

# HUNGARY

## EUROPLAN NATIONAL CONFERENCE

### FINAL REPORT

25 October 2013, Budapest



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# 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.

**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.

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## I. General information

Country	Hungary	
Date & place of the National Conference	Hunguest Hotel Griff*** (1113 Budapest, Bartók Béla u. 152.); 25/10/2013	
Website	europlan.rirosz.hu	
Organisers	<ul style="list-style-type: none"> <li>• HUFERDIS</li> <li>• Expert Group of Rare Diseases</li> <li>• Centre of Rare Diseases (In: National Health Institute)</li> </ul>	
Members of the Steering Committee	<p>Katalin Brunner, patient representative (HUFERDIS)  Dorica Dan, EURORDIS – EUROPLAN Advisor  Ildikó Horváth Kissné, government representative (EMMI)  György Kosztolányi, Coordinator of the National Plan (PTE)  György Németh, industry representative (Richter)  Gábor Pogány, patient representative (RIROSZ)  János Sándor, EUCERD representative (DE)  Helga Süli-Vargha, patient representative (RIROSZ)  Márta Szegedi, national insurance representative (OEP)  Ildikó Szy, government representative (EMMI)</p>	
Names and list of Workshops	A, Plenary	European policy and guidelines, Status of our National Plan
	1, Section	Methodology, Governance and Monitoring of the National Plan
	2, Section	Definition, codification and inventorying of RD (Information and training)
	3, Section	Research on RD
	B, Plenary	Reports of Worksop I-III.
	4, Section	Care for RDs - Centres of Expertise and European Reference Networks for Rare Diseases
	5, Section	Orphan Medicinal Products
	6, Section	Social Services for Rare Diseases
	C, Plenary	Reports of Worksop IV-VI., Closing
Workshop Chairs (and Rapporteurs, where applicable)	György Kosztolányi, János Sándor, József Vitrai, Márta Szegedi, Helga Süli-Vargha, Imre Boncz, László Szőnyi, Éva Oláh, Ferenc Oberfrank, Gergely Bujdosó, Sándor Túri, Katalin Komlósi, Gábor Pogány, Judit Mária Molnár, István Balogh, Judit Becskeházi-Tarr, Ildikó Szy, György Pfliegler, György Németh, Károly Fogarassy, Péter Horváth, Katalin Brunner, Beáta Boncz, Pál Vittay, Judit Váradiné Csapó	

## II. Main report

During the Hungarian EUROPLAN National Conference, six workshops were held, one for each of the six themes proposed in the National Conference common format. At the beginning of each workshop, depending on the theme, the relevant recommendations from the EU Council Recommendation on an action on rare diseases (2009/C 151/02) were quoted, followed by the EUCERD Indicators calculated to monitor the current situation as well as to follow future developments.

### A. The opening plenary session

- **Press Conference with Opening Ceremony**

Here we presented the Hungarian National Plan for RD first time. It is a strategy of health policy from 2014-2020 for RD.

- **Background knowledge about actions of European Union**

Dr. János Sándor presented the actions that were made at European Union level from the beginning to nowadays. He also presented the last EUCERD State of the Art report on both initiatives in the EU and activities in Hungary.

- **The situation of our National Plan**

Dr. György Kosztolányi, the Ministry Representative for the Hungarian National Plan gave an overview about the national actions, mainly about the preparation of the NP. He also spoke about the future plans.

### 1. Theme – Methodology, Governance and Monitoring of the National Plan

**Chairs:** Helga Süli-Vargha, Márta Szegedi, József Vitrai

**Date, place:** 25 October 2013, Hunguest Hotel Griff\*\*\*

#### **Council Recommendation (2009/C 151/02)**

*1. Establish and implement plans or strategies for rare diseases at the appropriate level or explore appropriate measures for rare diseases in other public health strategies, in order to aim to ensure that patients with rare diseases have access to high-quality care, including diagnostics, treatments, habilitation for those living with the disease and, if possible, effective orphan drugs, and in particular:*

*(a) elaborate and adopt a plan or strategy as soon as possible, preferably by the end of 2013 at the latest, aimed at guiding and structuring relevant actions in the field of rare diseases within the framework of their health and social systems;*

*(b) take action to integrate current and future initiatives at local, regional and national levels into their plans or strategies for a comprehensive approach;*

*(c) define a limited number of priority actions within their plans or strategies, with objectives and follow-up mechanisms;*

*(d) take note of the development of guidelines and recommendations for the elaboration of national action for rare diseases by relevant authorities at national level in the framework of the*

*ongoing European project for rare diseases national plans development (EUROPLAN) selected for funding over the period 2008-2011 in the first programme of Community action in the field of public health.”*

**EUCERD indicators**

INDICATOR	AREA OF COUNCIL REC. 2009/ C151/02	INDICATOR DESCRIPTION	TYPE OF INDICATOR	ANSWERS
1. Existence of Regulations/Laws, or equivalent official national decisions that support the establishment and development of a Rare Diseases (RD) plan	1	This Indicator refers to the fact that National Plans/Strategies for Rare Diseases should be devised/regulated at national level in accordance with the Council Recommendation on RD, relevant Recommendations of the EUCERD e.g. those on Centres of Expertise and European Reference Networks, as well as relevant legislation (Regulation EC n° 141/2000 on Orphan Medicinal Products, Directive EU/2011/24 on Cross Border Healthcare, etc.). The National Plan or Strategy is adopted via binding legislative acts, the exact nature or level of which may vary (regulation, laws, or other types of decisions). They may be established at the appropriate level of governance (federal vs. federated state level) depending on the country’s system of government. It is therefore embedded in a legislative or operational framework	Process	In progress /in development
2. Existence of a RD advisory committee	1	The Expert Advisory Committee refers to the existence of a coordination mechanism that oversees the development and implementation of the National Plan/Strategy for Rare Diseases. This body is composed of representatives of all relevant stakeholders, including patient representatives, national government, industry, treating physicians, payers, academia, etc.	Process	YES, exists but partly functioning and includes all relevant stakeholders
3. Permanent and official patients’ representation in plan develop	6	Patients are officially represented at all stages of plan development and governance, including its monitoring and evaluation.	Process	YES, but only as observers
18. Existence of a policy/decision to ensure long-term funding and/or sustainability of the measures in the RD plan/strategy t	7	The indicator verifies whether the financial commitment for rare disease care and treatment is clearly defined in a budget decision that supports the implementation of the National Plan/Strategy actions.	Process	In progress /in development
19. Amount of public funds allocated to the RD plan/strategy	7	The indicator is the overall budget (in EUR) allocated per year to the National Plan/Strategy (excluding reimbursement of care and cost of standard care, excluding cost of orphan drugs). As with the previous indicator, this indicator aims to ensure that RD actions include appropriate provisions to ensure their sustainability over time. Efficient and effective actions for rare diseases depend on integrating scarce and scattered resources both nationally and within a common European effort. .	Outcomes	3,022,600 EUR/7 years

**1.1 Mapping policies and resources**

**1.1.1.** The level of awareness on rare diseases and on patients’ conditions can be considered not too high in Hungary. It should be noted that Rare Diseases Day which has been organised already six times in Hungary and the associated publicity and media events increased significantly the level of awareness.

**1.1.2.** The mapping of resources, actions and research in health and social systems related to rare diseases has been started in 2009 led by the National Rare Diseases Centre (NRDC). The

dissemination of information on rare diseases, relevant services and drugs, patients' organizations has been started using Orphanet Internet facilities. The update of this knowledge base needs more dedicated resources. Professionals working with patients with rare diseases have built networks which provide good opportunity through formal or informal information exchange on actions, programmes in the field of diagnosis, care and research.

**1.1.3.** Although data coming from EurordisCare<sup>1</sup> and BURQOL-RD<sup>2</sup> surveys contribute to more detailed picture of the needs of Hungarian patients with rare diseases, detailed epidemiological data on rare diseases in Hungary are not available

**1.1.4.** A comprehensive picture on rare diseases in Hungary is presented in the National Plan based on all the information collected since 2008.

## 1.2 Development of a National Plan /Strategy

**1.2.1** As a legal and policy framework in Hungary, the National Plan for Rare Diseases has been just finished and presented in this EUROPLAN workshop. The preparation of the Plan was coordinated by the Minister for Health and the National RD Centre (NRDC) which was established in 2008 as a part of the National Centre for Healthcare Audit and Inspection (OSZMK). Due to the restructuring of the national public health system, the National Institute for Health Development (NIHD) became the new NRDC host organisation. The NRDC's main targets are to contribute to the development of collaboration between governmental bodies, providers and patient organisations; to maintain the national database of RD specialised health care providers; to support all RD related programs (screening, research, international coordination, prevention, teaching, etc.). The National Plan related task of NRDC is to co-ordinate the elaboration and monitoring of national policy on rare diseases. The development of the Plan was greatly moved forward by the previous National EUROPLAN Conference organized by HUFERDIS in 2010 as well as by other EUROPLAN-based model rare disease conferences organised in 2011 and 2012.

