Hungarian

EUROPLAN NATIONAL CONFERENCE

in the framework of the EU Joint Action RD-ACTION

Budapest, 20/10/2017

FINAL REPORT

Co-funded by
the Health Programme
of the European Union

FOREWORD

The EUROPLAN national conferences or workshops are organised in many European countries as part of a coordinated and joint European effort to foster the development of comprehensive National Plans or Strategies for Rare Diseases addressing the unmet needs of patients living with a rare disease in Europe.

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

The EUROPLAN national conferences/ workshops are jointly organised in each country by a National Alliance of rare disease patients' organisations and EURORDIS–Rare Diseases Europe. **Rare Disease National Alliances and Patient Organisations have a crucial role to shape the national policies for rare diseases.**

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

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

Since 2008, National Alliances and EURORDIS have been involved in promoting the adoption and implementation of National Plans and Strategies for rare diseases. Altogether, 30 EUROPLAN national conferences took place in the framework of the first EUROPLAN project (2008-2011) and the EU Joint Action of the European Committee of Experts on Rare Diseases – EUCERD - (2012-2015).

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

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

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

GENERAL INFORMATION

<table>
<tr><td>Country</td><td>Hungary</td></tr>
<tr><td>National Alliance (Organiser)</td><td>Hungarian Federation of People with Rare and Congenital Diseases (HUFERDIS) Rare Diseases Hungary</td></tr>
<tr><td>Date & place of the national workshop/conference</td><td>20/10/2017, 1134 Budapest, Tüzér u. 33-35.</td></tr>
<tr><td>Website</td><td>http://europlan.rirosz.hu</td></tr>
<tr><td>Members of the Steering Committee</td><td>Zsuzsanna Bojtor
Eszter Fogarassy
Anita Pataki
Gábor Pogány</td></tr>
<tr><td>List of Themes addressed</td><td><table>
<tr><td>1, Section</td><td>Status of our National Plan, European Reference Networks for Rare Diseases</td></tr>
<tr><td>2, Section</td><td>Implementation of Hungarian National Plan Centres of Expertise and Healthcare Pathways;</td></tr>
<tr><td>3, Section</td><td>Integration of RDs into Social and Family Policies, Early Intervention</td></tr>
<tr><td>4, Section</td><td>RD Communication/Information and Education/Training</td></tr>
<tr><td>5, Section</td><td>Research on RD</td></tr>
<tr><td>6, Section</td><td>Definition, codification and inventorying of RD</td></tr>
<tr><td>7, Section</td><td>Orphan Medicinal Products</td></tr>
</table></td></tr>
<tr><td>Section Chairs (and Rapporteurs, where applicable)</td><td>György Pfliegler, Kinga Hadzsiev, Gábor Pogány, Judit Mária Molnár, Péter Klivényi, Judit Becskeházi-Tar, Barbara Czeizel, Pál Szekeres, Éda Kedves, Zsuzsa Csató, Ferenc Oberfrank, Judith Zima, Mária Ábele, Ildikó Horváth, István Balogh, Judit Csapó, János Sándor, Imre Boncz, Helga Vargha Süli, György Németh, Melinda Sobor, Károly Fogarassy</td></tr>
<tr><td>Annexes :</td><td>I. Programme in English
II. List of Participants (by stakeholders’ categories)</td></tr>
</table>

FINAL REPORT

I. Introduction

This was our fifth EUROPLAN National Conference (the third in cooperation with EURORDIS). The Hungarian National Plan and Strategy for Rare Diseases reached the half time of the implementation period (within the period of 2014 to 2020). However, the approval process of the Plan has temporarily stopped because of the reorganization of the health authorities and background institutions, therefore dedicated and associated funding is still missing, except for some activities.

On the other hand, many steps of the RD National Plan was implemented (although often spontaneously, without central coordination). Therefore, we wanted to cast an account, to discuss the next possible steps, to help the life of RD patients, their families and experts. We tried the impossible, to review this broad, diversified and interesting area to decrease the marginalization of the families, and the number of patients spending years of uncertainty before their conditions are correctly diagnosed and treated!

We identified the next themes as key priorities to be discussed (besides the main topic – the implementation of our plan):

- European Reference Networks for Rare Diseases;
- Centres of Expertise and healthcare pathways;
- Integration of RDs into Social Policies;

Besides these, we also discussed the important topics, like research, registries, e-health, codification, guidelines, pricing, and giving information, etc. We always presented the actions that were made at European Union level, including the EUCERD and EUROPLAN indicators.

At the beginning of the day, we organized a **Press Conference** as well.

II. Themes

THEME 1: Status of our National Plan and the European Reference Networks for Rare Diseases

The implementation of the Hungarian National Plan, together with the organic participation of Hungarian Health Care Providers (HCPs) in the European Reference Networks (ERNs) are key priorities for the RD patients and families.

Several points of the Hungarian RD National Plan were realized (in accordance with the „Healthy Hungary 2014-2020" health sectoral strategy):

- The government decree 141/2000. (VIII. 9.) on rules of qualification and revision of severe disability and payment of disability assistance was modified in favour of patients whose condition is medium severe or more severe due to chromosome disorder. https://net.jogtar.hu/jr/gen/hjegy_doc.cgi?docid=a0000141.kor
- Decree 21/2014. (III. 20.) of the Ministry of Human Capacities on the Hungarian Congenital Abnormality Registry (HCAR) was altered for life long data collection and for the application of Orpha Codes. https://net.jogtar.hu/jr/gen/hjegy_doc.cgi?docid=a1400021.emm

