ОРФАННІ ЗАХВОРЮВАННЯ УКРАЇНИ
ГРОМАДСЬКА СПІЛКА

EUROPLAN
European Project for Rare Diseases National Plans Development

# *Ukraine*

# EUROPLAN NATIONAL CONFERENCE

*in the framework of the EU Joint Action RD-ACTION*

*Kyiv, 20 October 2017*

## FINAL REPORT

RD ACTION
DATA AND POLICIES FOR RARE DISEASES

Co-funded by
the Health Programme
of the European Union

EURORDIS
RARE DISEASES EUROPE

# FOREWORD

The EUROPLAN national conferences or workshops are organised in many European countries as part of a coordinated and joint European effort to foster the development of comprehensive National Plans or Strategies 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/ workshops are jointly organised in each country by a National Alliance of rare disease patients' organisations and EURORDIS–Rare Diseases Europe. **Rare Disease National Alliances and Patient Organisations have a crucial role to shape the national policies for rare diseases.**

The strength of EUROPLAN national conference/ workshop lies in its shared philosophy and format:

- **Patient-led**: National Alliances are in the best position to address patients' needs;
- **Multi-stakeholders**: National Alliances ensure to invite all stakeholders involved for a broad debate;
- **Integrating both the national and European approach to rare disease policy;**
- **Being part of an overarching European action** (project or Joint Action) that provides the legitimacy and the framework for the organisation of EUROPLAN national conferences/workshops;
- **Helping national authorities adhere to the obligations stemming from the Council Recommendation of 8 June 2009 on an action in the field of rare diseases.**

Since 2008, National Alliances and EURORDIS have been involved in promoting the adoption and implementation of National Plans and Strategies for rare diseases. Altogether, 41 EUROPLAN national conferences took place in the framework of the first EUROPLAN project (2008-2011) and the EU Joint Action of the European Union Committee of Experts on Rare Diseases - EUCERD - (2012-2015).

Within RD-ACTION (2015-2018), the second EU Joint Action for rare diseases, National Alliances and EURORDIS continue to get involved in a coordinated European effort to advocate for and promote integrated national policy measures that have an impact on the lives of people living with rare diseases.

The EUROPLAN national conferences or workshops taking place within RD-ACTION focus on specific themes identified by the National Alliances as the most pressing priorities to tackle with national authorities. These thematic priorities are addressed in sessions where all the stakeholders discuss relevant measures to be taken or ways to sustain the full implementation of already approved measures.

Each National Alliance prepares a final report on the national workshop, based on a common format such as the one that follows.

## GENERAL INFORMATION

| | |
|---|---|
| **Country** | UKRAINE |
| **National Alliance (Organiser)** | NGO RARE DISEASES OF UKRAINE |
| **Date & place of the national workshop/conference** | 20/10/2017 KYIV, Instytutska street, 21/8, Office of Ukrainian Parliament Commissioner for Human Rights |
| **Website** | www.facebook.com/orphandisua/ |
| **Members of the Steering Committee** | Tetyana Kulesha<br>Domenica Taruscio<br>Valeria Lutkovska<br>Oleksandr Linchevsky |
| **List of Themes addressed** | 1. Creating a state registry of rare disease patients in Ukraine<br>2. Screening of newborns in Ukraine |
| **Annexes** | I. List of Participants (by stakeholders' categories)<br>II. Programme of the conference<br>III. Resolution (English, Ukrainian) |

## FINAL REPORT

As active members of the Rare Diseases (RDs) community, Ukrainians have a strong desire to be on the cutting edge of trends that spread nationwide with innovative initiatives, activities and projects that will ultimately improve the quality of life of rare disease patients. We understand the importance of developing a comprehensive national strategy and plan for rare diseases; covering all aspects from research to care. Basic steps to facilitate the diagnosis and support the identification of Ukrainian RD patients should be put in place first. Indeed, access to screening programmes, gene testing, genetic counselling as well as the development of a national registry of rare disease patients are crucial initial steps which need to be implemented within a specific legal framework.

### I. Introduction/ Plenary session

The conference started with the announcement of the themes identified as <u>key priorities</u> to be discussed.

Then, the Ombudsman declared that the independent institution of Ombudsman secures certain control over the governmental authorities to the extent of compliance by them with their respective obligations.