**1.2.2** In 2011, the Ministry of Human Resources appointed a National Coordinator. With his coordination, the NRDC and the Ministry started to develop a Plan with the Rare Diseases Expert Committee (comprising all rare disease stakeholders: healthcare professionals, academics, national health insurance representatives, social affairs authorities and patients). At the end of 2011, the main content of the Plan was finalised, except for chapters on social issues, research activity, orphan and therapeutic tools supply, which are in progress. By the time of this National Plan conference, the National Plan has been signed by the Minister for Human Resources.

**1.2.3** The EU Council Recommendation on an action in the field of rare diseases (2009/C 151/02) and the Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare were taken into account in view of the development of the National Plan.

## 1.3 Structure of a National Plan /Strategy

**1.3.1.** The Hungarian National Plan was developed according to the recommendations for the development of national plans for rare diseases prepared by the European Project for Rare Diseases National Plans Development (EUROPLAN 2008-2011). Its structure follows the guidelines provided by EUROPLAN.

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<sup>1</sup> Surveys carried out by EURORDIS to investigate patients' experiences and expectations regarding access to diagnosis and to health services.

<sup>2</sup> BURQOL-RD is a project co-funded by the European Commission aimed to quantify the socio-economic costs and Health Related Quality of Life (HRQOL) for both patients and caregivers.



**1.3.2.** The health care administration sets the general aim of providing the highest quality of life possible for patients with rare diseases.

Primary aim: In accordance with the principle of equal treatment and solidarity, improvement in the diagnostics and treatment of rare diseases with a multidisciplinary approach, improvement of access to high-quality medical care and therapeutic options by creating rationalised patient pathways as well as supporting related education and research by exploiting the potential in the European cooperation.

Further objectives:

- Improvement in the diagnostics of rare diseases for early detection of diseases
- Creation and operation of multidisciplinary CEs (Centres of Expertise) for high-quality patient care
- Improvement of education and training for high-quality patient care
- Improvement of newborn screenings for establishing early diagnoses
- Improvement of access to medicine and medical appliances
- Support of research efforts in the field of rare diseases
- Development of social care and access to its services

**1.3.3.** There are specific actions envisaged in the National Plan accompanied by clear deliverables and measurable results in the form indicators.

**1.3.4.** In the National Plan a clear timeline shows the start and closing dates for all priority actions with specific deliverables.

#### **1.4 Governance of a National Plan**

**1.4.1.** The National Plan does not explicitly determine the governance of the Plan. Currently there is no appointed Steering Committee, state or governing body to coordinate or implement the Plan. As the representative of the Ministry informed the workshop, this task should be delegated to the Department of Health Policy under the Secretary of Health at the Ministry of Human Resources. Some participants noted that since the issue has numerous direct links to the social affairs this solution seems not likely. Should the Department of Health Policy take over the duty of governance of the National Plan, then the members of the Steering Committee would be appointed instead of elected.

**1.4.2.** According to the National Plan, the Expert Committee of Rare Diseases has a central role to assist in the decision-making activities related to the National Plan. Therefore all rare disease stakeholders will participate in the governance of the National Plan. These stakeholders cover all areas of expertise relevant to the National Plan such as pharmacology, regulatory, clinical, health and social services, epidemiology, administrative policies, etc. Through the Expert Committee of Rare Diseases, patients will participate in all phases of the National Plan so that they can decide on measures directed to them.

The professional body of the above mentioned NRDC would be competent to undertake this task; however, this would require extending its authorisation as well as involving the representatives of the pharmaceutical industry and the governmental sector into the process.

## 1.5 Dissemination and communication on the National Plan

**1.5.1.** The process around formulating the National Plan was open and transparent. The rare diseases patients could follow the process through their representatives working in the development of the National Plan.

**1.5.2.** The National Plan was first presented in this workshop and publicised to the general public via printed and electronic media. It is made public in all its parts, including all specific actions, timelines and results of its evaluation when performed.

**1.5.3.** According to the National Plan, the resources and rehabilitation centres will also have the duty of strengthening social awareness and the dissemination of knowledge. Moreover, the planned graduate education programmes in non-medical teacher's schools and colleges will provide information on the special problems of patients with rare diseases aiming at raising awareness of rare diseases. No specific communication actions are envisaged in relation to the different phases of the life cycle of the National Plan. Besides the National Plan, the Ministry plans rare disease specific communications in the project "Development of public health communication", supported by the EU Cohesion Fund.

## 1.6 Monitoring and evaluation of the National Plan

**1.6.1.** The EUCERD 21 Core Indicators are planned to be used to monitor the implementation of the National Plan. Some of the EUROPLAN Indicators were also selected to monitor the Plan.

**1.6.2.** There are no explicit statements relating to the evaluation of the National Plan. Participants in the workshop agreed that a well experienced external body, with international experience in the field of health and social affairs should be invited for evaluating the Plan.

**1.6.3.** It is necessary to increase the awareness on rare diseases in the national health care and social care systems by setting up registries on the available resources, activity programs, research (i.e. by turning initials of the NRDC webpage to be useful and functioning. The first steps of achieving this goal could be realized, nonetheless, by setting up an Orphanet compatible Hungarian registry, through the integration of the Orphanet registry, by establishing a compatibility with the existing Hungarian registries, as well as by insuring the availability of the whole Orphanet database in Hungarian.

## 1.7 Sustainability of the National Plan

**1.7.1.** A specific budget is attached to the National Plan in an amount of 3,022,600 EUR/7 years. The financial sources are different: in most of the budget lines the European multiannual financial framework 2014-20 is specified.

**1.7.2.** Each primary action has cost estimates with possible sufficient financial resources. The allocation of the budget by action seems adequate and altogether, the overall budget allocation promises ensuring the long-term sustainability of the actions planned.

## 1.8 Recommendations

**1.8.1.** The Ministry should appoint and - if needed- enlarge a Steering Committee responsible for the development and implementation of the plan. Participation of each stakeholder must be ensured, such as health care authorities, patients, medical experts, researchers, representatives of the industry etc. These participants could cover all the affected territories, i.e. pharmaceutical industry, state, clinics, health care and social care services, epidemiology, administration etc. The coordination mechanism is not clearly determined. The coordination role of NRDC at Nat. Inst. for Health Development needs to be more articulated.

**1.8.2.** The Steering committee should meet regularly (at least 3-4 times a year). At least once a year, a public report should be produced on the committee’s activities, on the outcome of its goals and on the activities of persons with key responsibilities. This report could be released on the International Rare Disease Day and could be available later on the webpage of NRDC.

**1.8.3** It should include statements on the regular revision of the National Plan and the external evaluation process.

**1.8.4** Make clearer in the National Plan where patients’ organisations will be involved in decision making.

## **2. Theme – Definition, codification and inventorying of RD (Information and training)**

**Chairs:** Imre Boncz, László Szőnyi, János Sándor

**Date, venue:** 25 October 2013., Hunguest Hotel Griff\*\*\*

### **Council Recommendation (2009/C 151/02)**

*2. Use for the purposes of Community-level policy work a common definition of rare disease as a disease affecting no more than 5 per 10 000 persons.*

*3. Aim to ensure that rare diseases are adequately coded and traceable in all health information systems, encouraging an adequate recognition of the disease in the national healthcare and reimbursement systems based on the ICD while respecting national procedures.*

*4. Contribute actively to the development of the EU easily accessible and dynamic inventory of rare diseases based on the Orphanet network and other existing networks as referred to in the Commission Communication on rare diseases.*

*5. Consider supporting at all appropriate levels, including the Community level, on the one hand, specific disease information networks and, on the other hand, for epidemiological purposes, registries and databases, whilst being aware of an independent governance.”*

### **EUCERD Indicators**

<b>INDICATOR</b>	<b>AREA OF COUNCIL REC. 2009/ C151/02</b>	<b>INDICATOR DESCRIPTION</b>	<b>TYPE OF INDICATOR</b>	<b>ANSWERS</b>
4. Adoption of the EU RD definition	2	The EU defines “rare diseases” as those with a prevalence of no more than 5 patients per 10.000 persons. This definition is laid down in Regulation EC n° 141/2000 on Orphan Medicinal Products, Directive 2011/24/EU on Cross Border Healthcare as well as in the Council Recommendation on an action in the field of rare diseases of 8 June 2009. I.	Process	YES, the NP/NS measures are applied using the EU definition
8. NP/NS support to the development of/participation in an information system on RD	2	This indicator includes the participation in the Orphanet Joint Action and eventually the production of information packages in national language(s).	Process	No, neither national or regional system, YES, participates in Orphanet JA and produces information in national language

9. Existence of Help lines for RD	2 & 6	<p>The availability of help lines is fundamental for the diffusion of information and expertise on rare diseases. They have an important role in orienting patients towards a solution to the issues that directly or indirectly affect him/her as a result of the condition and are the only service that can offer social, psychological and information solutions to all of these needs.</p> <p>Professionals (including those working in emergency departments) may learn about resources and pathways to diagnose their patients or receive important information regarding the management of patients with a rare disease.</p> <p>This indicator aims to account for the national help lines on rare diseases, either aimed at patients or professionals (or both), including those not publicly funded.</p>	Process	<ul style="list-style-type: none"> <li>• No</li> </ul>
11. Type of classification/coding used by the health care system	2	<p>The adoption and the daily use of an internationally recognised, comprehensive, health care codification system is important for RD management and would encourage the harmonisation of disease nomenclature worldwide. This enables budgetary and management decisions to have a more solid basis and would constitute one relevant tool for Health Technology Assessment.</p>	Process	<ul style="list-style-type: none"> <li>• ICD-10</li> <li>• No ORPHA Code</li> </ul>
12. Existence of a national policy on registry and data collection on RD	2, 3	<p>This indicator collects information on Member States' support, at all appropriate levels, to rare diseases registries and databases for epidemiological, public health and research purposes, as well as on the role ensured by public authorities for the coordination and sustainability of data collection.</p>	Process	<ul style="list-style-type: none"> <li>• YES, for national/centralised registry and data collection</li> <li>• YES, for regional registry and data collection</li> </ul>

## 2.1 Definition of RD

Presently the EU's official definition functions as the accepted definition, with a rare disease defined by a prevalence of less than 5/10 000 in the population. With 5 percent of the general population affected by one of the estimated 6000 rare diseases, this amounts approximately 20-30 million people in EU. The high number of patients and their family members, the burden of rare disease on them illustrate the size of the problem and the importance of managing it.

