

***EUROPLAN***  
***National Conferences***

**I. CROATIAN CONFERENCE ON RARE DISEASES**  
**FINAL REPORT**

**I. General information**

<b>Country</b>	<b>Croatia</b>
<b>Date &amp; place of the National Conference</b>	<b>17.09.-19.09.2010., Hotel Palace, Dubrovnik</b>
<b>Website</b>	<b><a href="http://www.rijetke-bolesti.hr">www.rijetke-bolesti.hr</a></b>
<b>Organisers</b>	<b>Croatian Society for Rare Diseases, DEBRA – Croatian Epidermolysis Bullosa Association, Cancer Patient Association „Za novi dan“, Croatian Cystic Fibrosis Association, Association of Persons with Prader-Willi Syndrome Croatia</b>
<b>Members of the Steering Committee</b>	<ul style="list-style-type: none"> <li>• Mrs. Christel Nourissier (EURORDIS),</li> <li>• Mrs. Vesna Skulic (Croatian Society for Rare Diseases),</li> <li>• Mrs. Vlasta Zmazek (DEBRA Croatia),</li> <li>• Mrs. Anja Kladar (Croatian Cystic Fibrosis Association),</li> <li>• Mrs. Ivka Čop (Association of Persons with Prader-Willi Syndrome Croatia),</li> <li>• Mrs. Katarina Katavic (Cancer patient association „Za novi dan“),</li> <li>• Dr. Ante Zvonimir Golem (Ministry of Health and Social Welfare),</li> <li>• Prof.dr. Mirando Mrsic (Croatian Society for Rare Diseases, EURORDIS)</li> <li>• Prof.dr.sc. Ingeborg Barisic (Croatian Medical Association – Croatian Society for Rare Diseases),</li> <li>• Prof.dr.sc. Ana Stavljenic-Rukavina (ORPHANET),</li> <li>• Doc.dr.sc. Fedor Santek (University Hospital</li> </ul>

	Centre Zagreb)
<b>Names and list of Workshops</b>	<p><b>W1 Methodology and Governance of a National Plan/Strategy (NP)</b></p> <p><b>W2 Patient Empowerment</b></p> <p><b>W3 Definition, codification and inventorying of RD</b></p> <p><b>W4 Standards of care for RD</b></p> <p><b>W5 RD Patients' rights in social welfare system</b></p>
<b>Chairs and Rapporteurs of Workshops</b>	<p><b>Chairs:</b></p> <p><b>W1 Prof.dr. Mirando Mrsic</b></p> <p><b>W2 Mrs. Vlasta Zmazek</b></p> <p><b>W3 Prof.dr.sc. Ingeborg Barisic</b></p> <p><b>W4 Mrs. Anja Kladar, Mrs. Gabriele Pohla Gubo, Prof.dr. Mirando Mrsic</b></p> <p><b>W5 Prof.dr.sc. Ana Stavljenic-Rukavina</b></p> <p><b>W6 Mrs. Vesna Skulic, Mrs. Anja Kladar</b></p> <p><b>Rapporteurs:</b></p> <p><b>Mr. Mario Harapin, Mrs. Andreja Santek</b></p>
<b>Attachments (programme, list of participants, etc.)</b>	<b>Programme</b>

### Official opening

On 17<sup>th</sup> September in Hotel Palace in Dubrovnik, Croatia I. National Conference on Rare Diseases was opened by video message from The President of Croatia Prof.dr.sc. Ivo Josipovic and opening speeches made by representatives of Croatian Society for Rare Diseases, Ministry of Health and Social Welfare and European Organisation for Rare Diseases.

Organised by The Croatian Society for Rare Diseases and its member organisations under the auspices of the Croatian President prof. dr. sc. Ivo Josipovic and with support of the European Organisation for Rare Diseases - EURORDIS the three day Conference lasted till mid-day of 19<sup>th</sup> September 2010 with involvement of more than 180 participants.

---

## The plenary sessions

In the plenary session of the Conference, participants were addressed by former president of the Croatian Society for Rare Diseases Mrs. Vesna Skulic and the representative of the Ministry of Health and Social Welfare dr. Dubravko Bajramovic. The Secretary General of EURORDIS, Christel Nourissier presented the EUROPLAN project and European strategy for rare diseases.

