2014 REPORT ON THE STATE OF THE ART OF RARE DISEASE ACTIVITIES IN EUROPE

STATE OF THE ART OF RARE DISEASE ACTIVITIES IN HUNGARY

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The European Union Committee of Experts on Rare Diseases (EUCERD) was established in 2009 and its mandate ended in 2013. It is replaced from 2014 by the Commission Expert Group on Rare Diseases. The EUCERD Joint Action continues to support the activities of the new Expert Group until 2015.

More information on the European Union Committee of Experts on Rare Diseases can be found at www.eucerd.eu.

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ACRONYMS

CAT - Committee for Advanced Therapies at EMA
CHMP - Committee for Medicinal Products for Human Use at EMA
COMP - Committee on Orphan Medicinal Products at EMA
DG - Directorate General
DG Enterprise - European Commission Directorate General Enterprise and Industry
DG Research - European Commission Directorate General Research
DG Sanco - European Commission Directorate General Health and Consumers
EC - European Commission
ECRD - European Conference on Rare Diseases
EEA - European Economic Area
EMA - European Medicines Agency
ERN - European reference network
EU - European Union
EUCERD - European Union Committee of Experts on Rare Diseases
EUROCAT - European surveillance of congenital anomalies
EUROPLAN - European Project for Rare Diseases National Plans Development
EURORDIS - European Organisation for Rare Diseases
FDA - US Food and Drug Administration
HLG - High Level Group for Health Services and Medical Care
HTA - Health Technology Assessment
IRDiRC – International Rare Diseases Research Consortium
JA - Joint Action
MA - Market Authorisation
MoH - Ministry of Health
MS - Member State
NBS - New born screening
NCA - National Competent Authorities
NHS - National Health System
PDCO - Paediatric Committee at EMA
RDTF - EC Rare Disease Task Force
WG - Working Group
WHO - World Health Organisation
GENERAL INTRODUCTION

This document was produced by the Scientific Secretariat of the European Union Committee of Experts on Rare Diseases (EUCERD), through the EUCERD Joint Action: Working for Rare Diseases (N° 2011 22 01), which covers a three year period (March 2012 – February 2015).

The European Union Committee of Experts on Rare Diseases (EUCERD) was established in 2009 and its mandate ended in 2013. It is replaced from 2014 by the Commission Expert Group on Rare Diseases. The EUCERD Joint Action continues to support the activities of the new Expert Group until 2015.

The present report aims to provide an informative and descriptive overview of rare disease activities at European Union (EU) and Member State (MS) level in the field of rare diseases and orphan medicinal products up to the end of 2013. A range of stakeholders in each Member State/country have been consulted during the elaboration of the report, which has been validated as an accurate representation of activities at national level, to the best of their knowledge, by the Member State/country representatives of the Commission Expert Group on Rare Diseases. The reader, however, should bear in mind that the information provided is not exhaustive and is not an official position of the European Commission, its Agencies or national health authorities.

The report is split into six parts:

Part I: Overview of rare disease activities in Europe  
Part II: Key developments in the field of rare diseases in 2013  
Part III: European Commission activities in the field of rare diseases  
Part IV: European Medicines Agency activities and other European activities in the field of rare diseases  
Part V: Activities in EU Member States and other European countries in the field of rare diseases  
Part VI: Activities at National level in each EU Member State and other European countries in the field of rare diseases

Parts I – V also include a description of the methodology, sources and validation process of the entire report, and a selected bibliography and list of persons having contributed to the report.

The present document contains the information from Parts II and V of the report concerning Hungary. A list of contributors to the report and selected sources are in annex of this document. For more information about the elaboration and validation procedure for the report, please refer to the general introduction of the main report.

Each year, there are around 15 000 downloads of the different sections of the report combined.
RARE DISEASE ACTIVITIES IN HUNGARY

Definition of a rare disease
Stakeholders in Hungary accept the European Regulation on Orphan Medicinal Products definition of a prevalence of no more than 5 in 10,000 individuals.