## 2.2 Codification of RD and traceability in national health system

**2.2.1** In current national health information system, there is possibility to follow the RD pathway, if RD has an individual ICD code. About 250 RD have individual ICD codes in ICD-10 coding system used in Hungary.

**2.2.2** The **codification system** is used for the registries and the normative **cost refund**.

**2.2.3** The World Health Organization (WHO) is undergoing the new revision of ICD, in order to ensure better traceability of RDs in health information systems by appropriate codification. The final ICD-11 is expected by 2017, when about 2500 RD specific codes will be available in coding system.

The National Registry of Congenital Disorders (VRONY) is member of the network of European Surveillance of Congenital Anomalies (EUROCAT) since 2003. Aim of ICD-11 is to facilitate accurate codification and classification of congenital anomalies, so therefore VRONY made a proposal in EUROCAT Joint Action for a ICD-11 revision of congenital anomalies.

**2.2.4** The codification is the basis of the claim for a normative subsidy, and often the physicians have opposing interests and prefer not to use the codes belonging to the exact diagnosis for reaching better financing through symptomatic treatment codes. Accurate registration of RDs is

achievable as a result of using Orpha codes and to build in Hungarian electronic data collection. 6760 RDs have an Orpha code.

## 2.3 Registries and databases

**2.3.1** VRONY is a database covering the whole Hungarian population – first in the world and unique in Europe. Doctors must report every case of congenital anomaly under 1 year of age according to a Hungarian law (1997/XLVII, published in: *Népjóléti Közlöny*, 1997. 12, 1722-4). VRONY is collecting notifications of congenital anomalies in electronic and online ways to comply with recommendations of EUROCAT from 2009 (e-VRONY). One obligation concerns that patient get a diagnosis under the age of one. The aim is to produce an integrated information system suitable for collaboration with international registries, which contains disease and health care specific data of Hungarian rare patients, make data accessible to users, and provides information to health care professionals, funding, and decision-makers. A RD population registry has the potential to collect data and follow patients, using ICD-10 codes of RDs, suitable for automatic adoption of ICD-11 expected by 2017. Making VRONY part of RD register should be considered.

**2.3.2** At the moment these registers and programs do not receive separated government support.

## 2.4 Information on available care for RDs in general, for different audiences

**2.4.1** It must be ensured that every affected and inquiring person gets the adequate quantity and quality information about RDs. It is important that everyone gets the information in the right time and form. For professionals it means ensuring that they attend trainings and forums. For the population at large, different communication channels should be used and public institutions (e.g. educational institutions) should disseminate this information.

**2.4.2** The EU RD definition ( $\leq 5/10\ 000$ ) must be used in order to facilitate the national and international cooperation, and action at community level (in e.g. diagnostic work, patients' registration, treatment and care).

**2.4.3** The using of the common EU registration "practice" (ORPHANET) should be supported.

**2.4.4** Good coding practice must be supported by:

- (1) thinking again the financing mechanism,
- (2) introducing and using ICD-11 (from 2017) and ORPHA codes, as well as
- (3) training professionals engaged in coding to facilitate the traceability of patients in health care system.

**2.4.5** Cross-references could be insured between different classification systems in Hungary. This could help us connecting to European systems like ORPHA code system.

**2.4.6** Participating in revising ICD-10 should be assured, allowing the immediate introducing of ICD-11 after the presentation.

**2.4.7** Possibility of adequate education and training should be ensured for all health professionals in field of RDs.

**2.4.8** Integrate use of administrative, demographic and health care data sources should be supported by adequate acts in case of managing of RDs. Training of health informatics should focus on creating and operating EU compatible registers. Students have to learn the good coding practice.

- 2.4.9** Health authorities should encourage data collection, dissemination and access for these data and information, as well as using them for healthcare purposes; all this, of course, in accordance with national regulations. Access to data should be ensured for researchers, patient organizations (and individual patients if necessary). Someone who is offering data should have the right to access these database or system in order to control pertinence of data.
- 2.4.10** In every reference center there must be a trained healthcare professional who manages the coding and registers.
- 2.4.11** Support the connection and participation of national registries to the existing European and international registries.
- 2.4.12** Identify the national and EU opportunities of register financing.
- 2.4.13** Support and encourage the special RD or RD groups and their organizations and alliances in preventive, research and curative activities.

## 2.5 Help Lines

- 2.5.1** It would be necessary creating and operating a help-line free of charge that could help RD patients and their families, as well as people who meet RD patients (e.g. family practitioners) to get aid on medical and social issues.
- 2.5.2** The person who answers the calls should be well-informed on RD related health questions and problems. She/he should coordinate the patients whether for health care, social or fellow sufferer help.
- 2.5.3** The human (knowledge, skills) and material (workstation, communication tools) needs of operating the help-line are well defined and its expected benefit is predictable.
- 2.5.4** The call administrative program of European help line system could be joined (rapsodyonline). The European free helpline number network (116), if and when set up, should be joined in order to draw on the resources of this network. The quality control of information and implementation of monitoring satisfaction is also required.

## 2.6 Training healthcare professionals to recognise and code RD

- 2.6.1** The number of medical and paramedical professionals in field of RDs should be increased and medical training should be emphasized. The curriculum should be focus on genetic, diagnostic, treatment and psychological themes. By improving training and increasing the number of professionals, the diagnostic delay could decrease and the quality of health care could improve, which would generate a better quality of RD patients' life.
- 2.6.2** Until now, the following training-related improvements have been achieved (in gradual and post gradual training): classroom lectures, mandatory optional credit courses, diploma and PhD thesis, further trainings (for internist and family practitioners), PhD courses, conferences and establishment of working committees.
- 2.6.3** The good coding practice is very important. There is a possibility to follow patients in hospital discharge records but it requires an appropriate coding method (it means that ICD-10 codes of RDs would be used and well used). Many more RDs will have their own ICD code in ICD-11 that supplemented with a good coding practice could give an opportunity to carry out epidemiological investigations. In addition introducing ORPHA codes and operating RD register(s) could improve and help the visibility and traceability of RD patients in the healthcare system.
- 2.6.4** Accreditation and coordination of the mentioned trainings and events is important.

## 2.7 Training healthcare professionals

**2.7.1** Continuous information and training of health care workers is necessary in RD-related topics. All possible devices should be used for this purpose (e.g. preparing information material for each profession, organizing trainings and conferences).

**2.7.2** Continuous information and training for professionals dealing with RD patients must be provided.

## 3. Theme – Research on RD

**Chairs:** Éva Oláh, Gergely Bujdosó

**Date, venue:** 25 October 2013., Hunguest Hotel Griff\*\*\*

### Council Recommendation (2009/C 151/02)

*6. Identify ongoing research and research resources in the national and Community frameworks in order to establish the state of the art, assess the research landscape in the area of rare diseases, and improve the coordination of Community, national and regional programmes for rare diseases research.*

*7. Identify needs and priorities for basic, clinical, translational and social research in the field of rare diseases and modes of fostering them, and promote interdisciplinary co-operative approaches to be complementarily addressed through national and Community programmes.*

*8. Foster the participation of national researchers in research projects on rare diseases funded at all appropriate levels, including the Community level.*

*9. Include in their plans or strategies provisions aimed at fostering research in the field of rare diseases.*

*10. Facilitate, together with the Commission, the development of research cooperation with third countries active in research on rare diseases and more generally with regard to the exchange of information and the sharing of expertise.”*

### EUCERD Indicators

INDICATOR	AREA OF COUNCIL REC. 2009/ C151/02	INDICATOR DESCRIPTION	TYPE OF INDICATOR	ANSWERS
13. Existence of a RD research programmes/projects in the Country	3	This indicator aims to describe the status of RD research in the country, most notably whether a dedicated programme exists, or whether RD research is carried out by individual projects within the general research programme.	Process	YES, specific PROJECTS for RD within general research programme
14. Participation in European and international research initiatives	3	Participation of national research agencies in international research initiatives (such as E-RARE – www.e-rare.eu, and IRDiRC – www.irdirc.org) is important to foster research on rare diseases a global level, by pooling resources and coordinating national research programmes to overcome the fragmentation of research on RD	Process	YES, E-RARE
20. Specific public funds allocated for RD research	3	This indicator aims to identify the policy decision(s) to allocate a portion of the national research budget specifically to RD research.	Process	No
21. Public funds specifically allocated for RD research actions/projects per year since the plan started	3	This indicator verifies the total amount of public funds (in EUR) allocated to RD research projects or programmes	Outcomes	N/A: it is incorporated in the general research funds

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

**3.1.1** Although attempts to map research resources for programmes for RDs and its different connected infrastructures have been made several times in the past, due to the diversity of the matter and the lack of appropriate financial incentives, all of them turned out to be unsuccessful. Hence, the evaluation of private and governmental finance opportunities which could be used to sustain these programmes have been abandoned as well.