Professor Ingeborg Barisic gave a short overview of Croatia's current situation in the field of rare diseases, pointing out the newly established National Commission for Rare Diseases whose main goal is development of the National plan for rare diseases. The members of the Commission, who represent different fields of work with rare diseases patients (medical and social welfare professionals from the Ministry of Health and Social Welfare, rare disease patient organisation representatives, Croatian Institute for Health Insurance representatives and medical staff from University Hospital Centre Zagreb and the Children's Hospital) were announced on Rare Disease Day 2010 and have been working together ever since.

Member of the Steering Committee of the Croatian Society for Rare Diseases and a member of EURORDIS' Board of Directors prof.dr. Mirando Mrsic stated that people with rare diseases deserve the support, care and all that modern medicine can provide to improve their health and to fully achieve parity with others. The Conference was opened by president of the Cancer patient association "Za novi dan" and vice-president of The Croatian Society for Rare Diseases, Mrs. Katarina Katavic.

Croatian EUROPLAN National Conference for Rare Diseases welcomed 180 participants, most of whom were patients and family members; patient organisation representatives from 15 different Croatian patient associations along with medical professionals; representatives from the pharmaceutical industry; representatives from public health institutions; and representatives from education institutions; who presented the main issues and priorities of patients with rare diseases in our country.

The Conference was attended by foreign experts and representatives of eight civil organisations from Slovenia, Bosnia and Herzegovina and Serbia, to exchange experiences and establish cooperation.

## II. Main Report

### *Theme 1 - Methodology and Governance of a National Plan/Strategy (NP)*

#### Sub-Themes

1. Mapping exercise before developing a National Plan
2. Development and structure of a National Plan / Strategy
3. Governance of a National Plan
4. Monitoring the National Plan
5. Sustainability of the National Plan

#### **Workshop 1**

**Chair: Prof.dr. Mirando Mrsic**

**Rapporteur: Mrs. Andreja Santek**

**Date, time, place: 18.09.2010., 11:30 - 13:00, hall Mare 1 in Hotel Palace, Dubrovnik**

**Participants: 86**

#### **Mapping exercise before developing a NP:**

Progress in raising public awareness about rare diseases in Croatia has been achieved by medical professionals in collaboration with patients, their families and patients associations. On the part of the patient organisations the active member of the community is Croatian Society for Rare Diseases, also very active in the work of European Organisation for Rare Diseases (EURORDIS) from the inception of their activities. The main goal of the organisation is to help patients and their families improve their quality of life.

When talking of the medical professionals working in the field of rare diseases, for years we have been relying on the impact of a few enthusiasts. Today we have the Croatian Society for Rare Diseases established within the Croatian Medical Association, the main medical organisation in the country, where every specialty dealing with rare disease patients has its representative in the Society.

There are also various associations of rare diseases, including the Croatian Association for cystic fibrosis, the Croatian Association of Osteogenesis Imperfecta, Association of persons with Prader-Willi syndrome Croatia, DEBRA – Croatian Epidermolysis Bullosa Association, the Cancer patient association "Za novi dan", Croatian association of people with scleroderma and others.

Since 2008 with the support and guidance of EURORDIS and The Croatian Society for Rare Diseases, in Croatia as well as in the rest of the world, the last day of February has been marked as the Rare Disease Day. After strong efforts made by the patients and the medical society what is needed for future improvement of the lives of RD patients is firmer support by the Ministry of Health and Social Care in implementing public health actions aimed at raising awareness of rare diseases.

In Croatia we currently do not have official epidemiological data on rare diseases or registry for rare diseases.

Some patients who suffer from rare diseases encounter difficulties in access to health care at the expense of the Croatian Institute for Health Insurance. Certain medical products (medicines, medical equipment) are not insured by the Croatian Institute for Health Insurance for those suffering from rare diseases. To this are added the different practices within the medical services of individual hospitals. The Croatian Institute for Health Insurance should recognise the rare disease as a specific entity. Rare diseases are rarely diagnosed, and some diseases patients wait for 10 years until having their illness correctly diagnosed.

Croatia is a relatively small country, with 4.5 million inhabitants. The creation of Centres of Expertise for certain rare diseases seem complicated, expensive and unnecessary. What we should discuss is establishing a network of physicians who have chosen to specialise in a particular disease, which should be leaning on Centres in Croatia and abroad.