The Deputy Minister of Health agreed to make a step forward and rely on the experience of national and foreign experts in areas relevant for rare diseases. The ministry assures of its readiness to cooperate.

## II. Theme

Implementation of national actions for patients with rare diseases by creating a patient registry.

- **Identification of specific gaps, challenges and needs**.

The Law of Ukraine on Rare Diseases provides provisions for the creation of a national patient registry. Since the adoption of the law in 2014, the unavailability of enough patients' data has prevented the assessment of needed actions as well as the expenditure planning. The inaccuracy of accounting for the number of rare disease patients in the country results in incomplete provision of assistance.

The complexity in creating the registry is explained by the fact that the different institutions have different vision of the entity responsible for creating such registry and the data that is to be recorded. It was stated that the medical reforms launched in Ukraine is an additional factor preventing the creation of the registry. The desire of the Ministry of Health to cover multiple fields, including e-health tools to manage the patients, has delayed establishing the rare disease patient registry and thus, providing necessary assistance to these patients.

However, the registry created by the State Expert Centre of the Ministry of Health shows that establishing a rare disease patient registry would be feasible as well specific registries.

Simultaneously, it was pointed out that the quality of data depends on the availability of the designated expert assessment centres forming the central registry and the need for communication between the institutions and centres.

- **Assessment of the integration of European guidelines and policy recommendations into the national system.**

Subject to the European experience disclosed in the report, the participants have agreed that the application of the recommendations to create the patient registry may rely on the suggested principles and existing models may be applied in Ukraine following some adjustments.

One of the steps on the way with regard to implementing European policies is the inclusion of Ukrainian institutions in European Reference Networks (ERNs) for rare diseases. It was reported that next year (2018), ERNs will be expanded and will be open to European Union's neighbouring countries, although not being EU member states. The intent is to take part in this endeavour, emphasising that such action will result in agreeing on the joint quality criteria of centres. It is a chance to clear a border between institutions inside the country.

- **Expected outcomes in the near future, medium and long term.**

The key expectation is that the state would provide a legal framework for accounting of the number of patients and give them rights for assistance. As of 2018, the rights for medical treatment will be secured.

The medium term plans for 2018 to 2020 provide the expansion and creation of new programmes for newborn screening. This initiative combined with patient registration will facilitate early interventions, thus maximising positive treatment outcomes.

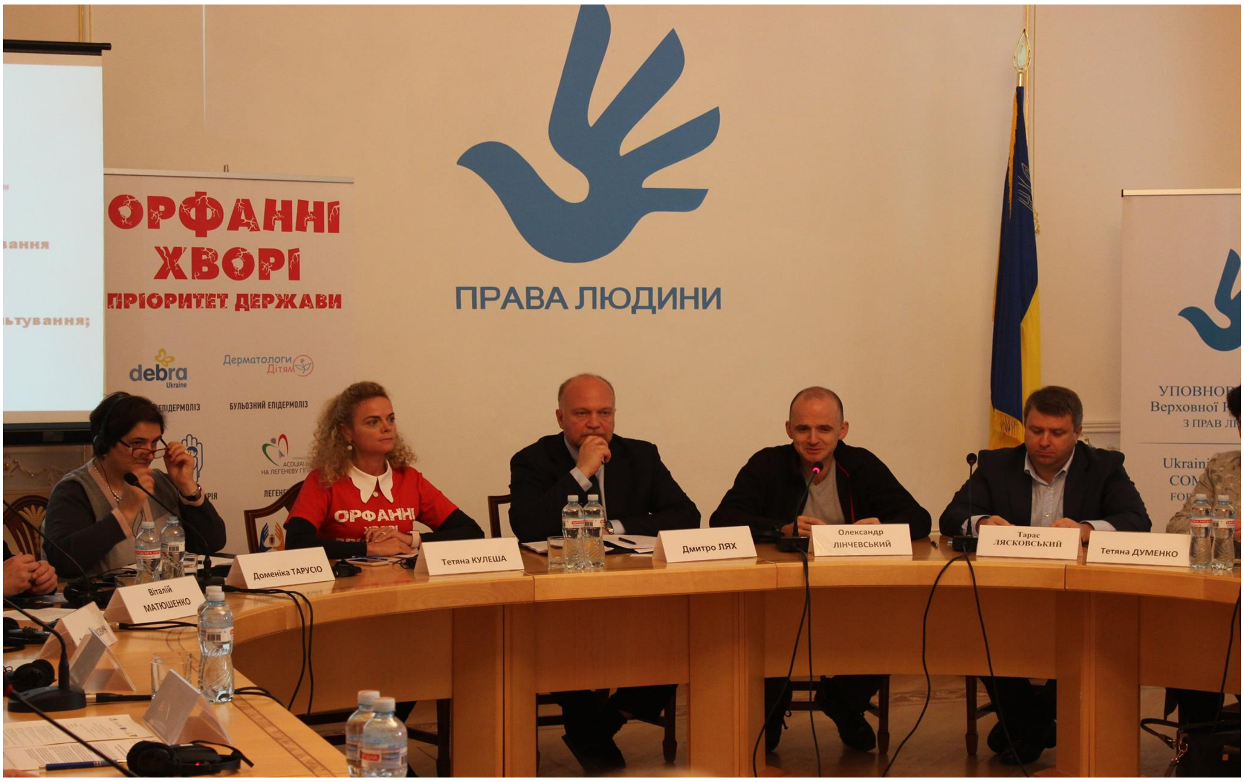
In the long run, rare disease patients getting not treatment will become controllable in terms of costs planning by the state.