National plan/strategy for rare diseases and related actions
A decree of the Ministry of Health established the National Rare Disease Centre (NRDC) in Hungary on 11 November 2008 as a part of the National Centre for Healthcare Audit and Inspection (OSZMK) by modifying its foundation deed. The National Centre for Healthcare Audit and Inspection was a part of the National Public Health Institute, and was empowered to investigate quality related issues in health care, public health consequences of health care operation, and to initiate interventions if needed. Due to the restructuring of the national public health institutions, OSZMK was disbanded on 30 April 2011. The National Institute for Health Development (OEFI) became the new host organisation, which is subordinated to the Chief Medical Officer and is a part of the central public health institutions. The continuation of the NRDC operation has been ensured by the modification of the foundation deed of OEFI.

The NRDC participates in preparation of recommendations for Governmental Health Authorities in the following ways:
- It elaborates its own data collecting technology and co-operates with other stakeholders in order to obtain rare diseases related data and to prepare indicators;
- It defines public health indicators for rare diseases;
- It initiates the elaboration of rare disease guidelines and carries out the audit projects;
- It maintains the national database of rare diseases specialised health care providers;
- It contributes to the assignment of national centres of expertise and their participation in European networks;
- It facilitates the establishment and operation of the quality management programs for the Hungarian rare diseases laboratories;
- It facilitates the application of e-health in rare disease related care;
- It initiates the rare diseases teaching programs launching in the universities;
- It participates in the work of national agencies responsible for orphan medicinal product and orphan medical device legislation;
- It supports the improvement of the availability of special social services for rare disease patients;
- It supports the effective primary preventive program;
- It evaluates the effectiveness of the rare diseases screening programs;
- It facilitates rare disease research projects, both national and the international co-operations;
- It contributes to the development of collaboration between governmental bodies, health care providers and patient organisations;
- It supports the Hungarian participation in the European rare diseases projects;
- It initiates programs, which contribute to the improvement of the perception of rare diseases among the general public;
- It co-ordinates the elaboration and monitoring of national policy on rare diseases;
- It reports on the Hungarian achievements regularly.

The NRDC is supported by an advisory group; the member experts were appointed by the Chief Medical Officer. Its members are from the four medical universities (the Hungarian Medical Universities’ representatives to the national advisory group are nominated by the deans), governmental institutions, and patient organisations. This group has a key advisory function of strategic planning, but does not have influence and control on the implementation of the decisions made.

The NRDC established a National Plan Organising Committee by supplementing the current expert committee with representatives of sectors such as government and industry and the patient groups. The Ministry is in the designation process of a competent, responsible Head of the expert committee, authorised to make decisions, to lead the development of the National Plan for Rare Diseases. However, currently there is no appointed Organising Committee, state or governing body to coordinate or implement the Plan.

The former IT centre facilities of OSZMK are under reconstruction for the systematic analysis of the hospital and outpatient discharge records of rare diseases patients (for rare diseases which have their own
ICD10 code), as well as laboratories, research programmes and patient groups. This investigation constrained to the rare diseases with distinct ICD10 codes tries to demonstrate the usefulness of rare disease specific indicators both for quality management and for public health purposes. On the basis of the results of these analyses, a project was initiated to introduce the orphan codes into the discharge records of hospitals and outpatient services.

The NRDC also works with the National Rare Disease Research Coordination Centre established in 2009 under the umbrella of OSZMK (host institution of NRDC) and the University of Pecs. This unit operates under the monetary support of the University of Pecs.

The NRDC cooperates with the National Ministerial Board for Clinical Genetics and with the officials responsible for rare diseases policy at the Ministry of Health, and at the National Institute for Quality and Organisational Development in Healthcare and Medicines (GYEMSZI). Project based collaboration has been established with universities’ rare disease coordination unites, sociological centres (for studying sociological characteristics of the patient groups), the National Centre for Statistics (for studying the mortality trends of rare diseases), and the Hungarian Federation of People with Rare and Congenital Diseases (HUHERDIS).

