

# UKRAINE

## EUROPLAN NATIONAL CONFERENCE

### FINAL REPORT

27 March 2013, Kiev

# FOREWORD

**The EUROPLAN National conferences are aimed at fostering the development of a comprehensive National Plan or Strategy for Rare Diseases addressing the unmet needs of patients living with a rare disease in Europe.**

These national plans and strategies are intended to implement concrete national measures in key areas from research to codification of rare diseases, diagnosis, care and treatments as well as adapted social services for rare disease patients while integrating EU policies.

The EUROPLAN National conferences are jointly organised in each country by a National Alliance of rare disease patients' organisations and EURORDIS – the European Organisation for Rare Diseases. For this purpose, EURORDIS nominated 10 EURORDIS-EUROPLAN Advisors - all being from a National Alliance - specifically in charge of advising two to three National Alliances.

In Ukraine, in the absence of a National Alliance of rare disease patient organisations, **CSMA** has been selected by EURORDIS to organise the EUROPLAN National Conference. CSMA is a member of EURORDIS and meets all the criteria to organise a successful conference based on the common format and to bring together relevant patient organisations and other stakeholders in the field of rare diseases, including policy makers.

**EUROPLAN National conferences share the same philosophy, objectives, format and content guidelines.** They involve all stakeholders relevant for developing a plan/strategy for rare diseases. According to the national situation of each country and its most pressing needs, the content can be adjusted.

During the period 2008-2011, a first set of 15 EUROPLAN National Conferences were organised within the European project EUROPLAN. Following the success of these conferences, a second round of up to 24 EUROPLAN National Conferences is taking place in the broader context of the Joint Action of the European Committee of Experts on Rare Diseases (EUCERD) over the period March 2012 until August 2015.

The EUROPLAN National Conferences present the European rare disease policies as well as the EUCERD Recommendations adopted between 2010 and 2013. They are organised around common themes based on the Recommendation of the Council of the European Union on an action in the field of rare diseases:

1. Methodology and Governance of a National Plan;
2. Definition, codification and inventorying of RD; Information and Training;
3. Research on RD;
4. Care - Centres of Expertise / European Reference Networks/Cross Border Health Care;
5. Orphan Drugs;
6. Social Services for RD.

The themes “Patient Empowerment”, “Gathering expertise at the European level” and “Sustainability” are transversal along the conference.

**REPORT of THE FIRST NATIONAL CONFERENCE**  
**"Ukraine and EUROPLAN2: development of the state strategy for rare diseases till 2020"**  
**March, 27th, 2013**  
**Kiev, Ukraine**

**Part I: General information**

<b>Country</b>	<b>Ukraine</b>
<b>Date &amp; place of the National Conference</b>	<b>Conference Hall of Hotel "Ukraine", Kyiv, Instytutska str. 4</b>
<b>Website</b>	<a href="http://www.csma.org.ua">www.csma.org.ua</a> ( <a href="http://csma.org.ua/europlan.html">http://csma.org.ua/europlan.html</a> )
<b>Organisers</b>	<b>CSMA - Kharkiv Charitable Foundation «Children with spinal muscular atrophy»</b>
<b>Members of the Steering Committee</b>	- <b>Oleg Kvlivdze</b> - Georgian Foundation for Genetic and Rare Diseases (GeRaD), EURORDIS Advisor in the frame of the EUROPLAN2 project, Georgia.  - <b>Victor Serdyuk</b> - National charitable organization «Council of protection of the rights and safety of patients», hereafter All-Ukrainian Council For Patients' Rights and Safety, Ukraine  - <b>Vitaliy Matyushenko</b> - Kharkiv charitable foundation «Children with spinal muscular atrophy», Ukraine
<b>Names and list of Workshops</b>	<b>Format of the Conference – Plenary session with open discussion.</b>
<b>Workshop Chairs (and Rapporteurs, where applicable)</b>	<b>According the Programme</b>
<b>Annexes:</b>	<b>1. Programme</b> <b>2. Success Indicators</b> <b>3. Resolution</b> <b>4. List of Speakers and list of Participants</b>

### **Presidents of the Conference:**

**Raisa Bohatyriova** - Minister of Health of Ukraine

**Tatyana Bahteeva** - Chairman of Committee of the Verhovna Rada of Ukraine concerning public health services, the Deputy of Ukraine

**Olena Grechanina** - Head specialist of Ministry of Health of Ukraine «Genetics medical, genetics laboratory», Corresponding member National Academy of Medical Sciences of Ukraine, M.D, Professor