We do not have the legislation for orphan drugs. The Expensive drugs fund that has been running for several years now has resolved the issue of access to therapy for most of the expensive drug treatments but has also been overloaded with other requests from patients with other diseases; and has also been inadequate in covering non expensive therapies that can also help RD patients.

Since 1986 we have been screening newborns for Phenylketonuria and Hypothyroidism in mandatory minimum measures of neonatal health care. We should consider expanding the screening for other rare diseases, with an estimate of costs and benefits for this action.

### **Development and structure of a National Plan/Strategy & Governance of a National Plan**

Croatia has not yet adopted a National Plan for Rare Diseases. However, thanks to the tireless efforts of patients and patients associations for rare diseases the awareness on rare diseases, patients and their everyday life problems has been raised in Croatia. The Ministry of Health and Social Care established the National Commission for the rare diseases at the end of February 2010 in order to produce the National Plan for the Rare Diseases. The Committee includes three representatives of civil organisations for rare diseases.

### **Conclusion:**

Dialogue between patients, medical experts and representatives of civil society was excellent. Through the presentation of certain problems and opinions, we identified the following priorities:

1. Increase knowledge of the epidemiology of rare diseases
2. Ensure equal access to medicines for rare diseases
3. Train professionals and organise a network of doctors and centres that deal with rare diseases
4. Improve access to treatment and quality health care along with screening for rare diseases

## *Theme 2 - Definition, codification and inventorying of RD*

### Sub-Themes

1. Definition of RD
2. Classification and traceability of RDs in the national health system
3. Inventories, registries and lists

#### **2.1. Information and training**

4. How to improve information on available care for RDs in general for different audiences
5. How to improve access to quality information on RDs
6. How to ensure adequate training of healthcare professionals on RDs

### **Workshop 2**

**Chair: prof.dr.sc. Ingeborg Barisic**

**Rappoteur: Andreja Santek**

**Date, time, place: 18.09.2010., 14:00 - 15:30, hall Mare 2, Hotel Palace, Dubrovnik**

**Participants: 65**

#### **Definition of RD**

Croatia has adopted the definition of rare diseases set by the EU stating that rare diseases are those that affect less than 5 out of 10 000 inhabitants in one country, with the addition of rare tumours emphasising their severity. In oncology the definition must include the problem of severity and incurability in order to reflect the incidence of oncological problems.

#### **Classification and traceability of RDs in the national health system**

Croatia uses ICD10 classification system to detect and index different diseases. One of the main problems in planning health care measures for rare diseases is that the overall burden is invisible to the health system due to difficulties in diagnosis, misclassification and the lack of appropriate coding. To take advantage of the new classification system ICD11 (expected in 2014) an appropriate and specific training for the health care professionals in coding must be foreseeable.

#### **Inventories, registries and lists**

Currently there is neither an official RDs list in Croatia nor a National registry for rare diseases. However, there are a small number of specific rare diseases registries managed by specialised clinics and

clinicians. The Croatian registry of congenital anomaly, a part of EUROCAT, has been functioning in the Referral Centre for Surveillance of Birth Defects (at the Children's Hospital Zagreb) since 1983.

In this workshop Dr. Davorin Herceg presented the project for the Registry of rare tumours, which was developed by the Cancer patient association "Za novi dan". The aim of this project is to collect epidemiological data on patients with rare tumours in Croatia and also to exchange the collected data with other European networks and registries. Participants agreed that registries are useful for health service planning, for assessing drug effectiveness and for controlling the quality of relevant health services.

Participants also stressed the need to create a National registry for rare diseases, emphasising the necessity for long-term EU funding and government support, crucial to the sustainability of registries and databases.

### **Information and training**

Substantial progress in raising public awareness about rare diseases in Croatia has been achieved by medical professionals in collaboration with patients, their families and patients associations. In Croatia there is The Croatian Society for Rare Diseases dedicated to reaching the objective of helping patients and their families to improve their quality of life. The Croatian Society for Rare Diseases has a national website [www.rijetke-bolesti.hr](http://www.rijetke-bolesti.hr) which provides information for patients with rare diseases and their families.

In Croatia there is also the medical organisation Croatian Society for rare diseases, which brings together various medical professionals dealing with rare diseases. They also have a website [www.rijetke-bolesti.org](http://www.rijetke-bolesti.org) which offers information for medical professionals. In addition various rare disease associations have their own websites with specific information for their members and the general public.