At the Europlan Hungarian national conference on rare diseases¹, organised by HUHERDIS on 18-19 October 2010 in Budapest, it was suggested that the issue of rare diseases should be adapted into the present, on-going reorganisation of the health care and social care system.

At the end of 2011, the main content of the plan was finalised and an expert meeting was held to finalise the chapters. Expert opinion was sought on the plan in March 2012. The third Hungarian Europlan Conference on Rare Diseases² (16-17 November 2012) was organised by HUHERDIS, together with the Ministry of National Resources, in Budapest. All stakeholders were present during the two day conference aimed at defining further concrete steps to develop a National Plan for Rare Diseases.

By the end of 2012 the National Plan for Rare Diseases was submitted to the Ministry of Health. The fourth Hungarian Europlan conference on 25-26 October 2013, organised with the participation of the Ministry of Health, examined the document and the budgetary question: the conference was a lively one thanks to the signing of National Plan for Rare Diseases by the Minister of Human Resources. The National Plan for Rare Diseases has now been approved (at the end of 2013)³ and there is an elaborated budgetary plan for the 7 year strategy. The approval of the budget proposal is expected in 2014 in order to finalise the plan. The National Plan for Rare Diseases 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 National Plan for Rare Diseases, including the allocation of a dedicated budget. The implementation of National Plan for Rare Diseases is jeopardised by some uncertainty caused by the prospective MP election, therefore the enhancement of national and international advocacy work is continuously necessary. Beside the National Plan for Rare Diseases the Ministry plans rare disease specific communications in the scope of the project “Development of public health communication” supported by Cohesion Fund.

In 2012 an expert group was also established at the Ministry of Health to identify the technical specification for a pilot study concerning the introduction of Orphacodes into hospital and healthcare centre records and the study started in 2013.

Hungary also has a National Cancer Plan where the rare cancers are covered.

The formation of a RD help line system, together with the development of rare disease emergency cards was also discussed in the 4th Europlan conference. However, no steps have been made yet.

**Centres of expertise**

There are currently no officially approved centres of expertise in Hungary, although eight are informally recognised. There are four university centres with expertise in the field of rare diseases and diagnostic and therapeutic facilities: Budapest, Szeged, Pecs and Debrecen. In Hungary, a committee on the treatment of rare conditions has been set up within the Scientific Health Council (Egészségügyi Tudományos Tanács). It ensures, *inter alia*, that people suffering from such conditions receive adequate care in all cases. People suffering from rare conditions in Hungary are registered at the treatment centres.

The much of the designation criteria for centres of expertise (objectives, scope, task, indicators, etc.) have already defined on the basis of the EUCERD Recommendations in this area in the National Plan for Rare Diseases. Two main factors are to be considered for the designation of Hungarian national centres of expertise:

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² [http://europlan.rirosz.hu/](http://europlan.rirosz.hu/)
the presence of equipment for diagnosis, and personal expertise of the medical professionals in the centre. In Hungary, the need for 5 rare disease centres playing a coordinating role has been identified. The 4 existing medical universities could play this role, but it has to be assured that the adequate expertise is provided in these centres. Healthcare pathways will be considered as will interdisciplinary, which should be a key feature of the designation. In the National Plan for Rare Diseases, therefore, the strategy will be to designate the four medical universities as centres of expertise due to the existing structure of the health system by speciality and the prominence and reputation of the medical university in terms of research, amongst other disciplines. There are expert groups outside of the medical universities who respect the criteria, but the ways of involving these groups into the existing structures need further examination.

NRDC initiated a collaboration with the National Health Insurance Fund for the listing and transparent accreditation of centres of expertise, hospitals, and laboratories working in the field of rare diseases taking into account existing resources and their concentration, as well as eliminating parallelism and formalising existing informal relations and determining patients’ pathways. The research project final report is expected to be published in 2014.

The NRDC has also initiated an open registry concerning the activities of centres of care and expertise, including the activities of consultants and laboratories requiring accreditation.

The legal base for establishing rare diseases expert centres had been set up at the end of 2013 by the Ministry of Health. The details of the designation process are to be elaborated.