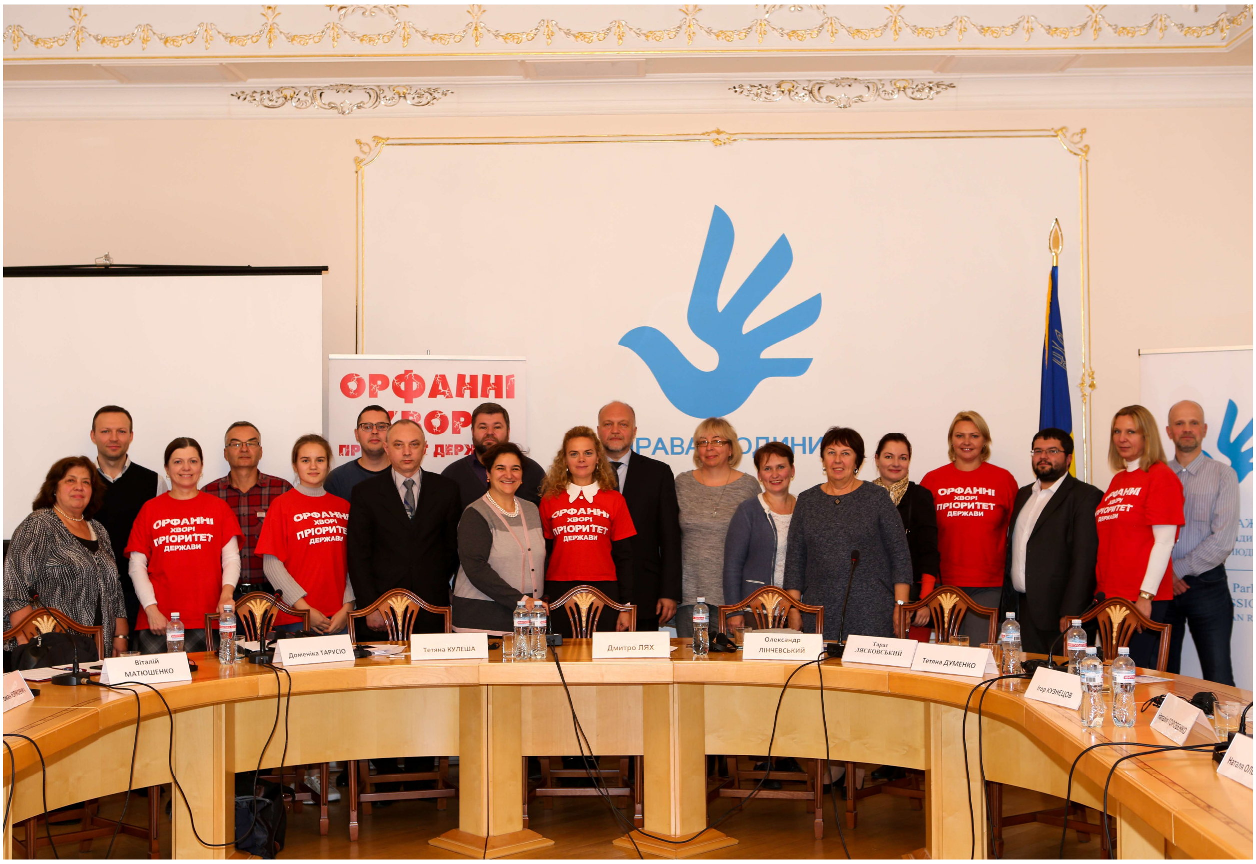
- **Which actions/ measures need to be taken.**