**Victor Serdyuk** - President of the All-Ukrainian charitable organisation «Council of protection of the rights and safety of patients», Ukraine

**Vitaliy Matyushenko** - President of the Kharkiv charitable foundation «Children with spinal muscular atrophy», Ukraine

### **Participants of the Conference by stakeholders' groups**

Deputies of Ukraine

Representatives of Ministry of Health of Ukraine

Representatives of the Ministry of social policy of Ukraine

Representatives of National academy of medical sciences of Ukraine

Representatives of pharmaceutical industry of Ukraine

Representatives of pharmaceutical industry of EU

Representatives of the organizations of patients with rare diseases and other public organizations

***Eight different rare diseases are represented by patient organization.***

### **Ads/Conference materials (on the electronic flash-key languages [UA] [EN] [RUS])**

- Themes the EUROPLAN (the full version)
- [UA] [EN] [RUS] Convention on the Rights of Persons With Disabilities.
- [UA]2012 The Order of Ministry of Health of Ukraine N574 (the adds) «About the statement of Conceptual principles of creation systems of rendering of the complex help to children suffering from rare diseases in Ukraine»
- [UA]2012 The Order of Ministry of Health of Ukraine from 7/30/2012 № 574 «About the statement of Conceptual principles of creation systems of rendering of the complex help to children suffering from rare diseases in Ukraine»
- [UA]Discussions of the project of Law of Ukraine «About changing to the Bases of the Legislation of Ukraine about Public health services concerning medical aid for persons with rare diseases» (from 4/24/2012 registration №10383) in Committee of Verhovna Rada concerning of European integration.
- [UA]Action Plan for Ukraine on 2011-2014, Recommendation of the Council of Europe.
- EUROPLAN European project for rare diseases national plans development.
- EUCRD Recommendation on rare diseases European reference networks (RD ERNS) from January, 31st 2013
- Council recommendation of 8 June 2009 on an Action in the field of rare diseases (2009/C 151/02)

- Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products.
- Communication from the Commission to the European Parliament, the Council, the European economic and social Committee and the Committee of the regions on rare diseases: Europe's challenges from November, 11th, 2008 COM (2008) №679
- Decision No 1295/1999/EC of the European Parliament and of the Council of 29 April 1999 adopting a programme of Community action on rare diseases within the framework for action in the field of public health (1999 to 2003)
- EURORDIS-NORD-CORD Joint Declaration of 10 Key Principles for Rare Disease Patient Registries 2012
- How health systems can address health inequities through improved use of Structural Funds. Recommendations WHO 2012.
- Rare Diseases: Addressing the Need for Specialised Social Services and Integration into Social Policies. Recommendations EURORDIS 2012
- [RUS] The Directory of WEB RESOURCES.
- Booklet of EUROPLAN.
- [RUS] For help to speakers, the extended questions of Conference.
- [RUS] Official EURORDIS video of Rare Disease Day

## Part II: Main Report

### Plenary session with open discussion

About 70 patients, medical professionals, representatives of government and industry participated in the Ukrainian EUROPLAN National Conference for Rare Diseases, part of the EUROPLAN2 project, funded by the European Commission and EURORDIS. The event was co-organized by the patient's organizations of people with rare diseases. The Conference aimed to provide information regarding the various steps in developing a strategic plan, to identify best practices and to exchange models and data on effective strategies for rare diseases. In this context, the event aimed to make the Ukrainian stakeholders familiar with the EU Council Recommendation on rare diseases and EUROPLAN recommendations for actions, to present and gather proposal on them, to discuss their transferability and adaptation in Ukraine; determine on expert level the priority draft proposals for actions to improve prevention, diagnosis, treatment and social integration of patients. Despite a limited time of the Conference, participants had the opportunity to learn best European practices and recommendations in the field of rare diseases, the priorities to build the future National Plan for rare diseases, to discuss these issues with policy makers, to express their proposals in order to implement in an optimal way the policies and strategies for rare diseases.

Approximately since 2010 year efforts of Patients Organizations have led to evolve a problem of rare diseases by State. Separation of the medical professionals and legislative approaches have led to formalisation of requirements of patients with rare diseases by profile ministry (the Ministry of health) that just in 2012 has let out the order "About the

statement of Conceptual principles of creation systems of rendering of the complex help to children suffering from rare diseases in Ukraine”.