- The electronic version of Hungarian Congenital Abnormality Registry (e-HCAR) was established by 2015, as a part of the National Professional Information System (OSZIR). https://www.antsz.hu/felso_menu/temaink/vrony_rbk/evrony_bemutatasa.html
- The National Contact Point was created according to the Directive 2011/24/EU on patients' rights in cross-border healthcare. http://www.patientsrights.hu/
- The Hungarian Rare Disease Centres of Expertise were officially approved in 2015.
- Thus, several health care provider were able to apply for membership in the forming European Reference Networks in 2016.
- HUFERDIS established the RD specific "Lifebelt" Information Centre and Help Line by 2015. http://mentoov.rirosz.hu
- After the reorganization of the Hungarian Health Care System, rare diseases are clearly expressed in the Organizational and Operational Rules of the Ministry of Human Capacities, together with the responsible people and their scope of duties. http://www.kormany.hu/download/b/7e/21000/SZMSZ%20egys%C3%A9ges%20szerkezetben.pdf
- The EFOP-1.9.5-VEKOP-16-2016-00001 programme started for the Intersectoral development of early intervention. https://www.onyf.hu/hu/hivatal/projektek/2233-efop-1-9-5-vekop-16-2016-00001.html
- The first Hungarian RD Dedicated Research Programme – RitBetKut (GINOP-2.3.2-15-2016-00039) - has started with a budget of 3.87 million €, for the research of pathogenesis of rare diseases, including the development of novel diagnostic and therapy procedures. https://www.u-szeged.hu/news-and-events/2017/investigating-rare?
- The Electronic Healthcare Service (EESZT) successfully launched after several years of development. All processes of the healthcare system became digital. The structure applies Orpha Codes in the e-profile of patients increasing the visibility of rare diseases. https://bbj.hu/economy/hungary-due-to-get-electronic-healthcare-system-this-year_128810
- The compassionate use of orphan drugs became possible in Hungary as well by the modification of Act XCV (2005). The competent authority (OGYÉI - National Institute of Pharmacy and Nutrition) gives the individual authorization. https://www.ogyei.gov.hu/engedelyezes_elotti_gyogyszeralkalmazas
- New in-patient department was opened in the Institute of Genomic Medicine and Rare Disorders of Semmelweis University for rare disease patients. http://semmelweis.hu/genomikai-medicina/fekvobeteg-osztaly
- Hungarian representatives contributed fundamentally to the formation of the NGO Committee for Rare Diseases in the UN, and for the development of UN Agenda 2030 for Sustainable Development, including the 17 Sustainable Development Goals (SDGs). Eight SDGs are relevant for the rare disease area as well.

Alongside these undoubtedly positive and long-awaited measures, some negative "systemic" aspects are evident, despite of the tenacious efforts of rare disease patients and their organizations, together with several experts:

- These fine measures fulfilled many times without the participants hearing about each other.
- They often formed spontaneously, without central coordination.
- The main reason is that the approval process for the National Plan has temporarily stopped due to the reorganisation of Hungarian Health authorities.

- There is only an advisory expert committee and not a steering committee to help the implementation of National Plan. Even this is partially functioning and does not always include all relevant stakeholders.
- Therefore, the participation of Hungarian Health Care Providers in European Reference Networks is incomplete and their geographical distribution is heterogeneous.

The participation of Hungarian Health Care Providers in European Reference Networks.

At present there are 10 HCPs participating as members of 12 ERNs from Hungary. That means, the other half of ERNs have no Hungarian participation (indicating by red letters in the table below) although we have good HCPs on several areas of them. One Department of the University of Pécs was able to join to the most ERNs, while the most HCPs were able to join from the Semmelweis University. We need to help and urge the participation of more Hungarian HCPs in the future. We also need to increase the number of three present Hungarian representatives in the European patient advocacy groups (ePAGs).

No.	Reference Network		Budapest	Debrecen	Pécs	Szeged
1	ERN RITA	Immunodeficiency, autoinflammatory and autoimmune diseases		Dermatology Clinic, Medical Center of the Univ. of Debrecen		
2	ERN BOND	Bone diseases				
3	ERN EURACAN	Adult solid cancers	Nat. Inst, of Oncology			
4	ERN GUARD HEART	Diseases of the heart				
5	ERN ReCONNET	Connective tissue and musculoskeletal diseases				
6	ERN ITHACA	Congenital malformations and rare intellectual disability			Dept. of Medical Genetics, Medical Center of the Univ. of Pécs	
7	Endo-ERN	Endocrine conditions	II. Dept. of Internal Med. Semmelweis Univ.			
8	ERN EYE	Eye diseases				
9	ERN TRANSPLANT-CHILD	Paediatric transplantation				
10	ERN Euro BloodNet	Haematological diseases				
11	ERN CRANIO	Craniofacial anomalies and ear, nose and throat (ENT) disorders			Dept. of Medical Genetics, Medical Center of the Univ. of Pécs	
12	ERN RARE-LIVER	Liver diseases				
13	MetabERN	Hereditary metabolic diseases		Medical Center of the Univ. of Debrecen		
14	VASCERN	Multisystemic vascular diseases	Semmelweis Univ. Heart and Vascular Center			

15	ERN-RND	Neurological diseases	Inst. of Genomic Medicine and Rare Disorders of Semmelweis Univ.		Dept. of Medical Genetics, Medical Center of the Univ. of Pécs	
16	ERN EURO-NMD	Neuromuscular diseases	Inst. of Genomic Medicine and Rare Disorders; II. Dept. of Pediatrics, Semmelweis Univ.		Dept. of Medical Genetics, Medical Center of the Univ. of Pécs	
17	ERN LUNG	Respiratory diseases				
18	ERKNet	Kidney diseases				
19	ERN Skin	Skin diseases	Dept. of Dermatology, Venereology and Dermatooncology, Semmelweis Univ.	Dermatology Clinic, Medical Center of the Univ. of Debrecen		Albert Szent.Györgyi Health Centre, Univ. of Szeged
20	ERN eUROGEN	Urogenital diseases and conditions				
21	ERN EpiCARE	Epilepsy				
22	ERNICA	Congenital malformations and diseases				
23	ERN GENTURIS	European Reference Network for rare genetic tumour risk syndromes			Dept. of Medical Genetics, Medical Center of the Univ. of Pécs	
24	ERN PaedCan	Paediatric oncology	II. Dept. of Pediatrics, Semmelweis Univ.			

To follow the development and the changes in time, we stated both the Europlan Indicators (comparing to 2010) and the EUCERD Indicators (comparing to 2013) for each theme.