It has been decided to **create a working group**, **before the end of the year 2017, on the development of a methodology for the creation of the state rare disease patients registry.** This working group will include interested stakeholders under the control of the Commissioner for human rights of the Parliament of Ukraine. The Ministry of Health of Ukraine will be responsible for appointing an authority empowered to create and maintain the register.

## III. Conclusions

The development of a national rare disease patients registry is currently a bottleneck. This registry is crucially needed to inform any governmental decisions on the care management of Ukrainian rare diseases' patients and implement relevant and necessary measures.





# ANNEXES

## List of Participants (by stakeholders’ categories)

### State bodies:

Olexandr Linchevskiy, Deputy Minister of Health

Taras Lysnevskiy, Ministry of Health

Valeria Lutkovska, Commissioner for human rights of the Parliament of Ukraine (Ombudsman)

Dmytro Lyah, Deputy Ombudsman

Tatyana Dumenko, Director of State Expert Centre of the Ministry of Health of Ukraine

### Patients’ organisations

Tatiana Kulesha, Head of NGO Rare Diseases of Ukraine

Vitaliy Matyushenko, President of Kharkiv Foundation "Children with spinal muscular atrophy"

Tatiana Zamorska, President of Foundation “BE”

### Scientific bodies

Domenica Taruscio, Director of the National Center for rare diseases at the Italian National Institute of Health

Natalya Horovenko, Chief geneticist, Professor, Corresponding member of the Academy of Sciences of Ukraine

Natalya Olkhovich, Head of the Laboratory of Medical Genetics of the National Children's Specialized Hospital "Okhmatdit"

Lyudmila Livshits, Professor, Institute of Molecular Biology and Genetics, NAS of Ukraine

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# PROGRAMME

WORKSHOP ON IMPLEMENTATION OF NATIONAL ACTIONS FOR PATIENTS WITH RARE DISEASES

in the frame of the programme UKRAINE-EUROPLAN 2

October 20, 2017

**Meeting Time and Location - Instytutska str., 21/8,** Ombudsmen office. $10^{00}$ - $16^{30}$

**Kyiv, Ukraine**

**The themes of discussion:**

- Implementation of national actions for patients with rare diseases;
- Patient registry;
- Best practices of genetic diagnostics and genetic counselling;
- Selections from experience of European countries.

10.00 - 11.30 (1.5 hours)
Moderator - Tatiana Kulesha, Head of NGO Rare Diseases of Ukraine.

**Welcome speech on support of rare diseases' patients in Ukraine**
*Speaker - (5 minutes) Olexandr Linchevskiy - Deputy Minister of Health.*

**Introduction to Europlan**
*Speaker - (10 minutes) Domenica Taruscio, Director of the National Centre for rare diseases at the Italian National Institute of Health*

**Recall Ukraine - Europlan2**
*Speaker - (10 minutes) Vitaliy Matyushenko, President of Kharkiv Foundation "Children with spinal muscular atrophy"*

**Rare Diseases Registries in Europe**
*Speaker - (30 minutes) Domenica Taruscio, Director of the National Centre for rare diseases at the Italian National Institute of Health*

**Rare Disease Registries for Ukraine**
*Speaker - (30 minutes) Tatyana Dumenko, Director of State Expert Centre of the Ministry of Health of Ukraine*

11.30 – 12.00 **Discussion with representatives of legislative initiative, guarantying of sustainability and cooperative work**

12.00 – 12.15 coffee break (15 minutes)

12.15 – 14.15 (2 hours)

**A role of a patient organisation in help to the State**
*Speaker - (10 minutes) Tatiana Kulesha, Head of NGO Rare Diseases of Ukraine.*

**Screening, genetic counseling, a confirmation\diagnosis, government regulation\cooperation with genetic laboratories (academic, private and international)**
*Speakers -*
*(15 minutes) Natalya Horovenko, Chief geneticist, Professor, Corresponding member of the Academy of Sciences of Ukraine*
*(15 minutes) Natalya Olkhovich, Head of the Laboratory of Medical Genetics of the National Children's Specialized Hospital "Okhmatdit"*
*(15 minutes) Lyudmila Livshits, Professor, Institute of Molecular Biology and Genetics, NAS of Ukraine.*

14.15 – 15.00 lunch (45 minutes)

15.00 – 16.30 (1.5 hours)

**Discussion of practical implementation of the issues, the prospects for international cooperation towards united efforts in help to Rare Diseases' Patients**
Moderator - Tatiana Kulesha, Head of NGO Rare Diseases of Ukraine.