This document became accessible in a legal field thanks to efforts of the Kharkiv Genetical Centre and Foundation Children with CMA (the original publication in magazine CLINICAL GENETICS OF MONOGENIC DISEASES (КЛІНІЧНА ГЕНЕТИКА Й ПРЕНАТАЛЬНА ДІАГНОСТИКА № 1(1) 2012) <http://repo.knmu.edu.ua/handle/123456789/2147>)

This basic document has put a basis to systems approach formation to a question of rare diseases in Ukraine. For the first time “Rare Disease” according to EU principles have been officially defined. However, an absence of the similar formulation within the limits of a state policy interferes with practical development and introduction of this document and the principles mentioned in the Conception.

## The main themes and sub-themes.

### Conference opening

Deputies of Verhovna Rada of Ukraine (Ukraine’s Parliament) opened the conference and welcomed participants. The former Minister of Ministry of Health and now the Head of Health Committee of Verhovna Rada of Ukraine– Mrs. Bahteeva, sent a greeting message to the participants, expressing once more her support for the rare diseases cause.

### Country status. Ukraine:

- Has a definition of rare disease as a disease affecting no more than 5 per 10 000 persons (1-2000) in adopted Official Conception of RDs. An important document in field of RDs in Ukraine was issued by the Ministry of Health, «About the statement of Conceptual principles of creation systems of rendering of the complex help to children suffering from rare diseases in Ukraine»;
- Has a definition of “rare disease” as EU recommended;
- Has a definition of “orphan drug”
- But there are no other adopted legislative acts regarding RDs;
- There is no united state program for RDs.

European policy and guidelines (EU document presentations and discussion, presentation of EUROPLAN, EUCERD and EUCERD Joint Action) were presented. Ukrainian Legislative acts were presented. One Project of the Law for RDs («About changing to the Bases of the Legislation of Ukraine about Public health services concerning medical aid for persons with rare diseases») wasn’t viewed despite being registered in Verhovna Rada last year. The maintenance of the first version of the orphan disease Act was extensive and mentioned many problem aspects simultaneously. A lot of attention was given details; elements which it was very easy to subject to criticism at discussion were registered. The Committee of European integration of Verhovna Rada recognized it such as not to contradict to the rules of EU. However, in such edition the bill has been doomed to a failure, as has descended in the middle of 2012. Therefore by working out of the second alternative design of the orphan disease Act accents are placed only on priority questions without which it is impossible to introduce the subsequent legislation about RDs.

Another one Project of Law for RDs (or orphan disease Act) was registered in Verhovna Rada on 5 of March 2013 and is waiting for hearing. Basic principles that were put in a design basis:

- the concept of a rare disease, in agreement with the recommendations of EUCERD and in the same edition,
- recognized the need to determine the priority list of the RDs for the formation of aid to patients with RD,

and as a consequence - a requirement of establishing a state registry of RDs.

According to the procedure of adoption of legislation in Ukraine of the Act provided for a January 1, 2014.

Practical proposal from POs – help legislators to determine and improve reflecting of patients’ needs. In order of the bill revising, a decision of Central Administrative Board of legal support of Verhovna Rada was received. At hearings in Committee of the Verhovna Rada of Ukraine concerning public health services and Committee on affairs of pensioners and invalids which are responsible for the bill preparation, POs’ opinions are considered and it is accepted to recommend the Verhovna Rada to vote for acceptance of this orphan disease Act.

## **Theme 1 - Methodology, Governance and Monitoring of the National Plan**

### **Sub-Themes:**

**1.1 Mapping policies and resources.** The fragmentations of State subordinate acts were discussed as well as access and informing of patients for it. There is not an integrative framework of the RDs.

**1.2 Development of a National Plan /Strategy** – needs to be systemized before, there is no coordination mechanism. Currently, no national plan or strategy for RDs exists in Ukraine. Unfortunately, the National Health Program still does not mention RDs in any way and RDs need to be added on it. The reasons of a fragmentation – absence of visibility of RDs at governance level. Instruments to solve it – attracting voices of POs to build the scheme, so the coming proposals can be summarized into one – to hear patients’ voices.

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

### **Sub-Themes:**

**2.1 Definition of RD** is adopted in Ukraine by law like in EU. There are no other definitions but dissemination of knowledge among doctors as well as inside social structures is insufficient and is not used in practice. The most common formulations of diagnosis are by name of disease without sub classification as Rare. The Rare Disease term is not used in standard medical documentation.

**2.2 There are RDs codifications on ICD10**, OMIM is also sometimes used, but traceability in national health system is absent. The ICD-11 system is recognized to use when ready. It was reflected in the Conception for Rare Diseases, mentioned above and in ads.