Methodology and Governance of a National Plan - EUROPLAN indicators				
Actions	**Indicators**	**Type of indicators**	**Answers 2010**	**Answers 2017**
Development of regulations/laws	Existence of regulations/ laws that support the creation and development of a RD plan	Process	Not existing, not clearly stated	Existing, clearly stated, partly implemented and enforced (approval process has temporarily stopped)
	National/regional (percentage of regions)	Process	Not relevant	Not relevant
Establishment of coordination mechanisms	Existence of coordination mechanisms	Process	An expert committee exists but without real tether for coordination and preparing National Plan	Existing, clearly stated, partly implemented and enforced (The expert committee still have no tether for coordination and implementation of the NP)

	Existence of an expert advisory committee	Process	Exists but partly functioning	Exists but partly functioning
Establishment of an external evaluation of Plan/Strategy procedure	Existence of an external evaluation body/procedure	Process	No	No
Degree of comprehensiveness	The number of priority areas included	Process	Not relevant	9
Establishing of a budget for developing the plan/ Strategy	Budget of plan/ strategy	Process	No	No dedicated budget

Methodology and Governance of a National Plan - EUCERD indicators				
Indicator	**Indicator description**	**Type of Indicator**	**Answers 2013**	**Answers 2017**
1. Existence of Regulations/Laws, or equivalent official national decisions that support the establishment and development of a Rare Diseases (RD) plan	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	Existing, but approval process has temporarily stopped
2. Existence of a RD advisory committee	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	YES, exists but partly functioning without tether
3. Permanent and official patients' representation in plan development, monitoring and assessment	Patients are officially represented at all stages of plan development and governance, including its monitoring and evaluation.	Process	YES, but only as observers	YES, many times as initiator
18. Existence of a policy/decision to ensure long-term funding and/or sustainability of the measures in the RD plan/strategy	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	In progress /in development
19. Amount of public funds allocated to the RD plan/strategy	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	There is no dedicated budget, only incorporated into the general budget.

THEME 2: Implementation of Hungarian National Plan and the Centres of Expertise and healthcare pathways

In Hungary there is an official policy for the designation of Centres of Expertise for rare disease, which has been fully implemented by 60/2003 decree of the Minister of Family Affairs, Health and Social Services regulate the CE designation. Based on this, four big universities were approved in 2015 for four years as the Hungarian Centres of Expertise.

There are criteria, which CEs need to fulfil in order to receive designation in harmony with the EUCERD Recommendations (24/10/2011). The institution must provide diagnostic and/or therapeutic services for rare disease patients, and cooperate with other institutions to participate in rare disease care, contribute to the pathways organisation, provide multidisciplinary care, implement research and training projects and maintain registries for patients. During their evaluation, all expert centres 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.

The HCPs who become members of ERNs needed to synchronise their evaluation system with the common criteria of ERNs which is a nice development.

Centres of Expertise ensure a holistic approach to care but they do have trouble. 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.

There is inequalities in the geographical distribution of the Hungarian CEs and Health Care Providers taking part in ERNs. Unfortunately, the lack of them is characteristic in the western part of the country!

Even with the designated centres and with the help of “Lifebelt” Information Centre of HUFERDIS, the access to possible CEs is occasional and pathways are often invisible and informal for the patients. There is no clearly defined map of expertise within the centres of expertise.

In the near future, the expectations are (especially from the patients and their families):

- Cleary defined referrals for centres.
- Decrease the delay for specific diagnosis and treatment.
- Increase the number of clinical trials in rare disease.

To fulfil these expectations, the next measures are necessary:

- New centres of expertise have to be opened, especially in the western part of the country.
- Detailed map of expertise has to be made in order to decrease the diagnostic delay.
- For these, sustainable support of the work of “Lifebelt” Information Centre and Help Line is necessary, together with the better cooperation and information flow with the centres.

Care for RDs - Centres of Expertise and European Reference Networks for Rare Diseases - EUROPLAN indicators				
Actions	**Indicators**	**Type of indicators**	**Answers 2010**	**Answers 2017**
Improve the quality of healthcare by defining appropriate centres with experience on RD	Existence of a policy for establishing centres of expertise at the national/regional level	Process	Not existing, not clearly stated	Existing, clearly stated, partly implemented

as well as pathways that reduce the diagnosis delay and facilitate the best both cares and treatments to patients	Number of centres of expertise adhering to the policy defined in the country	Outcomes	0-Officially, approx. 8-informally	Four big university centres, with several affiliated ones.
	Groups of rare diseases followed up in centres of expertise	Outcomes	Covering all or most of rare diseases	Covering all or most of rare diseases
	Centres of expertise adhering to the standards defined by the Council Recommendations paragraph d) of preamble	Outcomes	0 %	90%
	Participation of national or regional centres of expertise into European reference networks	Outcomes	Approx. 10 %	100% of the main centres

Care for RDs - Centres of Expertise and European Reference Networks for Rare Diseases - EUCERD indicators				
Indicator	**Indicator description**	**Type of Indicator**	**Answers 2013**	**Answers 2017**
5. Existence of a national policy for establishing Centres of Expertise on RD	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	YES, existing, partly implemented
6. Number of national and regional Centres of Expertise adhering to the national policy	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	Four big university centres, with several affiliated ones.
7. Participation of national or regional centres of expertise in European Reference Networks	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	100% of the main centres are full members in 12 ERNs
10. Existence of a national policy for developing, adapting and	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	Process	• YES, a policy exists for developing CPGs,	• YES, a policy exists for developing CPGs

implementing clinical practice guidelines	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.		• YES, a policy exists for adapting CPGs, • YES, a policy exists for implemen-ting CPGs	• YES, a policy exists for adapting CPGs, • YES, a policy exists for implementing CPGs

THEME 3: Integration of RDs into Social and Family Policies, Early Intervention

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. 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. Therefore, any improvement on this area is essential for the RD patients.

Disability assistance can be provided to people with severe disabilities over 18 years of age living in Hungary, whose condition is likely to be long-lasting or definitive, furthermore, who is unable to lead an independent life or requires the help of others permanently.

Although, social services are not specialized to support people who suffer from rare diseases, some options exist within the frame of general disability rules. Besides other “traditional” disability groups (the visually handicapped, the hear-impaired etc.) disability assistance can be granted to someone, whose condition is medium severe or more severe due to chromosome disorder. (Thanks to the fight of RD community) According to the government decree 141/2000. (VIII. 9.) on rules of qualification and revision of severe disability and payment of disability assistance, those persons shall be regarded as living with chromosome disorder, whose condition is medium severe or more severe due to being born totally or partly with the lack or excess of autosomes or sex chromosomes.

One important service for the children suffering from rare diseases is the integrated early childhood intervention – a system of professional services for very young children at risk of developmental delays, disabilities or atypical behaviour. They need a holistic, comprehensive, transdisciplinary support as early as possible to let children thrive and progress when their brain development is the most active. However, there is inefficient coordination between social, health, and educational sectors with lack of transparent communication. Therefore, the new Intersectoral Development Programme of Early Intervention in Hungary (EFOP-1.9.5-VEKOP-16-2016-00001) is important to improve the situation. One main contributor to the project is the Hungarian Health Visitor System, with the public health visitor nurses with highly educated (college degree) persons, specialised in health promotion and prevention.