16.30 **Closing remarks and closure of the workshop**

18.00 – 22.00 dinner

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# Resolution

adopted at the seminar on implementation of national actions for establishing registers and ensuring treatment of rare disease patients

within the framework of Ukraine-Europlan 2 programme

Kiev, Ukraine

October 20, 2017

Participants of the seminar on implementation of actions for creating registers and providing treatment to rare disease patients who are represented by the patient organisations with rare diseases in Ukraine - members of the public association 'Orphan diseases in Ukraine', representatives of human rights Commissioner of the Parliament of Ukraine, representatives of the Ministry of Health, representatives of European Organisation on rare diseases EURORDIS, medical doctors, scientists and other participants of the seminar

after listening to the presentations of international experts, representatives of patients' organisations, representatives of state authorities and scientists;

taking into account:

current legislation of Ukraine;

international obligations of Ukraine;

proposals of the seminar participants;

Considering recommendations of the Council of the European Union and address on rare diseases of European Commission to European Parliament;

Having studied recommendations of EUROPLAN programme on the development of national plan and strategy for rare diseases and on implementation of them in terms of creation of registers of rare diseases;

based on:

international obligations of Ukraine before the Council of the European Union and governed by declared principles of European integration;

current situation on implementation of state policy in regard to rare disease patients;

Recognising importance and urgent need to create Sate register on rare (orphan) diseases;

it was decided:

1. To consider that the state Register of rare disease patients is an inseparable part of the development and implementation of the state policy on rare diseases and serves as a basis for accounting of patients, planning and providing measures on assuring complete, timely and uninterruptible aid to such patients, calculation of expenditures for provision of

medical drugs, medical products and dietary medical nutrition for the patients with rare diseases.

2. To create a working group on the development of a methodology for the creation of the state Register of rare disease patients with participation of representatives of Commissioner on human rights of the Parliament of Ukraine, representatives of the Ministry of Health, representatives of the European Organisation on rare diseases EURORDIS, medical doctors, scientists and representatives of public organisation “Orphan diseases of Ukraine”

3. To make Ministry of Health of Ukraine responsible for appointing an authority empowered to create and maintain the register.

4. To approach the Ministry of Health with demand to spread and to ensure the programmes of screening of the newborn children, as a method of early diagnostics of rare and other diseases and prevention of early disabling of the patients.

5. To create a unified system of giving specialised aid to the patients with rare diseases on the state and regional levels.

6. To execute provision of contemporary equipment to diagnostic centers that fulfill the function of the referent centers on diagnostics of rare diseases by areas.

7. To forward this resolution to all stakeholders and to put efforts into ensuring of their participation in the implementation of the resolution.

Steering Committee of the seminar:

Head of Public Union "Orphan diseases of Ukraine"

Tetyana Kulesha

Director of the National Centre of rare diseases of Italian National Institute of Health

Domenica Taruscio

Commissioner on human rights of the Parliament of Ukraine

Valeria Lutkovska

Deputy Minister of Health

Oleksandr Linchevsky

1. Recommendations of the EUCERD – European Union Committee of Experts on Rare Diseases: Rare Disease Patient Registration and Data Collection, 5 June 2013
2. Recommendations of the CEG-RD – European Commission Expert Group on Rare Diseases: Ways to Improve Codification for Rare Diseases in Health Information Systems, 12-13 November 2014
3. EURORDIS-NORD-CORD Joint Declaration of 10 Key Principles for Rare Disease Patient Registries
4. Fact Sheet on Orphanet – the portal for rare diseases and orphan drugs
5. Data sharing in large research consortia: experiences and recommendations from ENGAGE (European Network for Genetic and Genomic Epidemiology)