**2.3 Registries and databases** are fragmented and supported only by POs. Both, clinicians and patients are showing huge interest in establishing registries for their diseases. Some of the patients' organizations in Ukraine who is strong and recognizes value of the "registry" as basis for rendering assistance, have their own registries created according to their own requirements - spinal muscular atrophy, muscular dystrophy, Mucopolysaccharidoses, cystic fibrosis, Gaucher disease, haemophilia. Some specialized genetic centers have own databases of wider spectrum of rare diseases. But, both those and other databases are fragmented by data about patients, since close cooperation in public health services sphere is absent. By an essential lack, both for patients' databases and official bodies, is recognized an absence of complete pattern about all diagnosed patients. Patients' organizations databases contain the data for constant monitoring of patients, whereas in the state records personal contacts are absent, though a wide medical characteristic of the patients is contained. There are existing numerous specific rare diseases registries, ran by the specialized clinics. But they are not standardized and cover only the areas of interest of the particular institution which is managing them. The orphan disease Act will change many things. Today the work has just begun over new list of diseases related to rare. There is no official RDs registry either. Its set-up should start after the adoption of the official RDs list. So far, no algorithms and standards of care for people with rare diseases exist. There is no national registry of rare patients. What does all this mean? The state does not see and does not know how much money that medicines and diagnostic tools necessary to purchase, which prepare professionals to develop educational health programs, and more. Thus, discussion recognizes necessity to fix in the legislation of the state registry which will be collectively sustained patients' organizations as well official bodies.

**2.7 Training healthcare professionals** is going on but must be advanced in aspect of RDs. It was highlighted that experts of the Kharkiv medical genetic centre and the Kharkiv medical University pay attention for special trainings which are accented on rare diseases.

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

#### **Sub-Themes:**

**3.5 Fostering interest and participation** of national laboratories and researchers, patients and patients' organizations in RD research projects currently depends on personal area of interest of the researchers. Involvement of patients and patient organizations is an initiative of patient organization. A realization is on stage of advertising of benefits for Institution as well as crucial for life of patients with RDs. Among researchers and doctors there are initiative persons who wish to make a breakthrough in science and to be at the cut edge of science. In a case when patients' organization meets such a person and, providing the advanced information in the area, there is the mutual cooperation. In that case the official body can assist initiative work, without putting any finance. Mutual benefit is obvious - rare patients receive the qualified and system medical support, and the establishment reduces a recent trend without budgetary (or with the minimum



investment) means.

No dedicated research programs for RDs, they are partly financed within general application process for state-funded research. No recruitment of scientists specifically for RDs.

**3.7 EU and international collaboration** on research on RD is fragmented and leads on separated basis. There is no centralized information about such RDs programs.

The overall assessment of this theme consisted of direct dependence - absence of any database for rare diseases and clear vision of direction to develop a new scientific ways as well financing specific program at National level.

## **Theme 4 – Care for RDs - Centers of Expertise and European Reference Networks for Rare Diseases**

### **Sub-Themes:**

**4.1 Designation and evaluation of CoE** on stage of defining. An existing system of Health care has specialized medical genetic centers since 2004 year. There is no specific designation for CoE. In context of suggested changes in Health care laws Kharkiv genetic center and Kyiv genetic center are pretending on status of CoE. A formalization of this process is not complete yet, but principles to provide a complex of aid is put. In existing structure of medical care a universal center for diagnosing, care and rehabilitation is not possible. For rare diseases problem of fast and right diagnostics is a priority task. Generally the same centers will solve it and direct a patient to proper medical institution. A list of centers can be found on the official web-site of MoH [http://www.moz.gov.ua/ua/portal/dn\\_20031231\\_641\\_.html](http://www.moz.gov.ua/ua/portal/dn_20031231_641_.html)

Patients' organizations representatives pointed on necessity of developing of collaboration for such centers with European networks for more efficient results. However was marked, till formalizing of accepting a center as "CoE" a developing of united network could not be possible.

**4.7 Diagnostic and genetic testing.** RDs are diagnosed rather well in regional cities and for not super-rare diseases. Genetic testing is provided for involved sibs and relatives. Storing of biological examples is available in genetic institutions in Kyiv and Lviv. Mostly, it used to provide a testing of sibs. However, an exchanging of samples is difficult within Ukraine as well as to abroad. An international exchanging is absent due to separated legal system, in fact the State is separated from novel scientific practices.

**4.8 Screening policies.** There are three National programs just for monogenic diseases, for instance – phenylketonuria, cystic fibrosis, inborn hipothereosys. Testing is provided around Ukraine and is a long-term government program with full financing. The first level of diagnostics is carried out at a birth; the subsequent conclusions, consultations and support are pitched in the regional centers of medical-genetic service. Genetic testing for other diseases is possible within the limits of the prevention of hereditary diseases, it is selective during genetic consultation of young pairs before registration of the act of civil

status (marriage).