**3.1.2** Currently there is no existing **special national RD research programme** funded from a purposefully isolated resource; at the same time, there are no obstacles to launch such a programme. There is a great need for it, done in a controlled, co-ordinated manner.

**3.1.3** The interconnection of basic and translational research with the centres of expertise is currently not regulated, though it would be necessary, since care, diagnosis, and research in RD are closely related to each other.

**3.1.4** Depending on the given research, the promotion of an **overall interdisciplinary view in research** realizes itself only incidentally.

### 3.2 Dedicated RD research programmes and governance of RD research funds

**3.2.1** The point is not relevant, because there are no isolated programmes yet.

**3.2.2** Every university organizes its RD research base in a different way. Therefore, it is unwise to elect one of them as a co-ordinating centre. At the same time, there is need for an independent co-ordinating centre.

### 3.3 Sustainability of research programmes on RD

**3.3.1** This point is also not relevant.

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

**3.4.1** Some attempts have been made to map the needs and priorities for research, but they brought no overall results.

**3.4.2** Similarly to the College of Clinical Genetics' survey on genetics care system, it is necessary to survey other research institutes (the survey is ready, international relations are included).

**3.4.3** Hungary stays connected to most of the research projects investigating the quality of life and needs of the patients with the help of HUFERDIS. It is necessary to direct the existing research resources for students into this direction (Students' Scholarly Circle topics).

Regarding basic, translational and clinical public health research, interest and capacity of university clinics and pharmaceutical industry is determinant. In these cases, civil society organizations take part in the recruitment of patients, in the development of research protocols and in sharing results at international level.

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

**3.5.1** Regarding research in the field of social sciences, member organizations of HUFERDIS show active cooperation and HUFERDIS also has a strong mobilizing force to the joint organizations.

**3.5.2** There are no special programs for supporting and recruiting young researchers but general opportunities are available in the field of RDs (ex. scholarships, study-tours).



- 3.5.3** Some patient organizations try to motivate the expansion of clinical trials to Hungary (SMA, MPS, etc.).

### **3.6 RD research infrastructures and registries**

- 3.6.1** Technical platforms and infrastructure including clinical, social and health industry research related to RDs should be developed and supported in order to improve everyday care of patients with RD. A possibility of cooperation between public and private services should be investigated.
- 3.6.2** The National Health Insurance Fund (OEP) should support some new examination methods (like new generation sequencing, micro-array CGH).
- 3.6.3** There are several research registries, but with different types of infrastructure and system.
- 3.6.4** The VRONY database is a good basis, if the data of patients older than one year were collected. Emphasized value of “Homogeneous patient group” (HPG) would improve the discipline of reporting.

### **3.7 EU and international collaboration on research on RD**

There are collaborations, however, encouragement and broadening is needed in the fields of basic, translational and clinical public health research, especially:

- BioBank Pécs joined E-RARE
- Corvinus University: participation to the BURQOL-RD project
- University of Debrecen: participation to EurordisCare2
- NRDC participates in the EU-supported „EUROCAT Joint Action” epidemiology research programs regarding rare diseases (mainly syndromes).

### **3.8 Recommendations**

- 3.8.1** Special national research programs concerning RDs are necessary (basic, translational, clinical, public health and social), and these should be supported from funds dedicated to these fields, possibly in the long run. However, not a transformation but an improvement and sustainability of present research funding system could move this field forward (notably more RD tenders from the Medical Research Council - ETT).  
Research tenders related to RDs should be made identifiable and traceable within the wider national research programs.
- 3.8.2** In defining research priorities and in the transparency of ongoing research, a constant interaction between researchers and patient organizations is needed.
- 3.8.3** National networks should be motivated to investigate RDs. Special attention is needed in the fields of translational and clinical research in order to facilitate application of new knowledge in RD therapy. In the same time, registry of research teams working on RDs should be developed.
- 3.8.4** Multidisciplinary national and international research should be promoted to reach a critical number of patients for clinical tests and to use international professional knowledge.
- 3.8.5** Specific programs should be initiated to support and recruit young researchers working on RDs. To motivate professionals (medical and paramedical professionals) and young researchers for studying RDs, a special scholarship fund should be established (following EU example, based on either governmental or public funds).

- 3.8.6** Support the survey of usage of new combination or new therapeutic targets of existing drugs, since this is a cost-efficient method of development of RD treatment.
- 3.8.7** Appropriate initiatives should be formed to foster participation in international research enterprises related to RDs including EU framework E-RARE and IRDiRC. National support of these initiatives should be consistently raised.
- 3.8.8** Access to EU projects and information should be guaranteed for national research centres.
- 3.8.9** Specification of separate source of support is needed for patient organizations to join EU research.
- 3.8.10** Patient organizations should be regularly updated about recent research and their results, ex. research centres invite patient organizations to their scientific councils.

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

**Chairs:** Judit Mária Molnár, István Balogh, Judit Becskeházi-Tarr

**Date, venue:** 25 October 2013., Hunguest Hotel Griff\*\*\*

#### **Council Recommendation (2009/C 151/02)**

*11. Identify appropriate centres of expertise throughout their national territory by the end of 2013, and consider supporting their creation.*

*12. Foster the participation of centres of expertise in European reference networks respecting the national competences and rules with regard to their authorisation or recognition.*

*13. Organise healthcare pathways for patients suffering from rare diseases through the establishment of cooperation with relevant experts and exchange of professionals and expertise within the country or from abroad when necessary.*

*14. Support the use of information and communication technologies such as telemedicine where it is necessary to ensure distant access to the specific healthcare needed.*

*15. Include, in their plans or strategies, the necessary conditions for the diffusion and mobility of expertise and knowledge in order to facilitate the treatment of patients in their proximity.*

*16. Encourage centres of expertise to be based on a multidisciplinary approach to care when addressing rare diseases.”*

**EUCERD Indicators**

INDICATOR	AREA OF COUNCIL REC. 2009/ C151/02	INDICATOR DESCRIPTION	TYPE OF INDICATOR	ANSWERS
5. Existence of a national policy for establishing Centres of Expertise on RD	4	This policy defines a strategy to identify and designate centres of expertise, aiming to improve the quality of health care by defining appropriate centres with experience on RD as well as pathways that reduce the diagnosis delay and facilitate both care and treatment for RD patients.	Process	In progress/in development
6. Number of national and regional Centres of Expertise adhering to the national policy	4	Member States identify and appoint Centres of Expertise (CEs) throughout their national territory, and consider supporting their creation. The Centres of Expertise should adhere to the national policy. It is to be remembered that the EUCERD adopted the “EUCERD Recommendations on Quality Criteria for Centres of Expertise” which are “intended to help EU Member States in their reflections or policy developments concerning national plans and strategies for rare diseases when addressing the issue of organisation of healthcare pathways at national and European level”. This indicator therefore also aims to count the number of Centres of Expertise that are compliant with the EUCERD recommendations.	Outcomes	official 0, informal about 8
7. Participation of national or regional centres of expertise in European Reference Networks	4	The information on the integration of national Centres of Expertise in European Reference Networks (ERNs) is essential to obtain the broader picture of RD care across Europe and enables the diffusion of expertise across the EU, regardless of the size/population of each country. According to the “EUCERD Recommendations on European Reference Networks for Rare Diseases”, different forms of affiliation to an RD ERN (association, collaboration) should be allowed to ensure inclusivity.” Therefore this indicator aims to differentiate between full and associated membership of RD Centres of Expertise to RD ERNs.  However, it should be taken into account that it will take some time before ERNs are established. Therefore it should be expected that this Indicator will provide meaningful information only a few years after the adoption of these Recommendations.	Outcomes	0
10. Existence of a national policy for developing ,adapting and implementing clinical practice guidelines	2	The indicator checks the existence of a policy for developing, adapting and implementing clinical practice guidelines (CPGs) for diseases/groups of diseases (“Adapting” refers to adaption of supra-nationally based clinical guidelines to the local context).The cumulative production of protocols and clinical guidelines is an instrument for equity of access to care by rare disease patients across the European Union.	Process	<ul style="list-style-type: none"> <li>• YES, a policy exists for developing CPGs</li> <li>• YES, a policy exists for adapting CPGs,</li> <li>• YES, a policy exists for implementing CPGs</li> </ul>

**4.1 Designation and evaluation of CE**

**4.1.1** The structures which provide expertise on rare diseases, are partly mapped, their different roles and competences have been acknowledged. There are four university centres of expertise with diagnostic and therapeutic facilities: Budapest, Szeged, Pécs, and Debrecen. These future CEs are providing specialized services in connection with specialized laboratories and other institutions.