Registries

The National Register of Congenital Anomalies (VRONY) operates countrywide according to the EUROCAT protocol. The former case definition of VRONY (congenital anomalies diagnosed from conception to the end of the first year of the newborn) has been extended by eliminating the age limit. Consequently, all the diagnosed congenital anomalies are to be reported from 2013 in an obligatory manner. The NRDC has initiated the establishment of an overall register for rare diseases. Currently, the clinical centres of rare diseases maintain registries of cared patients: these registries do not report their cases to a national data collecting system, and their registration methodology is developed according to the local need of care management and to the research requirements. All of these registries are in line with the Hungarian laws on genetic data handling and on the personal data protection. Hungary contributes to European Registries such as TREAT-NMD, EUROCAT, SCNIR and EUROCARE CF.

Neonatal screening policy

A compulsory, government-financed newborn screening program covering the whole country has been running since 1975, and after introduction of tandem mass spectrometry screening in 2007, now includes 26 diseases, amongst which phenylketonuria, hypothyroidism, galactosaemia and biotinidase deficiency which belong to the classic core. Two centres are responsible for the operation of this nationwide network.

A HURO-euro programme started in May 2011 on the “Newborn screening and molecular genetic diagnosis of rare diseases: developing a Euro-regional infrastructure and cooperation”. The University of Szeged is the project leader, and the Clinic de Urgența pentru Copii "Luis Țurcanu", (Timișoara) and Universitatea de Vest "Vasile Goldis" (Arad) are the Romanian partner institutions. The cooperation was active in 2012.

Genetic testing

The Genetic Professional College established in 2004 a protocol adopted by the Ministry of Healthcare entitled Genetic Consultation, which defines the conditions necessary for supplying the laboratory background, the infrastructure and the personal/operational costs for genetic diagnosis. Around 20% of laboratories have at least one diagnostic test validated by an external quality control scheme. The National Centre for Healthcare Audit and Inspection has also initiated an open registry including laboratories requiring accreditation.

Genetic diagnostic testing abroad is available through an application process to the National Health Insurance Fund and in many instances the Fund reimburses the costs.

Diagnostic tests are registered as available in Hungary for 57 genes and an estimated 77 diseases in the Orphanet database4.

4 Information extracted from the Orphanet database (January 2014): an update has been provided to the coordinating team which is being entered into the database.
National alliances of patient organisations and patient representation

The Hungarian Federation of People with Rare and Congenital Diseases (HUFERDIS) is the national alliance of 42 rare disease patient organisations in Hungary, affiliated with EURORDIS. HUFERDIS is currently encouraging the creation of a Hungarian Rehabilitation Centre for Rare Disease Patients which has got into the priority list of Norwegian grant of Hungary. HUFERDIS represents rare diseases patients in the Hungarian Expert Committee of Rare Diseases (which was the base of the forming National Plan Organising Committee), the Council of National Alliances (CNA) of EURORDIS, and at the EUCERD.

Patient organisations provide information and act as contact points for rare disease patients and organise conferences. HUFERDIS organised an Expert Committee to help the National Plan Organising Committee in the development of National Plan for Rare Diseases, and participates in the accreditation of centres of expertise, the determination of guidelines, and in the therapeutic education and care programs, medical and social care training etc.

Non-medical services for rare disease patients are currently available at local level or by non-profit organisations. Patient organisations are partly supported by the ‘1% Law’ which allows taxpayers to transfer 1% of their previous year’s taxable income to a non-profit organisation (which may be a patient organisation), without loss of income. Grants from the new National Fund of Cooperation are also available to patient organisations. There is no regular, direct governmental support for rare disease self-help groups, but there are many indirect governmental financing mechanisms: 25% of the civil budget is from governmental sources. HUFERDIS does not receive nominative state support such as that received by other umbrella patient organisations in Hungary.