The progress in this area is essential for all rare disease patients with all age, because their care is based on the same principles.

There are several varied social programmes offering rare disease patients a wide variety of assistance and support. However, the capacity of the services is not enough:

- Rare Disease specific information centre is run by HUFERDIS.
- An official website of the Government Office of the Capital City Budapest (http://www.kormanyhivatal.hu/hu/budapest/hirek/bfkh-nyomtatvanyai) together with the homepage of Unified Disability Information Portal (http://www.efiportal.hu/) provides information on all the social and child welfare services available in Hungary.
- The website, http://info.kezenfogva.hu/, provides information on benefits and allowances, sport and leisure programmes, social, child welfare, health care services and educational possibilities.

- A compulsory task of every municipality with a population of over 10,000 is to maintain a day care for people with disabilities. Every municipality must also offer care within rare disease patient homes. These services can be outsourced to NGOs or to churches. Unfortunately, several times they are not able to do it, because the lack of finances!
- In case of the day care services eligibility is based on a medical document verifying the existing disability of the person, while in case of home care service a care needs assessment is necessary that determines the degree of care for supporting a self-sufficient life. Home care service can be realized in the forms of social support or personal care.
- In order to make community-based services accessible to more disabled people, municipalities have the possibility to establish basic services without administrative barriers and regardless of the available capacities determined by the minister, i.e. not having to go through the process of entering the financial system of social services.
- Therapeutic recreation programmes are organized mainly by NGOs.
- The legal framework of employment for people with disabilities is the Act CXCI of 2011 on Benefits for Persons with Changed Working Capacity. The focus is on retained working skills, health condition, physical and mental capabilities in the care and occupational rehabilitation system of people with disabilities. In accredited employment, there are two forms of benefits: 'transit employment' for 3 years, and 'long- term supported employment'. The accredited employer has to provide e.g. rehabilitation advisor, rehabilitation mentor and personal assistant if needed, and must have an occupational rehabilitation program and a rehabilitation plan.
- The other type of employment for people with disabilities is the developmental employment since first of April 2017. It is under institutional care or use of social services getting intensive social assistance. It has two types: a) working under the Labour Code means preparation for employment in the open labour market b) employment with development contracts means preservation and development of physical and mental abilities and preparation for independent working.
- The Rehabilitation Card is a tool for persons with disabilities to promote their employment in the open labour market. It means absolute immunity of the social contribution tax for the employer up to the minimum wage twice for the entire duration of the employment.
- People with disabilities have the possibility to use home care service. To do so it is necessary to determine the degree of care required to live a self-sufficient life.
- Supported housing is more than just community-based services. It also provides housing and specific services that are based on the individual's needs but these services cannot be provided at the same place therefore urges people with disabilities to use community-based services and live a partly or fully self-sufficient life even if they do not live in their own homes. The process of deinstitutionalization is also considered a long-term effort to further the positive impacts of supported housing and extend community-based services to a greater number of disabled people including people with rare diseases. The operational programmes supporting above objectives are EFOP-2.2.2./VEKOP-6.3.2 and EFOP-2.2.5.
- To facilitate a multidisciplinary, holistic and continuous care provision, rare disease patients can have access to individual care plans which include access to social and support services.

Despite of these nice measures, we need to continue our efforts to integrate RD patients into social and family policies, since a lot of patients are still unable to get the necessary

service because of the lack of services or capacities. 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. We still recommend establishing a Resource Centre, like NoRo, namely the National Habilitation, Information, Development and Service Institution for Rare Diseases according to our National Plan, in cooperation with the patient associations.

Social Services for Rare Diseases - EUROPLAN indicators				
Actions	**Indicators**	**Type of indicator**	**Answers 2010**	**Answers 2017**
Compensating disabilities caused by rare diseases	Existence of official programs supporting patients and families with disabilities	Process	Existing, clearly stated, partially implemented and enforced	Existing, clearly stated, substantially implemented and enforced
	Existence of an official directory of social resources for patients with disabilities	Process	In preparation	Yes
Supporting social services aimed at rare disease patients and their families	Existence of national schemes promoting access of RD patients and their families to Respite Care services	Process	In preparation	In preparation
	Existence of public schemes supporting Therapeutic Recreational Programmes	Process	In preparation	In preparation
	Existence of programmes to support integration of RD patients in their daily life	Process	In preparation	In preparation
Supporting rehabilitation programmes	Existence of programmes to support rehabilitation of RD patients	Process	Yes	Yes

Social Services for Rare Diseases - EUCERD indicators				
Indicator	**Indicator description**	**Type of Indicator**	**Answers 2013**	**Answers 2017**
17. Existence of programmes to support in their daily life RD patients integration	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; d) supporting mechanisms to participate in work life for people with disabilities.	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 foster higher education d, exists some supporting mechanisms to	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 foster higher education d, exists some supporting mechanisms to

			participate in work life, but with a descending rate	participate in work life, but with a descending rate

THEME 4: RD Communication/Information and Education/Training

Spreading information on RDs is a challenge. People with RD feel they are lost in the healthcare, social and education system. They probably do not speak languages to find information from English sites. HUFERDIS operates a national RD webpage since 2006 (http://www.rirosz.hu) together with several project specific websites). HUFERDIS gain at the Norwegian Found source to launch the „LifeBelt" Information Centre and Helpline in March 2015. Thanks to several awareness campaigns, it is well known in Hungary. Since May 2016. (= end of Norwegian Fund's support) its support is given by HUFERDIS.

Volunteers have translated many of the NORD descriptions of rare diseases. ORPHANET and EURORDIS pages are used to extract information from on RDs as well. HUFERDIS helps patients with translating and searching descriptions, special centres, fellowship groups, etc. HUFERDIS has an overall mailing list, and social media sites, so all its members (patients groups) get the information spread on them. There are groups for most of the diseases, even for the undiagnosed ones.

Through structured coordination of the infrastructure, HUFERDIS was able to develop data bases, quality assurance, answering protocol, a non-disclosure agreement and an ethics code. Different communication channels were also developed which included a dedicated a phone-line and website (http://mentoov.rirosz.hu), a new Facebook group for searching fellow patients and a YouTube channel. There is also help for healthcare and educating professionals on the webpage. The staff operating the helpline is the staff of HUFERDIS as well, having great amount of problems to solve. Helpline gets no governmental support: it is supported via private donations and money gained from funds and tenders.

For answering the calls and helping for RD patients some more personnel is needed.