Since 2008 in Croatia with the support EURORDIS and organisation of The Croatian Society for Rare Diseases as well as in the rest of the world, the last day of February is observed as Rare Disease Day. What is needed is stronger support of Ministry of Health and Social Care in implementing public health actions aimed at raising awareness about rare diseases.

Due to the lack of knowledge about the existence, prevalence and pathology of rare diseases lives of patients' lives are often put at risk with damaging repercussions - pointless delay, frequent medical consultation and inadequate or even harmful prescription of drugs and therapies. In fact the curriculum in university medical training for future medical professionals does not provide sufficient data on rare diseases. In order to improve the education of medical professionals, it is proposed to encourage the study of education of those branches where the rare diseases are most common- genetics, oncology, immunology, neurology and paediatrics. It has also been suggested that in the sixth year of medical school the University provides at least one subject on rare diseases.

### **Conclusion:**

Discussion of patients, medical experts and representatives of civil society was successful. Through the presentation of certain problems and opinions, we identified these priorities:

1. The applied definition of rare diseases, which is set by the EU, states that rare diseases affect less than 5 out of 10 000 inhabitants, with the addition of rare tumours.
2. Establish an orphan code that will match the international classification ICD11.
3. Create a National Registry for Rare Diseases, with the suggestion that there is an obligation to report to the registry, as in infectious diseases. Emphasising the importance of funding the registry – financial support should be permanent, with the involvement of government and the EU.
4. Dissemination of information through professional societies and patients associations. Promoting the activities of Patients organisations in order to raise awareness about rare diseases.
5. Improving formal education and training of medical professionals.

### **Theme 3 - Research on RD**

#### **Sub-Themes**

1. Mapping of existing research resources, infrastructures and programmes for RDs
2. Needs and priorities for research in the field of RDs
3. Fostering interest and participation of national laboratories and researchers, patients and patient organisations in RD research projects
4. Sustainability of research on RD
5. EU collaboration on research on RD

#### **Workshop 3**

**Chair: Prof.dr.sc. Ana Stavljenic-Rukavina**

**Rapporteur: Mrs. Andreja Santek**

**Date, time, place: 18.09.2010., od 15:45 do 17:15, hall Mare 1, Hotel Palace, Dubrovnik**

**Participants: 55**

#### **Mapping of existing research resources, infrastructures and programmes for RDs**

Participants agreed that various reasons make research on rare diseases difficult, namely: the high number and wide variety of the diseases, the lack of suitable experimental models for most rare diseases, the poorly defined endpoints, the small number of patients and, above all, limited resources. Medical professionals agreed that Croatia can promote research and innovation in treatment and diagnosis of rare diseases by establishing translational centres to connect the clinical and basic science with social sciences and political sciences. The desired result is both patient care and also preventive measures which may extend beyond the provision of healthcare services. In Croatia there is only one database of clinical studies – [www.regpok.hr](http://www.regpok.hr). RegPok.hr is the prototype registry of clinical trials in the Croatian language. It includes clinical research taking place in Croatia and recorded in the world's largest registry of clinical trials at ClinicalTrials.gov medical library of the National Institutes of Health of the

United States. At the moment there are 355 studies, however, only 4 are related to rare diseases. The base is made by the Medical Faculty in Split, with support of the Ministry of Education and Sports of the Croatian Republic.

### **Needs and priorities for research in the field of RDs**

In addition to known lack of interest by most pharmaceutical companies in developing treatments for rare diseases due to the limited market potential for each individual disease, it is highlighted that the difficulty of performing clinical trials with new treatments for rare diseases is one important and often limiting step in developing new therapies for rare diseases. International collaboration in the performance of clinical trials is essential to reach a population size which provides sufficient statistical power to the study, hence improving the potential to assess the treatment efficacy for rare diseases.

The workshop highlighted the lack of communication within the specialists and lack of coordination of research projects. It is stressed that we need to create a database of clinical trials, as well as databases on current projects. For the sustainability of the registries it is crucial to provide permanent funding, with the help of EU and the government. Also, it was suggested that the work of the National Commission for Rare Diseases at Ministry of Health and Social Care includes a representative of the Ministry of Science, Education and Sports in their sessions. It is necessary to identify existing projects in RDs field. It is important that the National Commission for Rare Diseases at the Ministry of Health and Social Care identifies the people involved in the research of rare diseases that are in FP7 projects.

