

Sharing best practices on integrative approach
to rare diseases in different countries



The Croatian Society of Patients with Rare Diseases

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Strategies for rare diseases

- **The definition used in the country is –**
a rare disease affects less than 5 patients on 10 000 inhabitants
- **There is no National plan for rare diseases** (the only initiative to create one is coming from patient organizations)
- **There is no law regarding orphan drugs or rare diseases** (nevertheless, in 2001. we established a special fund for treatment of rare diseases and providing of “expensive therapies”)
- **Society’s main goal is improving the quality of life for patients suffering from rare diseases as well as their families.**
- In order to do so we need to establish wider range of activities regarding rare diseases on a national level , both providing a national standard of care (exp. Developing of an National plan for rare diseases, organizing National conferences on rare diseases, establishing an Orphan drugs fund etc.) as well as acting in a local community, again nation wide.



Funding of actions, regarding rare diseases



In Croatia following services are offered free of charge:

- Prenatal screening
- Neonatal screening
- Diagnostic genetic services
- Treatment (clinical management)
- Orphan drugs (there is no special designation for orphan drugs in Croatia)
- Rehabilitation
- Social care services

Provision of information for rare diseases

In the year 2007. The Croatian Society for Patients with Rare Diseases established a Rare Disease Information Centre

- *Located in the centre of Zagreb our office provides –*
- **Medical advice** (information on diseases, diagnostics help, second opinion, international medical services).
- **Social care advice** (information on social services provided for the rare diseases patients and their families).
- **Psychological support** (through organizing annual meetings for our members, one to one talk, organizing smaller support groups within one diagnosis).
- **Advocacy and raising awareness** (advocating and lobbying for the specific rights and needs of rare diseases patients with the Croatian government institutions, providing information on rare diseases to the professionals and the general public).
- **Media support** (making information on rare diseases and patients problems public, specific campaigns – exp. Rare Diseases Day).



Research on rare diseases



- *Croatia: No research program for rare diseases is implemented*

Prevention



Following prevention measures are regularly implemented in Croatian health system:

- Prenatal screening
- Neonatal screening
- Delay of diagnosis : Gaucher disease 5-7 years, Fabry disease 8-10 years

Empowerment of patients organisations



Society 's main activities:

- Supporting patients and their families in their efforts to solve their everyday life problems.
- Organizing gatherings for patients, their families and others interested in exchanging experiences and organizing problem-solving efforts in relation to rare diseases.
- Cooperating with health organizations and other relevant stakeholders in order to solve the particular issues that patients face.
- Organizing lectures and public gatherings through which foreign and domestic health experts can provide information and education, especially in terms of new developments in the treatment of rare diseases.
- Making a center for receiving and distributing rare disease data, using telephone technology and flyers.
- Publishing material concerning rare diseases and the activities of our organization.

Specialised social services



Wide range of social services provided by the –

- Government (national level)
- Local communities (the regional administration or the cities)
- **Some examples are – the disability compensation, work related rights for the parents with a child with disability etc.**
- **Of course, given the specific rareness of some diagnosis there is an growing issue on how to make the system recognize the specific needs of rare diseases patients.**



Thank you!



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