Writing applications for tenders takes a great amount of time from the staff; to avoid this the helpline should be supported by the government. It would NOT be useful to give the operation itself to the government; an affected personnel's empathic help can't be supplemented with a simple paid employee.

There was an official information centre for rare diseases in Hungary (National Rare Disease Centre (NRDC)), maintained by the National Public Health and Medical Officer Service. After the reorganization of this service, the NRDC is sustained by the Ministry of Human Capacities. The new home page of NRDC will be available shortly on the information portal of children's primary care (http://www.gyermekalapellatas.hu).

Another great information event is the International Rare Disease Day, which is celebrated at national level every year, coordinated by HUFERDIS (http://ritkanap.rirosz.hu). Beside this, HUFERDIS participates in several international projects including Europlan, EUPATI and InnovCare which means it is well placed to ensure rare disease patients and healthcare professionals gain access to the most up to date information possible.

Hungary also has an operational National Orphanet team, which produces information in the Hungarian National language however; there is no dedicated funding in place to support their activities. The Orphanet team have participated in many varied activities since the beginning of 2014.

Concerning the education, the curricula of medical faculties contain courses dedicated to rare diseases, and there are courses, which deal with rare diseases. Accreditation and coordination of the mentioned trainings and events is important. Continuous information and training for professionals dealing with RD patients must be provided.

Information and training - EUROPLAN indicators				
Actions	**Indicators**	**Type of indicator**	**Answers 2010**	**Answers 2017**
Existence of an information sites for both professionals and patients provided by the plan/strategy	Existence of a comprehensive national and/or regional RD information system supported by the government	Process	Yes, covers only some RD	Yes, covers most RD
	Help lines for professionals	Process	Not formal decisions have been taken	Yes, covers most RD
	Help lines for patients	Process	No	Yes
	Clinical guidelines	Outcomes	17	No (Hungarian guideline)
Promoting training activities and awareness educational campaigns among professionals and patients	Number of such as activities promoted by the plan/strategy	Process	Not relevant	Not relevant

Information and training - EUCERD indicators				
Indicator	**Indicator description**	**Type of Indicator**	**Answers 2013**	**Answers 2017**
9. Existence of Help lines for RD	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. 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. 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.	Process	• No	• YES, operated by HUFERDIS

THEME 5: Research on RD

The first Hungarian RD Dedicated Research Programme was launched at 2017 for the development of novel diagnostic and therapy procedures for rare diseases. This € 3.8 million project is supported by the European Union and the State of Hungary (GINOP-2.3.2-15-2016-00039) and it is a real breakthrough on this area. The outcomes of this programme will provide an unparalleled opportunity, to understand the pathogenesis of rare diseases, to develop novel diagnostic and therapy procedures, and for the long-term integration of all these processes into clinical practice.

Besides this big project, Hungary has some smaller specific programmes in place to fund and facilitate rare disease research. For example, the E-Rare-3 project entitled “ERA-NET rare disease research implementing IRDiRC objectives" which is supported by the Hungarian Scientific Research Fund. The total amount of public funds allocated to these smaller rare disease research each year is around € 150,000.

Another successful research area is the field of clinical trials. Their number is continuously growing.

The socio-economic aspects of rare diseases are searched systematically in Hungary. There is no institution devoted at least partly to this purpose. HUFERDIS is participating in several previous programme, and now in the InnovCare Project and has initiated a dialogue with the Ministry of Human Resources to research the socio-economic aspects further.

The other side of the coin is represented by the weaknesses that persist. E.g. although attempts to systematically 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.

The sustainability of research programmes on RD is also questionable.

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).

The National Institute of Health Insurance Fund Management (NEAK) should support some new diagnostic examination methods (like next generation sequencing, micro-array CGH).

In defining research priorities and in the transparency of ongoing research, a constant interaction between researchers and patient organizations is needed. Patient organizations should be also regularly updated about recent research and their results. It is recommended that research centres invite patient organizations to their scientific councils and scientific organizations should include those meetings into their conference programme.

Research on RD - EUROPLAN Indicators				
Activity	**Indicators**	**Type of indicator**	**Answers 2010**	**Answers 2017**
Building a research programmes for RD	Existing a RD National/Regional research programmes	Process	RD research programme included in the general research programme as a priority	Specific research programme exists for RD
	RD research programme monitoring	Process	Existing, clearly stated, partly implemented	Existing, clearly stated, partly implemented
	Number of RD research projects approved by year (if possible yearly starting the year before plan commencement)	Outcomes	There isn’t any independent database	There isn’t any independent database
	Clinical trials funded by public bodies	Outcomes	No actions have been taken	Under discussion
	E-RARE joining	Process	In process	On going
	Including public health and social research, in the field of rare diseases	Process	Yes, partly implemented	Yes, partly implemented

	Research platforms and other infrastructures are also funded by the research programme	Process	Yes	Yes
Existence of national policy in support of the recruitment of young researchers/ scientists specifically for rare diseases	Number of young scientists recruited every year to work specifically on rare diseases	Process	There is a registry but we have no exact data.	There is no regular mapping, so we have no exact data.
Allocate funds for the RD research programme	There are specific public funds allocated for RD research	Process	No	Yes
	Funds specifically allocated for RD research actions/projects per year since the plan started	Outcomes	Not relevant	Yes there are RD research projects

Research on RD - EUCERD Indicators				
Indicator	**Indicator description**	**Type of Indicator**	**Answers 2013**	**Answers 2017**
13. Existence of a RD research programmes/projects in the Country	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	YES, specific research programme
14. Participation in European and international research initiatives	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	YES, E-RARE and IRDiRC
20. Specific public funds allocated for RD research	This indicator aims to identify the policy decision(s) to allocate a portion of the national research budget specifically to RD research.	Process	No	YES
21. Public funds specifically allocated for RD research actions/projects per year since the plan started	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	Yes there are RD research projects

THEME 6: Definition, codification and inventorying of RD

The EU's official definition of RD is still the accepted definition in Hungary, meaning a prevalence of less than 5/10 000 in the population. With 5-8 percent of the general population affected by one of the estimated 6000-8000 rare diseases, this amounts approximately 600 000-800 000 people in the country. The high number of patients and their family members, and the burden of rare disease on them, illustrate the size of the problem and the importance of managing it.