### **Fostering interest and participation of national laboratories and researchers, patients and patient organisations in RD research projects**

There is a strong need for fostering collaborative programs on all fields of research on rare diseases, from fundamental/basic through to social research, at national, European and international level.

### **Conclusion:**

All participants agreed that there is currently almost no rare diseases research in Croatia. In order to foster that process they proposed:

1. Promoting scientific research projects in RDs,
2. Linking scientists and creating databases of projects that are currently under development and implemented,
3. Encouraging cooperation between research centres and connecting them with international institutions that can fund these projects,
4. Promotion of basic and clinical research, promotion of databases in which patients can participate.

## *Theme 4 - Standards of care for RDs - Centres of Expertise (CoE)/ European Reference Networks (ERN)*

### Sub-Themes

1. Identification of national or regional CoE all through the national territory by 2013
2. Sustainability of CoE
3. Participation in ERN
4. How to shorten the route to diagnosis
5. How to offer suitable care and organise adequate healthcare pathways for RD patients
6. How to ensure in CoE multidisciplinary approaches and integration between medical and social levels
7. How to evaluate CoE

#### **4.1. Orphan Drugs (OD)**

8. Future of OD
9. Access of RD patients to orphan drugs Pricing and Reimbursement
10. Compassionate use and temporary approval of orphan drugs. Off label use

### **Workshop 4**

**Chair: Mrs. Anja Kladar, Mrs. Gabriele Pohla Gubo, Prof.dr. Mirando Mrcic**

**Rapporteur: Mr. Mario Harapin**

**Date, time, place: 18.09.2010., od 14:00 do 15:30, hall Mare 1, Hotel Palace, Dubrovnik**

**Participants: 61**

#### **Identification of national or regional CoE all through the national territory by 2013**

Anja Kladar, through the example of the Croatian cystic fibrosis association, stressed the lack of skilled medical professionals in the field of rare diseases and the necessity of creating partnerships with relevant public institutions, professional associations and medical institutions. She said that care must be clearly defined. The workshop pointed out that the Republic of Croatia is a small country with only 4.5 million people and establishing Centres of expertise for certain diseases is expensive, and therefore it is necessary to establish a network of medical experts dealing with rare diseases and to promote cross border cooperation. Excellent example of cross border cooperation demonstrated guest Dr. Gabriele Pohl Gubo from Paracelsus Clinic in Salzburg, Austria, which organized DEBRA house. Thanks to the engagement of the Croatian Epidermolysis Bullosa Association the cooperation with the Austrian hospital has been established, which in long term improved diagnosis of patients with EB in Croatia.

#### **How to shorten the route to diagnosis**

Rare diseases are rarely diagnosed, and for some diseases patients wait up to 10 years before having the correct diagnosis. Establishing Centres of expertise for certain diseases is expensive and therefore it is necessary to establish a network of medical experts dealing with rare diseases and to promote cross border cooperation. Since 1986 Croatia provides screening of newborns for phenylketonuria and hypothyroidism in mandatory minimum measures of neonatal health care. Professor Ksenija Fumić stressed the need to expand newborn screening. However, the required technology is expensive. Medical professionals agreed about expanding the screening for other rare diseases with an estimate of costs and benefits.

### **Future of Orphan Drugs**

The availability of drugs enhanced since the establishment of the Fund for the expensive drugs at the Croatian Institute for Health Insurance, but the basic problem still remains – there is no legislation on orphan drugs. We do not have regulations enabling rapid orphan drugs access to patients. A regulation for ODs is currently being prepared by an initiative group and will be proposed to Ministry of Health and Social Care in due time.

### **Access of RD patients to orphan drugs Pricing and Reimbursement**

In many cases patients face difficulties in obtaining drugs, due to insufficient funding. There is a huge problem with unregistered orphan drugs. Croatia is a small country with a relatively small number of patients and pharmaceutical companies have no interest in registering an orphan drug. Supply of such medicines in Croatia is very difficult because the acquisition and reimbursement is transferred to the hospitals. From an administrative perspective acquisition of such medicines is often lengthy and complicated.