## **Theme 5 – Orphan Medicinal Products**

### **Sub-Themes:**

5.2. **Access to treatments** is limited by state accessing procedure. Some of the drugs, including ODs are reimbursed through the list of chronic disorders and are available through dedicated yearly therapeutic programs of national Budget. The second topic (ODs) was also so attractive for the participants and especially the patients. All the present rare diseases stakeholders expressed their dissatisfaction with the current ODs legal mechanisms which are very slow, with plenty of unclear criteria and rules. Additional proposals included a separate regulation for ODs, different from these for the traditional drugs and lower VAT on ODs.

5.4. **Off label use** of medicinal products is non transparent.

The main "stumbling block" - this is the cost of treatment of orphan patients. When it comes to how much is treatment and as economically difficult one to answer this question. Unfortunately, we have many disabled because their condition has not been diagnosed or treated improperly. What better and more humane for the State - to ensure timely diagnosis and treatment, and to be productive members of society, or pass them to disabled? So patients' organizations solve very difficult task - to change the attitude of the State and society for people with rare diseases reminded that they have a right to a normal life and not be rejected by society. The orphan disease Act should encourage pharmaceutical companies to register new drugs. Today orphan market in Ukraine is not developed. To understand how many and what medicines you need to know the number of patients who develop treatment protocols. And while no patient registries, protocols, hence there are no patients allegedly because pharmaceutical companies to come here. Domestic manufacturers also make no sense orphan drugs, because the commercial success of such drugs under the current legislation is not possible. Overseas development of treatments for rare diseases stimulated state. It is necessary to change the legislation, including the tender of purchase. The current system of tender procedures not only promotes but also hinders progress in this area. The most important thing for the public tender is the price. For example, in a tender three distributors are participating. Choose the one who offers the lowest price. One of the companies won the tender from year to year. The rest is interesting exhibit tendered his medication. Fortifying the market and taking advantage of monopoly, the company has a monopoly periodically increases the price of public money again lacking. We were driven to the cheapest products, but their older formula. But somehow the government buys them at exorbitant prices. And dictates its own rules - either drugs or anything. Tendering system of bureaucratic red tape strongly inhibits competition. Everyone knows that wins the cheapest drug that is corruption schemes, and do not try to register and deliver advanced and more expensive form. If the company sees that she cannot get to market advantage, it does not come to this market. Patients' organizations may try to influence the market, but it is very long and complicated process. Check the new product in the country depends on many factors, and

one of our few requests. Of course, we require buying a certain product, but if it is more expensive, the officer says that he does not want to break the law. So in the end creates a paradox - the official decides how to treat the patient.

## **Theme 6 –Social Services for Rare Diseases**

### **Sub-Themes**

#### **6.1. Social resources for people with disabilities.**

There are no specialized support programs for people with RDs. There are programs for patients with disabilities and their families, but they are too general, out-of-date and not available for all persons who need them. Most of the existing programs are managed and coordinated by the municipal authorities, so there is also a difference of the services available on a national level – the majority of the programs being almost in the biggest cities and a lack of specialized services in the regions. The access to these programs passes by a certificate of disability, issued by a territorial expert commission, composed by doctors. These commissions define and evaluate the degree of disability. Most of the proposals here were directed to the territorial expert commission activities. RDs patients complained about the bureaucracy, incompetence and lack of clear criteria when getting this certificate. Most of territorial expert commission commissioners are unaware of the RDs, RDs patients are made to prove evident facts. There are also no clear rules how the RDs patients have to be certified and even paradoxes of RDs patients in a severe condition not being evaluated by territorial expert commission. Patients insisted on involvement in the process of control of territorial expert commission, but it seems absolutely impossible in frame of legislative. By now, there are no steps taken in direction to improve availability and accessibility of such services, including public funding.

The existing programs include personal caregivers support, specialized transport and nutrition services, physical rehabilitation, psychological aid, social integration and professional training and pre-qualification programs. However, only limited persons have access to them. Most of the services are not fully appropriate for RDs patients, because they are intended for more general health disorders. But the access of RDs patients to them is really hard because of the existing legal framework which does not included RDs specifications. Most of the patients agreed that the methodology of territorial expert commission must be revised and updated, including clear definitions for RD condition and precise criteria how the patients must be classified. Additionally, since one part of the social programs is organized on a municipal level, the local authorities have to be introduced in RDs problems and be aware of the existence of such people.