In Hungary the ICD10 codification system is applied. It is used for the registries and the normative cost refund. As about 250 RD have individual ICD codes in ICD-10 coding system, there is a possibility to follow their pathway and mortality showing a great improvement of these data:

Indicators	1980-1984	2005-2006
Registered standardized death rate (SDR/1,000,000)	**208**	**141**
Proportion of death during the first year of life [95%CI]	**0,28 [0,27 -0,29]**	**0,09 [0,08 -0,10]**
Mean age at death attributed to rare diseases (p for change < 0.001)	**37,3**	**57,7**
Potential years of life lost attributed to rare diseases (PYLL)	**66119**	**21900**

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.

The Hungarian Congenital Abnormality Registry (HCAR or VRONY in Hungarian) is member of the network of European Surveillance of Congenital Anomalies (EUROCAT) since 2003. The Decree 21/2014. (III. 20.) of the Ministry of Human Capacities was altered for life long data collection and for the application of Orpha Codes.

In 2017, the Electronic Healthcare Service (EESZT) successfully launched after several years of development. All processes of the healthcare system became digital. The structure applies Orpha Codes in the e-profile of patients increasing the visibility of rare diseases.

Unfortunately, there are very few implemented strategies to produce statistics on rare diseases at national level. Nevertheless, the production of statistics on rare diseases is among the strategies included in our national plans being “in design” now.

Definition, codification and inventorying of Rare Diseases - EUROPLAN Indicators				
Actions	**Indicators**	**Type of indicators**	**Answers 2010**	**Answers 2017**
To officially adopt the EC RD definition (no more than 5 cases/10,000 inhabitants)	Adoption of the EC RD definition	Process	Yes	Yes
To include the best RDs classification currently existing into the public health care related services	Type of classification used by the health care system	Process	ICD-10	ICD-10 + Orpha Code
	Developing policies for recognising RD by the care information systems	Process	Not existing, not clearly stated	Existing, clearly stated, partly implemented and enforced
Defining a surveillance system based on a patient outcomes registry	Registering activity	Process	Multiple RD registries, not standardised	Multiple RD registries, not standardised
	Number of diseases included	Outcomes	26	26

Definition, codification and inventorying of Rare Diseases - EUCERD indicators				
Indicator	**Indicator description**	**Type of Indicator**	**Answers 2013**	**Answers 2017**
4. Adoption of the EU RD definition	The EU defines “rare diseases” as those with a prevalence of no more than 5 patients per 10.000 persons. This definition is	Process	YES, the NP/NS measures	YES, the NP/NS measures

	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.		are applied using the EU definition	are applied using the EU definition
8. NP/NS support to the development of/participation in an information system on RD	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	YES, information centre exists, but without support from the government YES, participates in JA
11. Type of classification / coding used by the health care system	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.	Process	• ICD-10 • No ORPHA Code	• ICD-10 with ORPHA Code
12. Existence of a national policy on registry and data collection on RD	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.	Process	• YES, for national/centralised registry and data collection • YES, for regional registry and data collection	• YES, for national/centralised registry and data collection • YES, for regional registry and data collection

THEME 7: Orphan Medicinal Products

In Hungary, the drug acceptance process (transparency) to the social insurance is based on the EU transparency Directive (89/105/EK). The relevant Hungarian legislation is implanting the EU Directive, according to which difference is made among original/innovative and generic drug during the acceptance process. Producers of innovative orphan drugs which have already received approval for market release can ask for social insurance support via the regular process. In this case, within the time limit set by the EU, 90 days are open for price discussion, plus another 90 days to determine the rate of support, altogether 180 days are open. In Hungary, during the transparency process, governmental organs will only decide on the rate of the support. Since the local legislation is based on the EU one, therefore in normal process, 90 days are the time limit of acceptance.

The decision making route for pricing and social security reimbursement is complicated involving several players. The producer must submit its request to the National Institute of Health Insurance Fund Management (NEAK). Then a Health Technology Assessment is done by OGYÉI-TÉF (National Institute of Pharmacy and Nutrition, Dept. of HTA) with Consultancy by Health Professional Colleges and Medical Research Council (ETT) if necessary. Based on these work an opinion of HTA Committee of the State Secretariat for Health Care, Ministry of Human Capacities (EMMI) is created and a Preliminary Decision is made by NEAK. This can be modified by the suggestions of other departments Of State Secretariat for Health Care and of the Ministry for National Economy (NGM).

Many times support is only available by individual justice via discretional claims. Consideration of claims is difficult and time consuming, simplifying and speeding up the procedure is needed. Financing capacity of the state significantly limits the yearly budget (macroeconomic situation).

New early access option is the compassionate use of orphan drugs by the modification of Act XCV (2005). The competent authority (OGYÉI - National Institute of Pharmacy and Nutrition) gives the individual authorization. It is considered for drugs, which are during a phase II trial or a marketing authorization in Hungary or in another (EU or non-EU) country, when is no therapeutic

alternative. A specialized physician need to request individual authorization from the competent authority. Decision is made within 21 days (3 days in case of emergency).

Off label use of Drugs is also possible if it offers the potential of a successful treatment. However, the administration procedure is strongly bureaucratic. 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. 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. 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 National Institute of Pharmacy and Nutrition (OGYÉI). International best-practice and feedbacks are essential.

Orphan Medicinal Products – EUROPLAN indicators				
Activity	**Indicators**	**Type of indicator**	**Answers 2010**	**Answers 2017**
Ensure the mechanism that facilitates ODD access and the reimbursement of their cost to patients after they got the market authorization by EMA.	Number of ODD market authorizations by EMA and placed in the market in the country	Outcomes	70 %	86 %
	Time between the date of an ODD market authorization by EMA and its actual date of placement in the market for the country	Outcomes	0-10 day(s)	0-10 day(s)
	Time from the placement in the market in the country to the positive decision for reimbursement by public funds	Outcomes	90 day in normal procedure, however, this could be elongated in case of complicated cases.	90 day in normal procedure, however, this could be elongated in case of complicated cases.
	Number of ODD reimbursed 100%	Outcomes	33	43 (transparent reimbursement + patient-base financing)
To develop mechanisms to accelerate ODD availability	Existence of a governmental program for compassionate use for Rare Diseases	Outcomes	No	Yes

Orphan Medicinal Products – EUCERD indicators				
Indicator	**Indicator description**	**Type of Indicator**	**Answers 2013**	**Answers 2017**
15. Number of Orphan Medical Products (OMPs) with a	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	Outcomes	38	45

European Union marketing authorisation and available in the country (i.e. priced and reimbursed or directly supplied by the national health system)	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.			
16. Existence of a governmental system for compassionate use of medicinal products	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	Yes

III. Conclusions

The well-structured recommendations of the EU expert groups on rare diseases (together with the supporting documents and indicators) have served again 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 realization 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.