### **Compassionate use and temporary approval of orphan drugs. Off label use**

The prescription and the use of off-label medicaments is difficult and reimbursement is categorically impossible. It is still the problem of thinking about the treatment of rare diseases for evidence based medicine. This is a very strong criterion, but in rare diseases, there is no evidence based medicine.

### **Conclusion:**

The workshop discussion focused mainly on the Centres of expertise. The workshop pointed out that the Republic of Croatia is a small country with only 4.5 million people, and establishing Centres of expertise for certain diseases is expensive, and therefore it is necessary to establish a network of medical experts dealing with rare diseases and to promote cross border cooperation. The topic of orphan drugs was stimulating for the participants, especially the patients. All the participants expressed their dissatisfaction with the current situation: lack of an orphan drug law, insufficient orphan drugs, problems with off-label medicines, plenty of unclear criteria and rules.

In order to improve access to treatment and the quality of patient care participants proposed the following:

1. Create a legislation act for Ods,
2. Improve and simplify the procedure for acquisition of an unregistered orphan drug,
3. Establish a network of medical experts dealing with rare diseases,
4. Develop Centres of expertise and promote cross border medicine.

The importance of a transnational approach to health care pathways is vital in the field of rare diseases considering the frequent lack at national level.

### *Theme 5 - Patient Empowerment and Specialised Services*

#### Sub-Themes

1. Involvement of patients and their representatives in decision-making processes in the field of RDs
2. Support to the activities performed by patient organisations
3. Specialised social services: Respite Care Services; Therapeutic Recreational Programmes; Services aimed at the integration of patients in daily life
4. Help Lines

#### **Workshop 5**

**Chair: Mrs. Vlasta Zmazek**

**Rapporteur: Mr. Mario Harapin**

**Date, time, place: 18.09.2010., od 11:30 do 13:00, hall Mare 2, Hotel Palace, Dubrovnik**

**Participants: 80**

#### **Involvement of patients and their representatives in decision-making processes in the field of RDs**

Chair of the workshop Mrs. Vlasta Zmazek presented successful 15-year-old work of the Croatian Epidermolysis Bullosa Association. Through the exchange of experiences present participants concluded that civil associations play a key role in providing information to patients, raising funds for research, and lobbying for higher quality care and treatment. Many of these patients and their family members are organised into national organisations, some of which are associated with the European umbrella organisations.

#### **Support to the activities performed by patient organisations**

It is primordial to support the work of patient associations thus enabling them to better educate patients, medical professionals and social communities. It is necessary to facilitate the cooperation between patient organisations and the media in order to ensure increased social awareness about rare diseases. It was concluded that we should work on networking associations, among themselves and with professional teams and international patient's organisations.

#### **Specialised social services: Respite Care Services; Therapeutic Recreational Programmes; Services aimed at the integration of patients in daily life**

We should work on better cooperation and partnership between patients associations and institutions. Participants concluded that it is necessary to generate a multidisciplinary approach with doctors, psychologists, social workers and above all patient associations working together with the RD patients.

### **Help Lines**

Participants agreed that Help lines, as well as other interactive information and support services for patients should be included in the provisions of a National Plan for rare diseases. Specialised social services, such as those facilitating attendance at school and participation in the workforce, are also important for the empowerment of people living with rare diseases and improvement of their wellbeing and social inclusion. It is highly necessary to maintain support to the work of the patients associations, so that they can work on educating patients, medical professionals and social community.

### **Conclusion:**

The workshop was attended by representatives from 15 RDs Croatian patients associations and eight representatives of patient's organisations from Slovenia, Bosnia and Herzegovina and Serbia with the main objective of exchanging experiences and establishing cooperation. Discussion, that was very dynamic, concluded with the thought that the empowerment of patients must be run through patient organisations. It is essential to support the work of patient associations, so that they can provide important information to patients, medical professionals and the general public. It is necessary to ensure the cooperation of patient's organisations and the media in order to raise awareness of the society about rare diseases. It was concluded that we should work on networking organisations, among themselves and with professional teams and international organisations. We should work on better cooperation and partnership between organisations and institutions. We should bear in mind that successful work with RD patients must include cooperation of doctors, psychologists, social workers and most importantly the patient organisations.