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# РЕЗОЛЮЦІЯ

## СЕМІНАРУ З ВПРОВАДЖЕННЯ НАЦІОНАЛЬНИХ ЗАХОДІВ ДЛЯ СТВОРЕННЯ РЕЄСТРІВ ТА ЗАБЕЗПЕЧЕННЯ ЛІКУВАННЯ ОРФАННИХ ПАЦІЄНТІВ

в рамках програми УКРАЇНА-ЄВРОПЛАН 2

***м. Київ, Україна***

*20 жовтня 2017 року*

Учасники Семінару з впровадження національних заходів для створення реєстрів та забезпечення лікування орфанних пацієнтів, які представлені пацієнтськими організаціями з рідкісними захворюваннями в Україні – членами Громадської спілки «Орфанні захворювання України», представниками Уповноваженого Верховної Ради України з прав людини, представниками МОЗ України, представниками Європейської організації з рідкісних захворювань EURORDIS, лікарями, науковцями та іншими учасниками Семінару

**ЗАСЛУХАВШИ** доповіді міжнародних експертів, представників пацієнтських організацій, органів державної влади, науковців;

**ВРАХОВУЮЧИ**:

чинне законодавство України;

міжнародні зобов’язання України;

пропозиції учасників Семінару;

**БЕРУЧИ** до уваги рекомендації Ради Європи та послання Європейської Комісії до Європейського Парламенту щодо рідкісних захворювань;

**ВИВЧИВШИ** рекомендації програми Європлан щодо розвитку національних планів і стратегій для рідкісних захворювань та виконання їх у частині створення реєстрів з рідкісних захворювань;

**СПИРАЮЧИСЬ**:

на міжнародні зобов’язання України перед Радою Європи та керуючись задекларованими принципами європейської інтеграції;

на поточний стан реалізації державної політики щодо пацієнтів з рідкісними захворюваннями;

**ВИЗНАЮЧИ** важливість та негальну необхідність створення Державного Реєстру з рідкісних (орфанних) захворювань;

**ВИРІШИЛИ:**

1. Вважати, що Державний Реєстр орфанних пацієнтів є невід’ємною частиною розвитку і впровадження державної політики щодо рідкісних захворювань та є основою для обліку пацієнтів, планування і забезпечення заходів по наданню своєчасної, повної та безперебійної допомоги таким пацієнтам, розрахунку витрат на забезпечення

лікарськими засобами, виробами медичного призначення та дієтичним лікувальним харчуванням пацієнтів з рідкісними хворобами.

2. Створити Робочу групу по розробці методології створення Державного Реєстру орфанних пацієнтів із залученням представників Уповноваженого Верховної Ради України з прав людини, представників МОЗ України, представників Європейської організації з рідкісних хвороб EURORDIS, лікарів, науковців та представників ГС “Орфанні захворювання України”.
3. Покласти визначення відповідального органу за створення і ведення Реєстру на Міністерство охорони здоров’я України.
4. Звернутися до МОЗ України з вимогою розширення та забезпечення програм скринінгу новонароджених, як методу ранньої діагностики рідкісних хвороб, з метою збереження життя і запобігання ранньої інвалідизації пацієнтів.
5. Створити уніфіковану систему надання спеціалізованої допомоги пацієнтам з рідкісними хворобами на державному та регіональних рівнях.
6. Здійснити забезпечення сучасним обладнанням діагностичних центрів, що виконують функції референтних центрів по встановленню діагнозу рідкісних хвороб (за напрямками).
7. Резолюцію направити всім зацікавленим сторонам та докласти зусиль для забезпечення їх участі у виконанні резолюції.

**Робоча група Семінару:**

| | |
|---|---|
| Голова Громадської спілки «Орфанні захворювання України» | **Тетяна Кулеша** |
| Директор Національного Центру з рідкісних захворювань Італійського Національного Інституту здоров’я | **Доменіка Тарусіо** |
| Уповноважений Верховної Ради України з прав людини | **Валерія Лутковська** |
| Заступник Міністра охорони здоров’я | **Олександр Лінчевський** |

1. Recommendations of the EUCERD – European Union Committee of Experts on Rare Diseases: Rare Disease Patient Registration and Data Collection, 5 June 2013
2. Recommendations of the CEG-RD – European Commission Expert Group on Rare Diseases: Ways to Improve Codification for Rare Diseases in Health Information Systems, 12-13 November 2014
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