The summarized developments can contribute to define better patient directions, and thus temper the family ramble in the maze of health, social and educational systems. The realization of Rare Disease National Strategy is needed to improve the current fragmentation of services and enable patients and health, social and educational professionals to provide and use best practices. This will ensure that all patients with rare disease cannot only be diagnosed quickly, but also have timely access to the care and support that they need, resulting a decreasing burden of families and society.

However, to reach our goals, we need to take several further steps. The most important are:

- To finish the official national approval process of our RD National Plan.
- To create a Steering Committee of the implementation of the Plan by supplementing the current expert committee with representatives of all sectors, for the necessary coordination.
- To revitalize the National Rare Disease Centre (NRDC) after the reorganization, with exact description of the division of tasks and labour, to ensure the necessary human resources and tools.
- To update the webpage of NRCD with information on the development of the National Plan, therapeutic options, organisation of patients pathways, EU harmonisation (indicators and bio banks etc.), training, appointment of reference centres, research, social services etc.
- Involving patient representatives as experts in the implementation of the National Plan.
- To secure a regular governmental contribution to the running expenses of “Lifebelt” Information Centre and Helpline.
- To ensure RD specific, regular quality control of the accredited institutions by an independent supervising body must be insured, using existing EU compatible regulations (accurate patient registration, coding, multidisciplinary care, patient satisfaction etc.)
- To use European tools for measuring invisible disabilities, such as fatigue and pain are adapted so as to allow patients living with rare and chronic diseases to make use of

care, support and benefits available to children with special training and care needs. When modifying the tax system, it should be considered that, in addition to tax allowances provided for big families, families raising chronic and disabled children should also enjoy tax allowances.

- The mapping of RD specific information and applied procedures is necessary in the authorities dealing with rare diseases.
- Need to designate new Centres of Expertise in the western part of the country, and urge the participation of Hungarian Health Care Providers in European Reference Networks, especially in the uncovered ones.

V. Hungarian Europlan Conference

Time	Session Chairs	Theme
9:00-10:00	Registration	
9:15-9:40	Press Conference	
9:45-10:00	Opening Ceremony (Dr. Pogány Gábor, president of HUFERDIS, Dr. Pozsgay Csilla, Director-General of OGYÉI)	
10:00-11:30	Dr. Pfliegler György (DE), Dr. Hadzsiev Kinga (PTE), Dr. Pogány Gábor (HUFERDIS)	Status of our National Plan, European Reference Networks for Rare Diseases, e-Health. 10:00-10:30 Pogány Gábor: Status of our National Plan. 10:30-11:00 Pfliegler György: European Reference Networks, e-Health. 11:00-11:30 Hadzsiev Kinga: Example of a Working ERN - ITHACA-ERN.
11:30-13:00	Prof. Dr. Molnár Mária Judit (SE), Prof. Dr. Klivényi Péter (SZTE), Becskeházi-Tar Judit (HUFERDIS)	Implementation of Hungarian National Plan Centres of Expertise and Healthcare Pathways. 11:30-12:00 Molnár Mária Judit: Centres of Expertise and Healthcare Pathways. 12:00-12:30 Klivényi Péter: Further Tasks for the Implementation of the National Plan. 12:30-13:00 Common Discussion of the two first Themes.
13:00-13:30	Lunch	
13:30-15:00	Czeizel Barbara (Ministerial Commissioner), Szekeres Pál (Ministerial Commissioner), Kedves Éda (HUFERDIS)	Integration of RDs into Social and Family Policies, Early Intervention. 13:30-14:00 Czeizel Barbara: Intersectoral Development of Early Intervention. 14:00-14:30 Almási Zsuzsa (EURORDIS, NoRo): The INNOVCare programmme and the NoRo Centre. 14:30-15:00 Round Table with the Prominent Representatives of the Area: Dr. Márkus Eszter (ELTE), Kovács Zsuzsanna (FSZK), Dr. Csató Zsuzsa (Sorstárs Alapítvány)
15:00-15:45	Dr. Oberfrank Ferenc (SE), Dr. Zima Judith (PTE), Ábele Mária (HUFERDIS)	RD Communication/Information and Education/Training. 15:00-15:20 Ábele Mária: Information Efforts of HUFERDIS, the "Lifebelt" Information Centre. 15:20-15:40 Oberfrank Ferenc: RD Education and Training.
15:45-16:00	Coffe break	
16:00-16:45	Prof. Dr. Kissné Horváth Ildikó (OKPI), Dr. Balogh István (DE), Váradiné Csapó Judit (HUFERDIS)	Research on RD. 16:00-16:20 Kissné Horváth Ildikó: International Tendencies and the Hungarian Engagement Possibilities. 16:20-16:40 Balogh István: The first Hungarian RD Dedicated Research Programme - RitBetKut.
16:45-17:30	Dr. Sándor János (DE), Prof. Dr. Boncz Imre (PTE), Dr. Süliné Vargha Helga (HUFERDIS)	Definition, codification and inventorying of RD. 16:45-17:05 Sándor János: "Invisibility" of Rare Diseases - Troubles and Solution Opportunities. 17:05-17:25 Dr. Horváth Lajos (Budai Irgalmasrendi Kh.): Orpha Codes in the Electronic Healthcare Service - Option for RD Patients.
17:30-18:15	Dr. Németh György (MSZMT), Dr. Sobor Melinda (OGYÉI), Fogarassy Károly (HUFERDIS)	Orphan Medicinal Products. 17:30-17:45 Németh György: Innovative Trends at the beginning of 21. Century. 17:45-18:00 Sobor Melinda: National and International Regulatory Practice. 18:00-18:10 Fogarassy Károly: Patient Experiences.
18:15-18:30	Closing Remarks	