Following previous collaboration established between HUFERDIS, NRDC and the Hungarian Orphanet team, new projects were not carried out because of the reorganisation of NRDC. However a new system was established at the National Health Insurance Fund for the evaluation of high valued medicines and care, and the representatives of HUFERDIS were invited to this expert committee. HUFERDIS takes part in several international projects including Europlan, POLKA, BURQOL-RD, Rare Disease Days, EUPATI. To foster the opinion of patient representatives on future European policies for rare diseases, or to collect their views on existing ones, HUFERDIS participated on the European POLKA project coordinated by EURORDIS. HUFERDIS started new cooperation with other international organisations as well, including DIA, EPF, EPHA, ECOP, ISOQOL.

During 2013 HUFERDIS played an important role in the establishment of Hungarian National Patient Forum, resulting in the election of its president as the Coordinator of the Forum. The federation was also a funder member of the new Hungarian Alliance of Patient Organisations (HAPO).

Sources of information on rare diseases and national help lines

Orphanet activities in Hungary

Since 2004 there is a dedicated Orphanet team in Hungary, initially hosted by the University of Pecs. After its establishment, the NRDC was designated as the official Orphanet team for Hungary in 2010 by the Ministry of Health. This team is in charge of collecting data on rare disease related services (specialised clinics, medical laboratories, ongoing research, registries, clinical trials and patient organisations) in their country for entry into the Orphanet database. The Orphanet website is widely used by professionals. There is an Orphanet national website launched in 2012, in the Hungarian language⁵.

Official information centre for rare diseases

There is currently no official information centre for rare diseases in Hungary apart from Orphanet.

Helpline

There is currently no rare disease specific helpline in Hungary. Many patient organisations provide support by telephone. HUFERDIS started a process to establish the necessary help line system, by applying for a Norwegian grant.

Other sources of information

Some websites, maintained by the government (www.gyemszi.hu, www.oefi.hu/aboutus.htm), have limited information concerning rare diseases. Scientific societies (www.mhgt.hu), non-governmental expert groups (www.betegmagzat.hu) and market-based organisations (www.webdoki.hu) have web based services for

⁵ http://www.orpha.net/national/HU-HU/index/honlap/
patients. The only other significant rare disease-specific website is the homepage of HUFERDIS (www.rirosz.hu). Several member associations of HUFERDIS have also detailed specific websites for a given rare disease.

Guidelines
Guidelines related to rare diseases had been produced by the Ministry of Health and are available including: autism spectrum, cystic fibrosis, diagnosis of the inherited metabolic diseases, genetic counselling, haemophilia, investigation of familial clustering of anomalies, investigation of multiple congenital anomalies, Legg-Calve-Perthes disease (Perthes disease), multiple sclerosis, myasthenia gravis, Osgood-Schlatter disease, prenatal screening of Down syndrome, Scheuermann disease, systemic lupus erythematosus, Tibial hemimelia, clubfoot. Hungary supports the participation of local experts in the development of international guidelines that should help diagnosis and care of rare diseases patients at national level. Some guidelines have been developed in collaboration with patient organisations and specialised clinics, in line with the new European guidelines (e.g. Williams syndrome). One of the missing guidelines in the field of rare diseases was a national protocol for the communication of a diagnosis: another expert team of HUFERDIS has thus developed a new rare disease protocol to properly communicate a diagnosis. The reformulation of the expired guidelines is the dedicated task of a governmental institution (GYEMSZI subordinated to the Ministry of Health).

Training and education initiatives
The education provided to health professionals currently includes information about the existence of rare diseases and the resources available for their care. This includes medical training in fields relevant to the diagnosis of rare diseases (genetics, oncology, immunology, neurology, paediatrics), further education for young doctors and scientists working in the field of rare diseases, and exchange and sharing of expertise between centres of expertise in the country.