## **Horizontal Themes**

### *Theme 6 – Patient rights in social welfare system*

#### **Workshop 6**

**Chair: Mrs. Vesna Skulic, Mrs. Anja Kladar**

**Rapporteur: Mr. Mario Harapin**

**Date, time, place: 18.09.2010., od 15:45 do 17:15, hall Mare 2, Hotel Palace, Dubrovnik**

**Participants: 53**

Chairs stressed that one of the basic preconditions for the equal inclusion of persons with disabilities is right to economic and social security and a decent life standard.

In accordance with the Law on Social Welfare a person or family in need shall be entitled to the following services: counselling, financial help in overcoming specific problems, maintenance assistance, assistance for housing costs, one-time financial assistance, the allowance for help and home care, personal disability, training for independent life and work, work outside the family, the status of their parents and other caregivers help and parent carer status.

In most cases, the request for the certain services shall be submitted to branch offices of the Centre for Social Welfare. However, in everyday life, people suffering from rare diseases and their family members when exercising their basic rights are faced with numerous limitations. It often happens that the government institutions that are directly responsible for protection of rights of persons with disabilities do not recognise real needs of RD patients and in cases when patients turn to them for help, they will withhold aid basing their actions on insufficient information about the severity of patients' situation.

In such cases the only criteria applied in decision making in granting access to social services provided is the fact that their illness does or does not meet or comply with the prescribed conditions for the exercise of certain rights. Since we're talking about a huge number of different diagnoses, which often manifest themselves individually in a different range of symptoms and difficulties, and many of these are represented by only few patients in Croatia, it is unlikely that they will be provided in a regulation for the exercise of social rights. A system that is institutionalised, centralised and inflexible, unable to follow the needs of people suffering from rare diseases as well as enable them to exercise their basic rights as Croatian citizens.

Furthermore, the social welfare system is still not sufficiently transparent, so when situations occur that people with disabilities and their families due to ignorance and lack of information do not get their rightfully claimed rights or they do but not in a timely manner, there is no one to recognise their frustration and an on growing sense that they are left alone.

Also, with few exceptions, local communities do not recognise the needs of people with disabilities in their area and therefore lack the appropriate programs to overcome the special limitations that arise from living with rare disease in an unadjusted environment. Stimulating social interaction and participation of persons with disabilities in society should be prioritised after establishing minimal social rights achievement according to specific needs of RD patients and their families.

### **Conclusion:**

After a very dynamic discussion, participants concluded:

1. Improve access to systematic, correct and manageable information on social rights and services provided,
2. Recognise lack of lawyers specialising in medical law,
3. Define the status of maternal caregivers, define the rights of carers of adults and educationally capable children,
4. Necessity of a social worker employed at the hospital,

- 
5. Rehabilitation of patients and returning to their community after longer periods of being in an institution and the importance of including trained social workers in work on educating the family and the community.

---

## Conclusion of the Final Report

When comparing today's achievements of patients and their families, patient organisations and individual health professionals with our future aspirations, we can only hope that our growth then will be handled with such tremendous will and imagination in using however small tools given to achieve our common goals. While having EUROPLAN Recommendations and EUROPLAN indicators as our tools, our requests become even larger in trying to fulfil the needs of the on growing RD community in Croatia and to ensure them equality and access for all. As many people and their conditions become recognised this opens up questions of treatment, rehabilitation, social services, education and social isolation.

The First National Conference on Rare Diseases seems as a pathway to restoring basic human rights recognition and protection for the RD patients. Giving their needs a legislative framework is the only way for giving RD patients backs their voices as equal citizens of our country.

Being very much aware of the importance of this Conference we have tried to gather all prominent stakeholders encouraging them to debate their opinions and to participate in the given workshops as much as they can. This has resulted in six vibrant, colourful and creative workshops whose conclusions give us a clear picture of what kind of change is really needed in Croatia today.

It is evident that the climate for development of a National plan was set prior to the Conference, with The Ministry of Health and Social Welfare establishing the National Commission for Rare Diseases in order to construct the legislative framework for Rare Diseases, only to be confirmed in the discussions held during the Conference. Huge interest of patients and their families, local patient organisations and the ones from abroad, medical professionals and representatives of the government institutions and their understanding of the given materials and interaction in the workshops has clearly validated the EUROPLAN's tremendous work in setting the theoretical ground for this Conference.