List of Participants

Name		Organization	Role	Stakeholder group
Ábele	Mária	Hemokromatózisos Betegek Egyesülete	Speaker	patient representative
Almási	Zsuzsa	Romániai PWE	Speaker	patient representative
Ambrus	Kiry Noémi	RIROSZ	participant	expert
Ambrus Dr.	Bence	Bethesda Gyermekkórház	participant	expert
Balogh Dr.	István	Debreceni Egyetem, Laboratóriumi Medicina Intézet	Speaker	expert
Becskeházi-Tar Dr.	András	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
Berényi- Tóth	Andrea	Országos Cisztás Fibrózis Egyesület	participant	patient representative
Béres Dr.	Judit	PTE ÁOK Orvosi Népegészségtani Intézet	participant	expert
Bocskai	Krisztián	Feledékeny Emberek Hozzátartozóinak Társasága	participant	patient representative
Bojtor	Zsuzsa	RIROSZ, MWSzT	participant	patient representative
Bóka	Krisztina	Zak Pharma Dienstleistung GES.M.B.H.	participant	Industry
Boncz Dr.	Imre	Pécsi Tudományegyetem Egészségtudományi Kar Egészségbiztosítási Intézet	Speaker	expert
Borka Dr.	Péter	Novartis	participant	Industry
Czeizel	Barbara	EMMI	Speaker	expert
Czirjákné dr. Kispál Mónika	Mónika	egyéni ügyvéd	participant	patient representative
Csató Dr.	Zsuzsa	Csupaszívek	Speaker	expert, patient representative
Csernyák	Mária	MWSzT	participant	patient representative
Csurján	László	HBK	participant	patient representative
Demcsik	Mónika	Mps Társaság	participant	patient representative
Farkas	Márk	VmKomm	participant	organizer
Farkas	Noémi	VmKomm	participant	organizer
Fehér	Zsuzsanna	Sanofi GEnzyme	participant	Industry
Fehér Dr.	Gabriella	AOP Orphan Pharm.	participant	Industry
Fekete	Gáborné	Védőnő	participant	media
Fogarassy	Eszter	RIROSZ/Fabry-Betegekért Alapítvány	participant	patient representative
Fogarassy	Károly	Fabry-betegekért Alapítvány	Speaker	patient representative
Fogarassy	Lili	Fabry-Betegekért Alapítvány	participant	patient representative
Győri-Horváth	Dóra	CSBO	participant	expert
Hadzsiev Dr.	Kinga	PTE KK Orvosi Genetikai Intézet	participant	expert

Héveiné	Szabó Ágnes	SBH	participant	patient representative
Horváth	Katalin	SBH	participant	patient representative
Horváth Dr.	Ildikó	Orsz. Korányi Pulm. Int.	Speaker	expert
Horváth Dr.	Lajos	Budai Irgalmasrendi Kh.	Speaker	decision maker
Huszti Dr.	Zoltán	Nemzeti Egészségbiztosítási Alapkezelő	participant	decision maker
Ispán	Fanni	Nemzeti Egészségbiztosítási Alapkezelő	participant	decision maker
Juhász Dr.	Hedvig	Sanofi-Aventis Zrt.	participant	Industry
Kalmár	Mónika	Rirosz	participant	patient representative
Karcagi Dr.	Veronika	OKI-MGDO	participant	expert
Kedves	Éda	SVOE	participant	patient representative
Kereki	Judit	CSBO	participant	expert
Kiss	Zsoltné	Primer Immunhiányos Betegek Egyesülete	participant	patient representative
Kiss	Gergő	SE	participant	expert
Kiss	Orsika	Narkolepszia klub	participant	patient representative
Klivényi Dr.	Péter	SZTE	Speaker	expert
Klujber Dr.	Valéria	EMMI	participant	decision maker
Koltai	Tünde	Lisztérzékenyek Érdekképviseletének Országos Egyesülete	participant	patient representative
Koncsek	Rita	Világgazdaság	participant	media
Koós	Ilona	Hemofiliások Baráti Köre	participant	patient representative
Kovács	Zsuzsanna	FSZK nonprofit Kft.	participant	expert
Köbli	Anikó	Medical Online	participant	media
Lengyel Dr.	Ingrid	EMMI IJSZ	participant	decision maker
Magyar	Éva	Mitokondriális Betegek Baráti Köre	participant	patient representative
Márkus Dr.	Eszter	ELTE Bárczi Gusztáv Gyógypedagógiai Kar	Speaker	expert
Medgyesy	Brigitta	Országos Cisztás Fibrózis Egyesület	participant	patient representative
Mészáros	Fanni	OTFHÁT	participant	expert
Miletic Lajko	Dragana	MPS Serbia	participant	patient representative
Mina	András	IJSZ	participant	expert
Molnár Dr.	Mária Judit	SE Genomikai Medicina és Ritka Betegségek Intézete	Speaker	expert
Nagy	Ákos	VmKomm	participant	organizer
Nagyné Kijátz	Edina	Nemzetközi Pető Intézet	participant	expert
Németh Dr.	György	MSZMT, Richter	Speaker	expert
Oberfrank Dr.	Ferenc	MOTESZ, MTA KOKI	Speaker	expert
Paszterkó	Judit	CSBO	participant	expert
Pataki	Anita	MWSzT	participant	patient representative
Pfliegler Dr.	György	DE OEC B.I. Ritka Beteg Központ	Speaker	expert

Pogány Dr.	Gábor	RIROSZ, NBF	Speaker	patient representative
Pogány	Krisztina	MWSZT	participant	patient representative
Pozsgay Dr.	Csilla	OGYÉI	Speaker	decision maker
Rétlaki Dr.	Szilvia	Nemzeti Egészségbiztosítási Alapkezelő	participant	decision maker
Sándor Dr.	János	DE EOC Népegészségügyi Kar, Megelőző Orvostani Intézet, Biostatisztikai és Epidemiológiai Tanszék	speaker	Expert
Schultheisz Dr.	Judit	Gézengúz Alapítvány	participant	expert
Sipos	György	SOE Bioinformatika	participant	expert
Sobor Dr.	Melinda	OGYÉI	Speaker	expert
Süli-Vargha Dr.	Helga	RIROSZ	speaker	patient representative
Szekeres	Pál	EMMI	Speaker	decision maker
Szilágyi	Anna	Magyar Idők	participant	media
Szili	Danijela	MRSZA	participant	patient representative
Tischler Dr.	Erika	Sanofi-Aventis Zrt.	participant	Industry
Uhlyarik Dr.	Andrea	Sanofi Genzyme	participant	Industry
Urbanovszky	Zsuzsanna	FSZK nonprofit Kft.	participant	expert
Varga Dr.	Gábor	Magyar Hemofília Egyesület	participant	patient representative
Vidákovich	Nándor	Fabry-Betegekért Alapítvány	participant	patient representative
Vidor	Eszter	VmKomm	participant	organizer
Vinczicki	Áron	NEAK	participant	decision maker
Zima Dr.	Judith	Pécsi Tudományegyetem/Klinikai Központ/Orvosi Genetikai Intézet	Speaker	expert