- 4.1.2** The designation of CEs is the responsibility of the Ministry of Health. Much of the designation criteria for CEs (objectives, scope, task, indicators, etc.) have already been defined on the basis of EUCERD Recommendations (24/10/2011, [http://www.eucerd.eu/?post\\_type=document&p=2204](http://www.eucerd.eu/?post_type=document&p=2204)) in the National Plan.

The selection and quality requirements are defined in the principles and criteria of EUCERD Recommendations, however some adaptation is yet to come, for instance: all elements of Quality management system and/or the Accreditation system; to assess whether the designation criteria are such to adapt to the characteristics of the disease or group of diseases covered by each CE; conditions and possibilities for cooperation between centers of expertise; conditions for participation in clinical trials; performance measurement method of the each CE).

- 4.1.3** The process of designation of CEs is at initial stage in Hungary. The MoH is currently working on the development of the designation process, therefore a national directory and guideline of designation of CEs has not yet been generated.
- 4.1.4** The patient organizations have not been involved in the designation process of CEs.
- 4.1.5** During their evaluation, all expert centers operate a quality management system, usually based on ISO 9001 or ISO and health care standards (MEES) together. As a result there are regular internal and external audits, patient satisfaction measurement and outcome measurement. For now, the problem is that these quality systems and certificates are not comparable at national level. Therefore, we would need common indicators to measure patients' satisfaction with services obtained.
- 4.1.6** Selection and training of professionals involved in the assessment has not happened yet, and there is no established organization in the evaluation/accreditations body. Health care in adulthood is exceedingly difficult and a main issue even in those successful patient groups where a child centre is organized because rare disease centres for adults are very scarce. Most adults visit paediatricians in an informal way leading to an overload and a transfer of costs to that side. Therefore, the organization of management, treatment and care specifically addressed to adults with rare diseases are especially needed.

## **4.2 Scope and functioning of CEs**

- 4.2.1** This point is irrelevant yet, because the lack of designated CEs.

## **4.3 Multidisciplinarity, healthcare pathways & continuity of care**

- 4.3.1** Until the official and visible designation of our RD centres, the access to possible CEs is occasional and pathways are often informal for the patients. The large University centres are involved in education, research as well as in medical attendance and function in the above fields as centres of expertise. When CEs are designated, we will highly regard multidisciplinarity, research and educational activity, beside the clinical work and also the opportunities of social care.

## **4.4 Access to information**

- 4.4.1** The distribution of suitable information about the available services is essential. It must contain also the available paramedical services in or in cooperation with university hospitals (ex. hospital teachers, physical therapists, social workers, psychologists). The

homepage of NRDC that is under preparation should display supplementary social and educational institutions and the competent civil organizations as well.

- 4.4.2** Medical knowledge of special centres reaches general practitioners through local presentations when the current protocols are also introduced.
- 4.4.3** The rare disease information webpage of NRDC must be updated with information on the development of the National Plan, therapeutic options, organisation of healthcare pathways. The identification and the consequent extra budget of National and Regional CEs should assume the obligation of registration in E-RARE, orpha patients, EU harmonisation (indicators and bio banks etc.), training, appointment of reference centres, research, social services etc.

#### **4.5 Research in CEs – How to integrate research on RDs and provision of care**

- 4.5.1** Linking of basic and translational research to CEs is not yet regulated.
- 4.5.2** Our National Plan includes contribution for the cooperation of future CEs and /or other public health structures, health and research authorities in order to broaden knowledge about different aspects of RDs.

#### **4.6 Good practice guidelines**

- 4.6.1** The national Pharmaceutical and Healthcare Quality and Organization Development Institute (GYEMSZI) is currently developing a national accreditation standard of healthcare institutions, and the revision of previous standards. The project scope neither includes the accreditation of expert centers, nor the development of standards for rare diseases.

#### **4.7 Diagnostic and genetic testing**

- 4.7.1** The medical laboratories that are involved in the diagnosis of rare diseases are mainly laboratories of the four medical universities. There is no formal inventory of the performed analyses, but there are efforts to collect the special rare analysis types (directory of rare analyses). Accreditation according to the international standards (ISO15189, 17025) is possible, some laboratories have already accredited some processes. To link the diagnostic laboratories to the future CEs would not be too complicated as they are already linked to the universities that would be the basis of the CEs. Partnerships with foreign partners are being continuously formed. Unfortunately, there is no reimbursement system for special testing in Hungary.
- 4.7.2** The sending of DNA samples are organized at the local level; typically both the clinical and laboratory geneticist are involved.
- 4.7.3** The diagnostic odyssey of patient suffering from rare diseases might be still unacceptably long. It is important to note, that both the clinical geneticist and the clinical laboratory geneticist postgraduate trainings are available in the country. In the case of clinical genetics, the European recommendations are followed, while in the case of the clinical laboratory geneticist at the moment a specialization of laboratory medicine (or pathology, etc.) is a prerequisite of the training. For multiple malformations, genetic counselling is indeed part of the routine procedure, but for more common genetic testing the low number of clinical geneticists (app. 30-40 in the country) might result in imperfect counselling. Designation of CEs will certainly help in solving this situation.

#### 4.8 Screening policies

- 4.8.1** Newborn screening is well organized for 26 diseases and regulated with a ministerial regulation. It is mandatory and performed in two centres (Budapest and Szeged). No other targeted screening has been implemented lately.

#### 4.9 European and international collaboration – Cross-border healthcare and ERNs (European Reference Networks)

- 4.9.1** The identification and the consequent extra budget of National and Regional CEs should assume the obligation of registration in E-RARE, orpha.net and reference centres of the attended diseases. The optimal conditions of national CEs and the conditions of admission to Orpha.net network should be ensured by accreditation processes and participation in international research. Namely, the condition of participation is the continuous accreditation of laboratories and workstations and participation in international conferences.
- 4.9.2** There are several collaborations, however, encouragement and broadening is needed in the fields of basic, translational and clinical public health research, especially.
- 4.9.3** The travel of diagnostic material has yet to be formally organized in the country. There have been many efforts to set up the necessary information technology system that will be capable to fulfil these needs.

#### 4.10 Sustainability of CEs

- 4.10.1** The funding of CEs of rare diseases should be accentuated in order to cover diagnostics, therapy and care. In the field of diagnostics, beside the costs of examinations and tests, costs of amortization of the equipment should be taken into account when calculating the budget. In every case, CE teams should have a member who is responsible for the operation of registers and biobanks apart from medical doctors, psychologists and physical therapists.
- 4.10.2** The specific budget attached to the National Plan contains financial sources for the designation process of CEs as well.

#### 4.11 Recommendations

- 4.11.1** Mapping of the presently informally functioning CEs, centres of care, genetic counselling and laboratories in Hungary developing an official accreditation process within the possible shortest time.
- 4.11.2** Designations of the CEs and publication on the homepage of NRDC.
- 4.11.3** Support of the development of CEs performing extra tasks by separate financing mechanisms.
- 4.11.4** Control of accredited institutions based on EU compatible standpoints (exact patient registry, coding, multidisciplinary care, patient satisfaction etc.) by an independent supervisory organization. Data of patient registry (of satisfactory quality) would be utilized in the evaluation of efficiency and in the quality assurance in a multilevel control approach where publicity would be realized in a number of ways including patient access.
- 4.11.5** Assurance of joining medical and paramedical services when determining healthcare pathways. Assurance of covering the missing human resources.

- 4.11.6 Accomplishing the connection to European Reference Networks and other EU programs even with application of sanctions.
- 4.11.7 Ensuring cross border access to the specific healthcare by shortening time of the application process when needed.
- 4.11.8 Introduction and spreading the information about the homepage constructed and the structure and function of the forming care system for RD's. Need to utilize the information channels of the National Institute for Health Development and the Hungarian National Public Health and Medical Officer Service as well as different programs, flyers and media appearance
- 4.11.9 Involving civil organizations in every step of the development and implementation of the National Plan

## **5. topic – Orphan drugs and treatments**

**Chairs:** György Pfliegler, Károly Fogarassy

**Date, venue:** 25 October 2013., Hunguest Hotel Griff\*\*\*

### **Council Recommendation (2009/C 151/02)**

*Whereas:*

[...]

*(19) It is of utmost importance to ensure an active contribution of the Member States to the elaboration of some of the common instruments foreseen in the Commission communication on rare diseases: Europe's challenges of 11 November 2008, especially on diagnostics and medical care and European guidelines on population screening. This could be also the case for the assessment reports on the therapeutic added value of orphan medicinal products, which could contribute to accelerating the price negotiation at national level, thereby reducing delays for access to orphan drugs for rare diseases patients.*

*(The Council of the EU) hereby recommends that Member States:*

[...]