The Conference has gives us an opportunity to review what has already been done and what needs to be done, speaking in short and long term objectives. Some of the EUROPLAN recommendations have no real possibility of implementation in Croatia due to the relatively small number of inhabitants. These being the establishment of Centres of Expertise for certain rare diseases, but when confronted with such a situation, participants agreed on new creative ways of solving shared problems with solutions adjusted to our local situations (in this case recommending a cross border network of physicians dealing with specific rare diseases in the region). Most of them also requested the Croatian Society for rare Diseases to organise National Conferences for Rare Diseases every subsequent year to follow up on the development of the National Plan.

And to conclude, the greatest accomplishment for the organisers comes from reviewing the evaluation cards filled by participants of the Conference. They have given us, in their own words, a sense of how important this Conference was and all expected after affects that will hopefully follow the gathering.

### III. Summary

The First Croatian National Conference on Rare Diseases was held under high auspices of the Croatian President prof. Dr. Ivo Josipovic and in cooperation with the European Organisation for Rare Diseases - EURORDIS.

The Conference had 180 participants - patients, representatives of 15 Croatian patients organisations (Croatian Society for Rare Diseases, DEBRA – Croatian Epidermolysis Bullosa Association, Cancer Patient Association „Za novi dan“, Croatian Cystic Fibrosis Association, Association of Persons with Prader-Willi Syndrome Croatia, Croatian association of people with scleroderma, Association of parents of children with visual impairments and additional disabilities "OKO", Association suffering from collagenosis, Association to help those suffering from phenylketonuria, Association of persons with physical disabilities Pula, Association to help children and families faced with malignant diseases "Krijesnica", Croatian Hemophilia Society, Association of Parents for Children hardest physical disabilities and children with special needs "Anđeli", Association of patients with disabilities Križevci, Coalition of Associations in Healthcare), medical professionals dealing with rare diseases, the representatives of the pharmaceutical industry (Pfizer , Roche, Medis Adria, Genzyme, Solpharm, GM Pharma), representatives of public health institutions (Ministry of Health and Social Welfare, the Croatian Health Insurance Institute, the Agency for Quality and Accreditation, Agency for Medicines and Medical Devices, Office of the Ombudsman for Persons with Disabilities), representatives of the Education (Agency for Education, National Centre for the Evaluation of Education), who presented the main issues and priorities of patients with rare diseases in our country.

The Conference was honoured to have foreign experts as guest lecturers (Dr. Gabriele Pohl Gubo from the Austrian clinics - DEBRA house) and 8 representatives of patients organisations from Slovenia, Bosnia and Herzegovina and Serbia (Citizens Association for the fight against rare disease in children "Život", Association of patients with acromegaly and diseases of the pituitary gland in Bosnia and Herzegovina, the Association for cystic fibrosis of the Republic of Serbian, Muscular Dystrophy Association of Serbia, Society suffering from epidermolysis bullosa – DEBRA Bosnia and Herzegovina, Society suffering from epidermolysis bullosa – DEBRA Slovenia, Society of Gaucher disease patients in Slovenia, Society of Fabry disease patients in Slovenia, to exchange experiences and establish cross border cooperation.

The First Croatian National Conference on rare diseases was organized through six workshops, in which all participants were actively engaged in discussions. Based on the proposed conclusions of the workshops, the Conference recommended the urgent adoption of National Plan for Rare Diseases. For efficient operation we have recommended to assembly a *Partnership for rare diseases* - Partnership between Patient Organisations, Ministry of Health and Social Welfare and the Croatian Institute for Health Insurance in order to achieve better coordination in preparing the Plan.

Conference participants have agreed on the following general proposals and guidelines for actions at national level:

1. Improve access to health care and social services, including the protection of patients' social rights and their right to multidisciplinary approach to care,
2. Ensure the introduction of the category of rare diseases in the legal acts of health and social care,
3. To establish Registry of RD and ensure it's long term sustainability by providing ongoing funding,
4. Establish a network of Centres of Expertise or a network of physicians working with RD patients,
5. Empowerment and support for the Patient Associations,
6. International networking and cooperation in the field of rare diseases.

#### IV. Document history

Status (Draft/Reviewed/Final)	Draft
Version n°	30092010
Author(s)	Katarina Katavic, Nataša Bijelic
Reviewer	Prof.dr. Mirando Mrsic
File name	I. Croatian National Conference on Rare Diseases – Final Report