Existing specialized social services are financed by government institutions and budget. There are some private initiatives, but they are locally concentrated and focused on a very specific target group not often especially for RDs, for instance – epilepsy or cerebral palsy. All of the RDs patient organizations are not financially supported through government programs.

POs representatives are voted for creating a “help line” to assist RD patients and healthcare professionals through web-site as well “hot call”. Especially patients’ organizations provide advocacy support and information about the rights of the patients.

The participants defined obstacles to establish united “help-line” as lack of funding, and the lack of adequate information. The most popular recourse in EU like ORPHANET, well known among the RDs stakeholders in EU, couldn’t be developed in Ukraine due to absence of the State support. In addition, the regular patients do not always know foreign languages as well internet resources, so they are not able to extract the information they need. A proposal to organize a national directory for RDs information has evolved. Representatives of RDs patients’ organizations voted for uniting of efforts for all RDs strategies and intense and efficient public awareness campaign to appropriate possibilities for governmental funding of the RDs associations’ activities.

### **Closing Session – Conclusions**

The aim of the First EUROPLAN National Conference is comprehensive, so participants couldn’t give concrete solutions at each point separately. At least a proposal to organize more precise workshops that can be eventually used for national work plan on RDs was suggested.

The First EUROPLAN National Conference can be a pathway to restoring basic rights 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. Assessment of the usefulness of the European guidelines and policy recommendations were accepted. Identification of path through specific committee to define challenges and needs across all themes was voted.

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

- full support of the priorities set out in the EU Council Recommendation on rare diseases actions, adopted on June 9, 2009;
- need for urgent legislative initiatives to protect the rights of people with rare diseases and ensure adequate prevention, treatment, rehabilitation and social cares;
- encouraging the establishment of national registries for rare diseases
- implementation of an integrated approach to people with rare diseases and their families.

According the EUROPLAN Recommendations «in last minute» to discuss hot themes on a short high level session with MoH representatives the Chairman of Committee of invalids and veterans Valery Sushkevich, the Deputy, has in writing assured about full assistance to initiatives of patients organizations in preparing and lobbying.

### **Closing remarks**

Of course, while Rare Diseases are still outside of state policy, any among the Themes of EUROPLAN is dealt with inadequate government coverage and a deeper study of each theme is needed. This review is essential as points of view of participants and shows a deep and wide range of questions discussed on the Conference.

## Annexes

### Annexe 1: Programme

#### Plenary session I

**Theme I: Creation of the National plan, management and control. Legislative definition of rare diseases.**

**Chairmen Sushkevich V. (Ukraine), Kvlividse O. (Georgia/France)**

EUROPLAN Project - the International experience on creation of National plans for rare diseases.

Stefanov R.,

*Department of Public health services of Plovdiv Medical University, a member of Interdisciplinary Committee of EU "IRDiRC", Bulgaria*

Legislative initiatives in Ukraine

Sushkevich V.,

*Chairman of Committee of the Verhovna Rada of Ukraine on affairs of pensioners, veterans and invalids, the Deputy of Ukraine*

**Theme II: Orphan drugs and therapy for rare diseases**

**Chairmen Serdyuk V., Sergiyenko S. (Ukraine)**

Access to not registered medical products, use an off-label of medicinal products

Rukavishnikov I.,

*General manager of Genzyme, Russia and CIS (Russia)*

Access to non-registered medical products for Eastern Europe.

Wikki K., Hughes-Wilson W.,

*General manager of SOBI, Russia and CIS (Sweden)*

National regulation of access to the market orphanных preparations in Ukraine

Hobzey M.

*Head of Department of reforms and development of medical aid of Ministry of Health of Ukraine*

National regulation of access to the market of orphan drugs in Ukraine

Konoshevich L.,

Chief of the department of medical products and medical production of Ministry of Health of Ukraine

The State help in providing of access to treatment of patients with MPS.

Kulesha T.

*Head of foundation of patients with MPS*

Gaucher Disease in Ukraine: treatment, problems.

Martynenko E.

*Head of foundation of patients with Gauche disease*

The interdisciplinary approach to management of EB

Derevjanko L.

*President of the International public organization «Dermatologists - for children»*

## Plenary session II

### Theme III: Codification of rare diseases

**Chairmen Grechanina O., Kulesha T. (Ukraine)**

Codification of rare diseases and traceability in national system of public health services. Registries and databases. Training and improvement of professional skill of workers of public health services in a question of rare diseases.

Grechanina O.

*Head specialist of Ministry of Health of Ukraine «Genetics medical, genetics laboratory»,  
Corresponding member National Academy of Medical Sciences of Ukraine, M.D, Professor*

Rare diseases: international and national experience of providing of access to treatment.