#### **V. GATHERING THE EXPERTISE ON RARE DISEASES AT EUROPEAN LEVEL**

*17. Gather national expertise on rare diseases and support the pooling of that expertise with European counterparts in order to support:*

*(e) the sharing Member States' assessment reports on the therapeutic or clinical added value of orphan drugs at Community level where the relevant knowledge and expertise is gathered, in order to minimise delays in access to orphan drugs for rare disease patients.*

**EUCERD Indicators**

INDICATOR	AREA OF COUNCIL REC. 2009/ C151/02	INDICATOR DESCRIPTION	TYPE OF INDICATOR	ANSWERS
15. Number of Orphan Medical Products (OMPs) with a European Union marketing authorisation and available in the country (i.e. priced and reimbursed or directly supplied by the national health system)	5	The actual availability of OMPs in the national market is essential to illustrate patients' access to treatment in their country. Moreover, with patient access to OMPs differing across Member States, the success of cross border healthcare depends on the harmonisation of access to diagnosis and treatment. Therefore, quantifying the drugs that are available in each country, either in ambulatory or in-hospital regimens, is also important to bridge the existing gap between Member States.	Outcomes	38
16. Existence of a governmental system for compassionate use of medicinal products	5	The indicator aims to identify whether a system exists to provide medicines to rare diseases patients prior to approval of new drugs (so-called compassionate use). The existence of such programmes is relevant for the assessment of overall RD	Process	no

**5.1 Support to Orphan Drug (OD) development**

There is no special program in Hungary that would facilitate the development of Orphan drugs, however, patient organisations occasionally urge it (for example, firazyl). It is important to facilitate information exchange in this area. It would be necessary to incite research and young researchers via foundations and tender funds.

**5.2. Access to treatments**

Available orphan medicinal products in Hungary according to financing:

- Classic reimbursement system (Eü100+90): **6 products**
  - Compassionate use by reimbursement system: **12 products**
  - Classic reimbursement system (Eü100) or compassionate use by reimbursement system depending on the indication: **8 products**
  - Institutional use (itemized financing) **2 products**
  - Compassionate use by hospital care: **5 products**
  - Extra financing by hospital care: **1 product**
- Altogether: **34 products**

(Remark: **3 medicines** (2 institutional, 1 compassionate use by hospital care) are not orphan designated products, but indicated for rare diseases. Furthermore **1 medicine**, reimbursed by compassionate use, contains similar agents to an orphan designated product. Consequently altogether: **38 products**)

It is necessary to improve licensing and acceptance by social insurance through acceleration of information exchange. The GYEMSZI-OGYI (the national Pharmaceutical and Healthcare Quality and Organization Development Institute) is taking part in the CAVOMP, and in the MOCA's accession process. The relationship between GYEMSZI-OGYI and EMA is already existing and vital.



The health budget is coordinated by the OEP (National Health Insurance Fund) constantly inviting professionals, patient organizations (such as RIROSZ) also participate in meetings, respectively. Expertise and management centres should spread information on Orphan Drugs and access through patient organizations.

### 5.3. Compassionate use programmes

Regular and multi-level (e-newsletter, conference, etc.) briefing is necessary. Hungary is in accordance with the suggestions made by the EU. If the demand is bigger than the supply, there is no directive on selecting patients, since it would not be ethical. Incidentally, age and supposed life expectancy could be taken into consideration. Incorporating patient organizations into compassionate use programs can be basically achieved through information exchange, doctor-patient clubs, and inviting representatives to professional conferences.

It is worth considering to support medicines and methods that are in the research stage with government funds (health insurance or R&D), similarly to the conditions that apply to drug research protocol and data collection. Pharmaceutical companies could support the necessary diagnostic background investigation.

### 5.4. Off-label use of medicinal products

**Off-label drug prescription and reduced compensation are possible if the patients are using it purportedly.** It is possible in the current Hungarian administration procedure; however, it is strongly bureaucratic. It is expedient to simplify and rationalize the procedure in the future.

Off-label use of drugs requires individual weighing. The patient can buy the product based on the receipt. Accurate documentary, clarity and electronic registry are necessary. The GYEMSZI-OGYI has a wrought procedure. Two matters need to be addressed here: the procedure of “conditional authorization” and the establishment of “temporary therapy protocol”. In case of only few patients, naturally, a “protocol” is unnecessary, individual management is enough. When certain products are not on the market, their ingredients can be licensed from the relating pharmacopoeia, and in the appropriately equipped pharmacy the formulations could be produced as a magistral for a minimum price.

The centres of expertise carry significant power in delivering opinions concerning the evaluation of pros and cons of using off-label products. Supporting the filling out the off-label worksheet could help in the data acquisition regarding the off-label use of authorized drugs. The support of this data acquisition is disease-dependent, therefore it should be done by the professionals of that given area. Also, it is important to participate in international registers!

Information on Orphan drugs - such as the number of patients, diagnosis, formulations, and level of support - that receive social insurance support, and are in off label usage is being handled by the OEP. At the same time, however, given the small number of patients, sharing these information with a third party would raise questions on data security.

### 5.5. Pharmacovigilance

Since off-label use of products are often requested by the patients themselves as an experiment, tighter controls and reporting of adverse side effects is necessary. Data collection is made by the appropriate department of GYEMSZI-OGYI. International best-practice and feedbacks are essential.

### 5.6. Recommendations

**5.6.1.** Putting the National Plan into action is important! Centres of expertise and management should spread information on possible therapies through patient organizations.

**5.6.2.** It is advised to take note of the positive examples in international practices while working on the national acceptance procedure for reimbursement.

**5.6.3.** It would be an immediate help for families if the list of officially prescribable drugs could be updated in a more simple and flexible fashion when a doctor decides on changing the prescription.

## **6. theme – Social Services for Rare Diseases**

**Chairs:** Péter Horváth, Katalin Brunner, Beáta Boncz

**Date, venue:** 25 October 2013., Hunguest Hotel Griff\*\*\*

### **Council Recommendation (2009/C 151/02)**

*I. 1. Establish and implement plans or strategies for rare diseases at the appropriate level or explore appropriate measures for rare diseases in other public health strategies, in order to aim to ensure that patients with rare diseases have access to high-quality care, including diagnostics, treatments, habilitation for those living with the disease and, if possible, effective orphan drugs, and in particular:*

*(a) elaborate and adopt a plan or strategy as soon as possible, preferably by the end of 2013 at the latest, aimed at guiding and structuring relevant actions in the field of rare diseases within the framework of their health and social systems;*

*III. 7. Identify needs and priorities for basic, clinical, translational and social research in the field of rare diseases and modes of fostering them, and promote interdisciplinary cooperative approaches to be complementarily addressed through national and Community programmes.*

**1. 17. Gather national expertise on rare diseases and support the pooling of that expertise with European counterparts in order to support:**

*(a) the sharing of best practices on diagnostic tools and medical care as well as education and social care in the field of rare diseases;*

### **EUCERD Indicators**

<b>INDICATOR</b>	<b>AREA OF COUNCIL REC. 2009/ C151/02</b>	<b>INDICATOR DESCRIPTION</b>	<b>TYPE OF INDICATOR</b>	<b>ANSWERS</b>
17. Existence of programmes to support in their daily life RD patients integration	6	Rare Diseases often lead to disability and a need for continuous care. Specialised Social Services are instrumental in providing patients with a full, rewarding life. Their existence and number demonstrate the political commitment of Member States to this mission. Examples of social services to integrate patients in their daily life and support their psychological and educational development are:  a) educational support for patients, relatives and caregivers;  b) individual support at school, for both pupils with rare diseases and teachers, including disease-specific good practices;  c) activities aimed to foster higher education for people with rare diseases;	Process	In progress - people living with RD can access general programmes for persons with a disability There exist specific actions and programmes for them. a, a few educational support exists b, individual support at school in some cases c, there are few activities aimed to

		d) supporting mechanisms to participate in work life for people with disabilities.	foster higher education d, exists some supporting mechanisms to participate in work life, but with a descending rate
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## 6.1. Social resources for people with disabilities

**6.1.1** Diagnosing disabilities in case of rare diseases. How “visible” are rare diseases in our social care system.

The regulation of the rights of people with disability in Hungary does not cover specifically those who suffer from rare diseases, the term “rare disease” is not defined in the legislation frame. Patients with rare diseases receive social benefits based on certain signs of their illnesses, or on certain disabilities. The special social needs of those with rare diseases - special meals, special habilitation procedures, special support, etc. - are needs that are not “visible” to the social care system, hence, there are difficulties in providing them.

It is also important to improve the evaluation process of disabilities so, that among others, the functional quality of the disease is also taken into consideration, for example, chronic degenerative processes and behavioural changes.

**6.1.2** Information sources for rare disease patients which enables them to access existing social benefits.

Each year the relevant Ministry makes a summary of already existing social benefits, which information is handed down to the patients via the professionals. The knowledge of these professionals does not cover the wide range of variety of rare disease patients, therefore social information reaches the patients apropos of their disabilities. Practice proves that often the professional is unable to match the possibilities of social care with the needs of rare disease patients.

Another source of information are the patient organizations or the associations of said organizations, where on one hand, patients’ experiences are conveyed to people who suffer from a similar disease; on the other hand, through their own data collection, organizations may also help in informing patients.

The internet is often used as a source of information. However, it must be pointed out that it is also risky, despite its wide scope of information, because if it is not supervised by an official agency, the information found on the sites could be misleading. Currently there is no easily accessible medium which would provide trustworthy and accurate information regarding the social care system and legislation to patients with disabilities.

