming Centers of Excellence for Rare Diseases, but demand remains much higher.
- Continuous changes at political level slowed down the progress.



The European Conference on Rare Diseases 
The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Patient involvement

- In all three countries, patient organizations have *started the fight for a national strategy for RD;*
- They organized information campaigns in the context of Rare Diseases Day with stronger and stronger impact, initiated information networks and help lines, contributed in designing social and medical health care services, initiated therapies, respite care, involve in research and clinical trials, etc.



The European Conference on Rare Diseases 

The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Patient involvement

Organized working groups with members of the national alliances, involved professionals and advocated at national level;

- **Still problems:** lack of funding, poor communication, patients are most of the time too focused on their own diseases



The European Conference on Rare Diseases 
The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



What do we need?

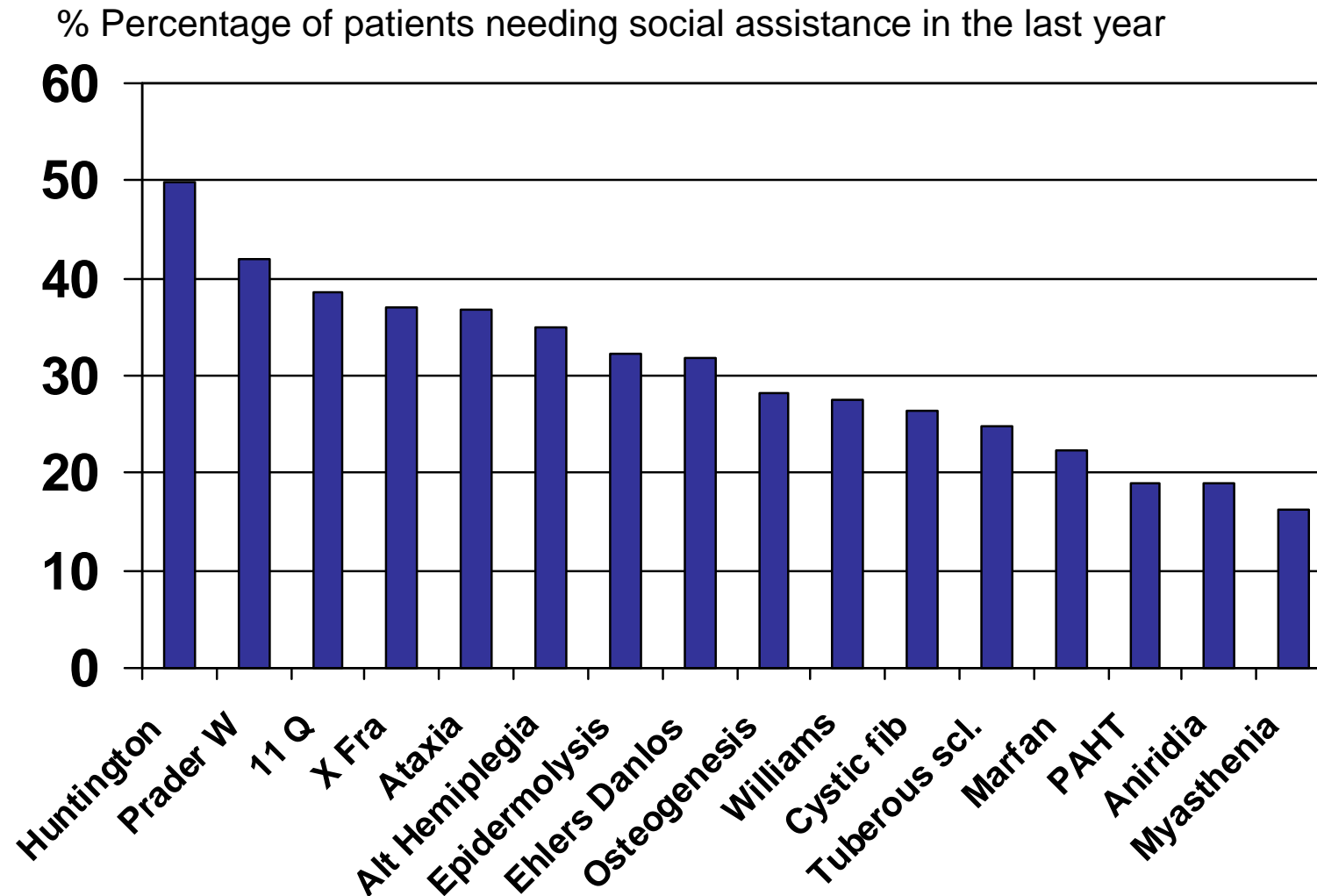
- Patient organizations (alliance) and medical professionals are the main engine for drafting and lobbying for a national strategy on RD.
- The plan should be promoted both to parliament members (legislative power) and Ministry of Health (operational health management).
 - ***Rare disease patients need social assistance, as much as medical care !!!! – more “actors” have to be involved;***



The European Conference on Rare Diseases 
The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



The need for social assistance varies according to the disease



How to do it?

- EURORDIS had empowered POs offering specific tools for lobby and advocacy;
- Organizing events in the same time at EU level made our voices stronger;
- Even if not legally binding for Member States, Recommendations and Communications carry much political weight that empower our activities at national level;
- We have now the best tool to further promote rare diseases as a public health and research priority;



The European Conference on Rare Diseases 

The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Realities

- Advocacy of patients is recognized more and more as an important element in defining policies on rare diseases;
- All the three countries have been established national alliances for rare diseases;
- RD Alliances are influencing decisions making processes in the field of rare diseases in these countries;



The European Conference on Rare Diseases 
The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Concrete actions:

Hungary, Romania and Bulgaria are involved in the elaboration of the national plans: ***Bulgaria already have adopted it and have a budget allocated!!!***

- **How to ensure, through appropriate funding mechanisms, patient representativeness in decision-making processes relevant to RDs?**
- This is not an easy answer



The European Conference on Rare Diseases

The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Patient empowerment

- **Patient empowerment** concept: people must bring about changes in their social situations and in the environment that influences their lives, not only in their personal behavior.
 - **Can we talk about Patient empowerment in EE countries in this context?**
 - **3 different countries, same problems and limits and most probably not so different realities;**



The European Conference on Rare Diseases 
The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow



Conclusions:

- Learning from RD patients by listening to their daily experience is a vital component in the process of improving the quality of health services designed for RD patients.
- Patients have a right to know and a right to be heard.
- Trust and respect are critical components of partnership.

Conclusions:

POs and professional organizations are working together in EE countries:

- **to build** the rare disease community;
- **to advocate** for patients and raise awareness on rare diseases;
- **to improve** access to care for patients and help develop public health policies;
- **to foster** therapeutic development and research;
- and for funding our activities;

Helping others you help yourself!



The European Conference on Rare Diseases 
The 5th European Conference on Rare Diseases - 13 to 15 May 2010 in Krakow