Serdyuk V.

*President of the All-Ukrainian charitable organization «Council of protection of the rights  
and safety of patients»*

### Theme IV: Scientific research in the field of rare diseases

**Chairmen Corresponding members of AMS of Ukraine Grechanina O., Gorovenko N., Arbuzova S.**

Scientific researches, infrastructure and programs of research for rare diseases

EU and international collaboration in scientific research in the field of rare diseases (IRDiRC)

Stefanov R.

*Department of Public health services of Plovdiv Medical University, a member of  
Interdisciplinary Committee of EU "IRDiRC", Bulgaria*

## Plenary session III

### Theme V: Social protection of patients with rare diseases

**Chairmen Kulesha T., Serdyuk V. (Ukraine)**

***Patients' message concerning access to treatment and participation in treatment***

Providing of access to effective and safe treatment of patients with rare diseases.

Nayshtetik E.

*Vice-president of the All-Ukrainian charitable organization «Council of protection of the  
rights and safety of patients»*

Maintenance of the rights of patients with rare diseases regarding performance of the international obligations of Ukraine. Aspects of implementation of the European Social Charter.

Scorina O.

*Head of legal service of the All-Ukrainian charitable organization «Council of protection of the  
rights and safety of patients»*

### **Panel discussion**

**Grechanina O., Stefanov R., Serdyuk V., Nayshtetik E.**

Centers of Expertise and European Reference networks for rare. Access to information for rare diseases. Diagnostics and genetic testing of rare diseases. Screening of rare diseases. Social resources for patients with rare diseases and specialized social protection for patients with rare diseases.

**Conclusions** from participants' hearing, acceptance of the Resolution of Conference

## **Annex 2: Success Indicators**

### **Quantitative indicators:**

1. Number of representatives of the Verhovna Rada of Ukraine, the central executive authorities, National academy of medical sciences of Ukraine participating in Conference (at least, 20 - done 12);
2. Number of experts and the scientists working in the field of rare diseases (at least, 10 - done 5);
3. Number of representatives of the organizations of patients with rare diseases and other public organizations (at least, 15 - done 25, the 8 different RDs);
4. Number of representatives of pharmaceutical industry (at least, 5 - done 3).

### **Qualitative indicators:**

#### ***The Short-term***

1. Acceptance of the Resolution reflecting necessity to begin work on the National plan/Strategy in the field of rare diseases.

#### ***The Long-term***

1. Put into the Verhovna Rada of Ukraine the Law of Ukraine for rare diseases;
2. Creation of Coordination council to interact with program EUROPLAN and to performance purposes in Ukraine.



### Annex 3: Resolution

#### **Resolution of the First National Conference "Ukraine and EUROPLAN-2: development of national strategy for rare diseases till 2020"**

March 27, 2013 - Kyiv, Ukraine

The **Coordinating Committee**, representing the project EUROPLAN-2, the European Organization for Rare Diseases EURORDIS, the All-Ukrainian Council of patients' rights and safety, Kharkiv Charitable Foundation "Children with spinal muscular atrophy" and participants:

After hearing the reports of international experts, representatives of patient organizations, public authorities, the Academy of Medical Sciences of Ukraine and the pharmaceutical industry;

**In consideration of:**

- Legislation of Ukraine;
- Ukraine's international legal obligations;
- Proposals of the Conference's participants;

**Taking into account** Council recommendations on an action in the field of rare diseases and the Communication from the Commission to the European Parliament on rare diseases;

**Having** EUROPLAN recommendations for the development of National Plans and Strategies for rare diseases and implementation it for a development of the national program for rare diseases;

**Recognizing:**

- Ukraine commitments to the Council of Europe before the end of 2013 to develop and approve the National Plan of Action on Patients' Safety;
- The actual stage of developing of the National Plan of Action on Patients' Safety in Ukraine;
- The necessity to expand the National Plan of Action on Patients' Safety of part reflecting measures that address the needs of patients with rare diseases and the need to create a state program for rare diseases;

**Decided to:**

1. Create at the All-Ukrainian Council of patients' rights and safety expert group with representatives of all stakeholders: public authorities, academia, international organizations and patient organizations, businesses, and independent experts for the following tasks:
  - 1.1. Conduct a comprehensive analysis of the situation with the availability of treatment and the needs of patients with rare diseases at a maximum level of knowledge.
  - 1.2. Amend the National Action Plan on patient safety (paragraph 1.4.1., CoE Action Plan for Ukraine for 2011-2014 "Partnership for Reform") action plan for rare diseases which has become the basis for the creation of relevant national policies.
2. Present developments of the expert group at the First National Congress of the rare disease in late 2013.
3. The Resolution to direct all interested parties and make efforts to ensure their participation in the implementation of the Resolution.