**6.1.3** Specialization of social services to finance rare diseases

Social services are not specialized to support people who suffer from rare diseases. Often these benefits can only be obtained through diligent work, and are based on symptoms or disability. It should be seen as a positive first step that disability benefits have been expanded to cover those rare disease patients whose condition is regarded as serious or moderate. In order to provide and finance appropriate services to match the needs of rare disease patients, it is necessary to do an

overall supervision and definition of the recipients, and the introduction of guidelines and quality assurance systems.

Overall regulation of the rights of people with disability should cover those cases as well, when the level of disability of the person with a rare disease does not manifest itself as an actual disability, but due to their chronic condition, social benefits are indispensable.

The assurance of social services make the definition of rare diseases inevitable, since based on that definition will we be able to fit them into our current social care system. Patients should have clarity on the legislation of complex rehabilitation procedures; only this way will the patients' rights to equality, independent lifestyle, and active participation in social events be assured. It is best to give priority to the specific needs of rare disease patients, which they need even if they are not being regarded as disabled, or their condition is permanent, but they would need social care to sustain their state. When making these decisions, it is essential to note that rare disease patients are equal members of society, therefore, it is obligatory to create the criterion which ensure their participation in social activities despite the fact that this entails applying for social benefits.

## 6.2. Specialised social services for rare diseases

Specialised services developed in some European countries, such as:

- Respite care services
- Therapeutic recreation programs
- Adapted houses

have become practice, and they facilitate rare disease patients and their families getting access to a normal life.

There are no specialized services established yet for Hungarian rare disease patients, but several services are available for patients among normal service options. The accepted National Plan includes a development program which makes a larger scale of special services available. In the long-term plans, the following particularly important social goals have been included:

- after diagnosis, rare disease patients and their families should be educated on how to deal with the disease and its consequences in special Rare Disease Centres of Expertise;
- work out in detail programs to improve the quality of life for rare disease patients - help them connect with others with similar conditions, reduce their sense of exclusion of communities, and help them reconnect with society;
- draw up complex individual development protocols with constant counselling, keeping in check with the patients, family counselling, event management with a life-long assurance working closely together with life sciences centres;
- development of special social service appliances in order to improve rehabilitation services - organizing therapeutic recreation programs, programs to help the patients integrate into normal everyday activities, helplines;
- it is necessary to create a methodology of care services which includes programs for rare disease patients to their existing and their improvable abilities, respectively - regularly evaluating ability improvement during the rehabilitation process, and prescribing adjustments in the rehabilitation program, if it is needed;
- assurance of access to special appliances required by disease;
- regular updates on the service provider organizations and on their data gathering of rehabilitation services, to keep the patients and their families informed;

- establishment of a National Centre of Information, Rehabilitation, Development and Service for Rare Diseases.

The main objective of the centre would be to diagnose early the injured or missing functions with the appropriate team of experts, so healing and rehabilitation may start as early as possible.

This multidisciplinary service centre included in the National Plan would fill a large hole in the system. It will operate based on a Scandinavian example (the Norwegian Frambu Centre), as a centre for a variety of developmental methods and possibilities. The goal of the institute is to help children and adults with rare diseases and their families in a coordinated manner, working in teams and achieving complex habilitation, preventive, and re-education goals, with the help of civil organizations. They are planning not only early treatment, but development regardless of age, which would mean a modern solution not only to Hungarian, but to all rare disease patients living in the Carpathian Basin.

The Institute would directly provide services to those rare disease patients, whose symptoms often go unnoticed, and left without appropriate treatment. Activities would include close communication and counselling with the concerned children and their families.

### **6.3. Policies to integrate people living with rare diseases into daily life**

#### **6.3.1. Helping the education of rare disease patients with social appliances.**

People who suffer from rare diseases, given their disadvantaged condition need to go under appropriate development treatment, and in accordance with the patients' age receive early care in education (nursery school, primary school etc.), and the parents should cooperate with the supporting expert and recreational committee in choosing a fitting style of education.

#### **6.3.2. Developing employment for rare disease patients.**

If the condition of the patient justifies it, he or she is entitled to work in an integrated, or protected environment, similarly to those who have disabilities. If integrated work is not an option, the patient's right to work should be assured by operating special workplaces.

Establishment of a National Institute of Information, Rehabilitation, Development and Service for Rare Diseases, and helpline service.

Establishing a centre would significantly stimulate the completion of the abovementioned tasks. From public health's point of view, most problems concerning rare diseases are caused by the lack of information, out of which stems late diagnosis, and possibly inappropriate treatment. Besides public health, the education, employment and social systems could do most for improving the life quality of these families. Rare disease patients with chronic diseases that are considered life-threatening and often lead to disability needing daily care and supervision. To compensate for the disadvantages caused by the diseases, the efficiency of social solidarity and governmental support needs to be improved.

If public health is capable to assure the earliest possible diagnosis, early development could strengthen the basis for prevention, development, rehabilitation, and social integration.

### **6.4. International–supranational dimension**

In the case of those social services, which have already become practice in other European countries, for example, temporary care services, therapeutic recreation programs, adapted accommodation (Resource Centres), special services for rare disease patients, helpline, etc., deliverance of foreign expertise and guidelines, presentations, and establishment of personal work relations would lessen the time of working out and introducing said social services in Hungary.

Professional meetings would be great opportunities to exchange and spread existing methods, and

due to personal relations, presenting the results would become easier, and make attempts to development more attractive.

## **6.5. Recommendations**

**6.5.1.** For rare disease patients it would be a real breakthrough if the legal background affecting social services they are entitled to, and an appropriate expansion were made in the disability law, so that patients with rare diseases could receive treatment based on their condition, including special services and appliances.

**6.5.2.** Opportunity for HUFERDIS/RIROSZ to take part in the activities of the National Disability Council, maybe as an observer at first; expanding the National Disability Program with action plans in regard of rare disease patients.

**6.5.3.** Realization of the social goals included in the National Plan as quick as possible is not only exceedingly important for rare disease patients, but would also be advantageous in respect of using EU resources.

**6.5.4.** We are confident that the needs of people with rare diseases are complex, and concern several branches and policy. Apart from the public health and social branches, there is work to do in public and vocational training, in higher education, in employment, and also in the field of human rights. We find it important to create coordination between the different branches, and while realizing the National Plan, establish co-operation between the various portfolios.

**6.5.5.** We suggest expanding the knowledge of teachers and social experts via trainings, including the patients themselves, as to give hands on experience. Such trainings would help solve the problems arising from rare diseases (for example, in social organizations and institutes, in education, in help centres, in patients' rights advocacy and in social services).

**6.5.6.** We propose, as a part of the fund creation, the establishment of a social incentive system with valid tax allowances, or other economic advantages to facilitate the donation activity of sponsors and private individuals.

**6.5.7.** Apart from medical research, it is important to fund social sciences studies, educational and social research concerning rare diseases. The introduction of new diagnostic and therapeutic methods should be considered a priority, which is stimulated by the establishment of a research-oriented application system.

**6.5.8.** We recommend establishing the National Habilitation, Information, Development and Service Institution for Rare Diseases and Help Line in Cooperation with the patient associations according to our National Plan.

## IV. Conclusions of the final report

### 1. The role of the EUROPLAN recommendations and indicators in serving as support to the elaboration of our national strategy:

The well-structured recommendations (together with the supporting documents) have served as a wonderful basis both for the professional organisation of the conference and for its high level execution, for the final report and for the steps starting the elaboration of the National Strategy. Indicators served as objective frame for evaluating the current situation as well as for monitoring the future development. In several cases, it turned out that adequate measures need rearranging currently available data and elaboration of the possibility of a new statistical separation.

General principles and consequences of the recommendations served as valuable guide and proved to be simply followed.

### 2. Identification of specific gaps, challenges and needs across all Themes.

Summarizing, we can report that all the participating experts, politicians are equally motivated to implement the National Plan (NP). The signing of NP by the Minister of Human Resources meant a great stimulation for everybody. The NP covers widely the needs of RD patients, extending all important areas and in harmony with the EU recommendations. All stakeholder groups supported the implementation of NP, including the allocation of a dedicated budget. The implementation of NP is jeopardized by some uncertainty caused by the prospective MP election; therefore the enhancement of national and international advocacy work is continuously necessary.

#### Major challenges:

- Finishing the process of inter-ministerial agreement and public consultation as soon as possible, to start the implementation of the NP.
- Rapid designation of the CEs including the organization of visible patient pathways.
- NRDC and Health secretary closer cooperation and exact description of the division of tasks and labour, to ensure the necessary human resources and tools.
- Establishing RD Help Line and Information Service with the necessary resources.
- Involving patient representatives as experts in the implementation of the National Plan.