## Annexe 4

## List of Speakers of the Conference

Dudka	Verhovna Rada	Deputy	Politician
Sushkevich	Verhovna Rada	Deputy	Politician
Bahteeva	Verhovna Rada	Deputy	Politician
Wikki	SOBI	General Manager	Industry
Rukavishnikov	Genzyme	General Manager	Industry
Grechanina	Institution of Clinical Genetic	Director	Academic/Researcher
Stefanov	Center of Rare Diseases	IRDIRC	Other
Kvlividze	GeRaD	Adviser EURORDIS	Other
Hobzey	Ministry of Health	Chief of Department	Public administration, national
Konoshevich	Ministry of Health	Chief of Department	Public administration, national
Matyushenko	Foundation CSMA	President	Patient representative
Scorina	Foundation of Patient's rights	Chief of Department	Patient representative
Nayshtetik	Foundation of Patient's rights	Vice-president	Patient representative
Sergienko	Foundation of Patient's rights	Chief of Department	Patient representative
Zamorskaya	Foundation BE	Vice-president	Patient representative
Derevyanko	Foundation BE	President	Patient representative

## List of Participants to the Conference

Dudka	Verhovna Rada	Deputy	Politician
Sushkevich	Verhovna Rada	Deputy	Politician
Bahteeva	Verhovna Rada	Deputy	Politician
Wikki	SOBI	General Manager	Industry
Rukavishnikov	Genzyme	General Manager	Industry
Ostapchuk	ROCHE	Manager	Industry
Grechanina	Institution of Clinical Genetic	Director	Academic/Researcher
Gorovenko	National Academy of Medical Sciences (AMS)	Corresponding member of AMS	Academic/Researcher
Arbuzova	National Academy of Medical Sciences (AMS)	Corresponding member of AMS	Academic/Researcher
Bilovol	Institution of Therapy, Verhovna Rada	Director, Academic of AMS	Academic/Researcher

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Antipkin	Institution of Pediatric	Director, Academic of AMS	Academic/Researcher
Livshits	Institution of molecular biology and genetic	Director of department, Academic of AMS	Academic/Researcher
Stefanov	Center of Rare Diseases	IRDIRC	Other
Kvlividze	GeRaD	Adviser EURORDIS	Other
Pichkur	"OHMATDET"	Doctor	Clinician/GP
Gladush	"OHMATDET"	Director	Clinician/GP
Olhovich	"OHMATDET"	Doctor	Clinician/GP
Bohatyрева	Ministry of Health	Minister	Public administration, national
Hobzey	Ministry of Health	Chief of Department	Public administration, national
Konoshevich	Ministry of Health	Chief of Department	Public administration, national
Bocyura	Foundation CF	Head	Patient representative
Matyushenko	Foundation CSMA	Vice-president	Patient representative
Bachisheva	Foundation DMD	Head	Patient representative
Kulesha	Foundation MPS	President	Patient representative
Martynenko	Foundation Gaucher	Head	Patient representative
Matyushenko	Foundation CSMA	President	Patient representative
Tatarinov	Foundation CSMA	member	Patient representative
Serdyuk	Foundation of Patient's rights	President	Patient representative
Scorina	Foundation of Patient's rights	Chief of Department	Patient representative
Nayshtetik	Foundation of Patient's rights	Vice-president	Patient representative
Sergienko	Foundation of Patient's rights	Chief of Department	Patient representative
Shevchenko	Foundation Autists	Head	Patient representative
Zamorskaya	Foundation BE	Vice-president	Patient representative
Derevyanko	Foundation BE	President	Patient representative
Andriychuk	Foundation	President	Patient representative
Ocheretenko	Foundation of Patient's rights	Head	Patient representative

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Krulko	Foundation of Patient's rights	member	Patient representative
Leman	Foundation of Cancer	Head	Patient representative
Pashalna	Foundation PKU	Head	Patient representative
Kalinichenko	Foundation LH	Patient	Patient representative
Krupiy	Foundation LH	Patient	Patient representative
Kozhan	Foundation of Patient's rights	Chief of Department	Patient representative
Menshutkina	Foundation of Patient's rights	Chief of Department	Patient representative
Safurak	Foundation of Patient's rights	member	Patient representative
Nikityuk	Foundation of Patient's rights	member	Patient representative