## V. Annexes

### 1. Glossary

<b>Abbreviation</b>	<b>Meaning</b>
ÁNTSZ	National Public Health and Medical Officer Service
BURQOL-RD	Social Economic Burden and Health-Related Quality of Life in Patients with Rare Diseases in Europe
CAVOMP	Clinical Added Value of Orphan Medicinal Products
CE	Centre of Expertise
DE	University of Debrecen
EMA	European Medicine Agency
EMMI	Ministry of Human Resources
E-Rare	European Rare Diseases Research Network
ERN	European Reference Network
ETT	Medical Research Council
GYEMSZI	National Institute of Quality and Organizational Development in Healthcare and Medicines
OGYI	National Institute of Pharmacy
HBCS	Homogeneous patient groups
HRG	Hydrotherapeutic Rehabilitation Gymnastics
ICD	International Classification of Diseases
IRDiRC	International Rare Diseases Research Consortium
MOCA	Mechanism of Coordinated Access to orphan medicinal products
NP	National Plan
NRDC	National Rare Disease Centre
OC	Organizing Committee
OD	Orphan Drug
OEFI	National Health Institute
OEP	National Health Insurance Fund
OSZMK	National Centre for Healthcare Audit and Inspection
OTKA	National Scientific Research Programme
PE	University of Pécs
QALY	Quality Adjusted Life Year
RD	Rare Disease
RIROSZ	Hungarian Federation of People with Rare and Congenital Diseases (HUFERDIS)
TDK	Scientific Students Club
TSMT	Planned sensomotor training
VRONY	National Registry of Congenital Diseases



## 2. Final Programme of the National Conference

### 8:45-9:00 Registration

**9:00-9:30 Press Conference with Opening Ceremony** *Enikő Széll (EMMI), György Kosztolányi (NT coordinator), János Sándor (EUCERD), Domenica Taruscio (Europlan), Gábor Pogány (Rirosz)* – Emerald room

### 9:30-11:00 Plenary presentations

Session Chair: *György Kosztolányi, János Sándor* – Emerald room  
EU background *János Sándor*  
Hungarian National Plan - present status *György Kosztolányi*

### 11:15-12:45 Parallel I-III Workshops

**I. Workshop:** Methodology, Governance and Monitoring of the National Plan *József Vitrai, Márta Szegedi, Helga Süli-Vargha* – Emerald room  
**II. Workshop:** Definition, codification and inventorying of RD (Information and training) *Imre Boncz, László Szőnyi, János Sándor* – Amethyst room  
**III. Workshop:** Research on RD *Éva Oláh, Gergely Bujdosó* – Crystal room

### 12:45-13:45 Lunch

### 13:45-14:45 I-III Plenary Reports of Worksops I-III.

Session Chair: *Katalin Komlósi, Gábor Pogány* – Emerald room

### 15:00-16:30 Parallel IV.-VI Workshops

**IV. Workshop:** Care for RDs - Centres of Expertise and European Reference Networks for Rare Diseases *Judit Mária Molnár, István Balogh, Judit Becskeházi-Tarr* – Emerald room  
**V. Workshop:** Orphan drugs and treatments *György Pfliegler, Károly Fogarassy* – Amethyst room  
**VI. Workshop:** Social Services for Rare Diseases *Péter Horváth, Katalin Brunner, Beáta Boncz* – Crystal room

### 16:30-17:00 Coffee break

### 17:00-18:00 IV-VI. Plenary Reports of Worksops IV-VI., Closing

Session Chair: *Pál Vittay, Judit Váradiné Csapó, János Sándor* – Emerald room

### 3. List of Participants

Name		Institute	Role	Stakeholder group
Ataldia	Antonio	EU	Advisor	EU
Baksa	Sarolta	SI-KOMMENT Kft.	participant	industry
Balogh	István	Debreceni Egyetem Laboratóriumi Medicina Intézet	Speaker	clinician/GP
Bánlaki	Szilvia	Gyógyító József Alapítvány	participant	patient representative
Bárkányi	Erika	Országos Egészségbiztosítási Pénztár	participant	Insurer
Becskeházi- Tar	András, dr	RIROSZ	participant	patient representative
Becskeházi- Tar	Judit	SGS SSC, Lead auditor, quality professional	Speaker	expert
Bencsikné Mayer	Mónika	Cri Du Chat Baráti Társaság	participant	patient representative
Bodnár	Ágnes	MED 13 Kft.	participant	industry
Bodnár	Andrea	ACTELION	participant	industry
Bokk	Ágota	Genzyme -a sanofi vállalata Sanofi-Aventis Zrt.	participant	industry
Boncz	Imre	Pécsi Tudományegyetem Egészségtudományi Kar Egészségbiztosítási Intézet	Speaker	expert
Boncz	Beáta	RIROSZ, MWSZT	Speaker	patient representative
Boncz	Balázs	MWSZT	participant	patient representative
Brunner	Katalin	RIROSZ	Speaker	patient representative
Bujdosó	Gergely	Misko Alapítvány	Speaker	patient representative
Csányi	Péter	EMMI Szociális és Családügyért Felelős Államtitkárság	participant	government
Dan	Dorica	EURORDIS	Advisor	EU
Domenica	Taruscio	EUCERD	Advisor	EU
Fogarassy	Károly	Fabry-betegekért Alapítvány	Speaker	patient representative
Földvári	Anett	DE OEC Népegészségügyi Kar Megelőző Orvostani Intézet Biostatistikai és Epidemiológiai Tanszék	participant	expert
Fülöp	Rita	-	participant	other
Garami	Márta	OKI 1097 Budapest Albert F. út 2-6	participant	expert

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		Bárczi Gusztáv Óvoda, Általános Iskola és Készségfejlesztő Speciális Szakiskola	participant	expert
Gyírkis	Ágnes			
Herczegfalvi	Ágnes	SE. II. sz. Gyermekklinika	participant	clinician/GP
Horváth	Péter	ELTE BGGYK	Speaker	expert
Huszárikné V.	Klára	MWSZT	participant	patient representative
Juhász	Orsolya	Reina Kft.	participant	industry
Karcagi	Veronika	OKI-MGDO	participant	expert
Kertész	Beáta	Swedish Orphan Biovitrum s.r.o Magyarországi Fióktelepe	participant	industry
Kaselya	Anita		participant	other
Kiss	Orsika	Narkolepszia klub	participant	patient representative
Komlósi	Katalin	Orvosi Genetikai Intézet, Pécsi Tudományegyetem	Speaker	clinician/GP
Kosztolányi	György	OEFI RBK	Speaker	expert
Kovács	Nóra	DE OEC-NK Megelőző Orvostani Intézet	participant	clinician/GP
Kovács	Ildikó	NVMIL Kft	participant	industry
Losonczi	Erika	Genzyme, Sanofi	participant	industry
Lukács	Andrea	Misko Alapítvány	participant	patient representative
Maros- Szabó	Zsuzsanna	Astridbio Kft.	participant	industry
Molnár	Mária Judit	SE Genomikai Medicina és Ritka Betegségek Intézete	Speaker	expert
Mudri	Barnabás	GENZIME	participant	industry
Nagy	Éva	Élet Hősei Alapítvány	participant	patient representative
Nagy	Zsuzsanna	Sclerosis Tuberosa Baráti Társaság	participant	patient representative
Nagy	István	Magyarországi Angio- Ödémás Betegek Egyesülete	participant	patient representative
Nagyné Kijácz	Edina	Nemzetközi Pető Intézet	participant	expert
Oláh	Éva	DE OEC Gyermekgyógyászati Intézet Klinikai Genetikai Központ	Speaker	clinician/GP
Pataki	Anita	MWSZT	participant	patient representative
Pfliegler	György	DE OEC B.I. Ritka Beteg Központ	Speaker	szakmák
Pincz	Nikoletta	Élet Hősei Alapítvány	participant	patient representative
Pogány	Gábor	RIROSZ, NBF	Speaker	patient representative
Pogány	Krisztina	MWSZT	participant	patient representative

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Pozsonyi	Zsuzsanna	SynCon International - klinikai vizsgálatok szervezése	participant	Industry
Román	Tamásné Zsuzsa	MPS Társaság	participant	patient representative
Sándor	János	DE EOC Népegészségügyi Kar Megelőző Orvostani Intézet Biostatistikai és Epidemiológiai Tanszék	speaker	Expert
Somogyiné Toronyi	Brigitta	Élet Hősei Alapítvány	participant	patient representative
Süli-Vargha	Helga	RIROSZ	speaker	patient representative
Szegedi	Márta	OEP	participant	Insurer
Széll	Enikő	EMMI	speaker	Government
Szentirmai	Ágnes	Magyar Katolikus Rádió Zrt.	participant	Media
Szili	Danijela	MRSZA	participant	patient representative
Szőnyi	László	SE I. sz. Gyermeklinka	speaker	Expert
Törtei- Takács	Krisztina	G.rádió	participant	Media
Tukacs	Edit	Astridbio Kft.	participant	Industry
Trautmann	Eszter	ELTE BGGYK	participant	Other
Váradiné Csapó Judit	Judit	RIROsz, Angyalszárnyak MIT	speaker	patient representative
Várady	Hortenzia	GENZIME	participant	Industry
Varga	Gábor	Magyar Hemofília Egyesület	participant	patient representative
Varga	Orsolya	DE OEC NK Megelőző Orvostani Intézet	participant	clinician/GP
Várhelyi	Ferenc	MWSzT,Q16	participant	patient representative
Vékás	Anna	VM.KOMM	participant	Media
Vetró	Piroska	Magyar Porphyria Egyesület	participant	patient representative
Vitrai	József	OEFI	Speaker	Government
Vittay	Pál	GYEMSZI	Speaker	Government