Annual courses on rare diseases for graduates and postgraduates have been held at the Debrecen University, Department of Rare Diseases, since 2003 (with 40-100 participants). Rare diseases are also discussed at the Days of Internal Medicine of Debrecen (100-150 participants) which have been held seven times to date. The Department of Medical Genetics at the University of Pécs has organised since 2009 3-day clinical genetics course covering among others the diagnosis and management of selected rare diseases; the meeting is intended for specialists in the field as well as for family practitioners. The course in October 2012 aimed to show the interdisciplinary participation of various disciplines in the diagnosis and care of rare disease patients. The institutions and clinics participating in rare disease care constitute a Rare Disease Network of the University of Pécs established in February 2012. The Hungarian Clinical Neurogenetic Society organises annual meetings, which focuses on inherited neurological and neuromuscular disorders. The Semmelweis University also organises courses even for patients such as the “Molecular Medicine for Everybody”. Regular conferences are organised on the area of rare diseases by the Hungarian Society of Personalised Medicine or by Industry, like the Personalised Healthcare Days of Roche.

The Epidemiology of Rare Diseases has been accepted as research area by the Health Sciences Doctoral School of University of Debrecen. The students are involved in the folic acid supplementation, prenatal screening, patient pathway and diagnostic delay investigations.

National rare disease events in 2013
HUFERDIS, the Hungarian rare disease alliance, organised a number of events to mark Rare Disease Day in Hungary in both Budapest and Pécs.

In the central event, there were parallel professional meetings in Budapest. A professional conference brought together patient associations, healthcare professionals, pharmacists, technical diagnostic support specialists and also decision makers to share their opinions and ideas on how to act in the field. There was a roundtable discussion about the National Plan for Rare Diseases, updating the authorisation procedures concerning rare diseases. An overview of diagnostic opportunities and new horizons was given, summarising the experiences and finished projects from last year’s efforts. There were several useful programmes for families as well, including patient organisation presentation booths, poster sessions, a press conference, play ground, crafts and entertainment. HUFERDIS also organised a Rare Beauties exhibition to mark the Day in an artistic fashion. A Solidarity Walk of 1,7 km through the City Park was also organised bringing together patients and those supporting them.

As a joint effort of the Department of Medical Genetics of the University of Pécs, the Éltes Mátéys School for Children with Special Needs, and the Pécs Gallery at the Zsolnay Cultural Center, an information day for all rare disease patients in the region was held on 23 February 2013. The Zsolnay Cultural Centre, located on the site of the famous Zsolnay porcelain factory in Pécs, proved to be an attractive location for the patients,
families, caregivers, and local people interested in rare diseases. The backbone of the program consisted of musical performances by the students of the Éltes Mátyás School, many of whom are affected by rare diseases themselves, and information stands of various Hungarian rare disease patient organisations and caregivers from the region. Speeches given by the Dean of the Medical Faculty, the Director of the Clinical Centre, and the Vice Rector of the University, stressed the role of the University and its academic resources in the region’s rare disease care and research.

As previously mentioned, the fourth Hungarian Europlan Conference on Rare Diseases was organised by HUFERDIS on 25 October 2013, when the Hungarian National Plan for Rare Diseases was publicly presented for the first time. It is a strategy of health policy from 2014-2020 for RD.

Hosted rare disease events in 2013
No reported events.

Research activities and E-Rare partnership

National research activities

Governmental research funds for rare diseases are available from the Hungarian Scientific Research Fund. The Ministry of Health announces its health related research grants through the Scientific Health Council (ETT), Department of Research Coordination every three years. In the last evaluated period (2009-2011) 166 research grants were supported from 495 applications. In these programs, rare diseases were not one of the priority areas, but many rare diseases related grants were financed (e.g. governmental supported the project on the periconceptional folate status and on attitude towards different supplementation programs).

A multidisciplinary centre had been established in the Semmelweis University (Budapest) on rare disorders. The centre organises its work according to the principals published in the Communication from the European Commission on Rare Diseases. The centre has a patient registry, a diagnostic department, a multidisciplinary care providing network, research projects, and a teaching program. The coordinator of this Rare Disease Centrum is the Institute of Genomic Medicine and Rare Disorders.

To ensure the scientific expertise for NRDC, the general director of the National Centre for Healthcare Audit and Improvement, the rector of Pecs University, and the head of the Department of Medical Genetics signed the detailed agreement which established the National Rare Disease Research Coordinating Centre on the 21 April 2009; this Centre is still embedded into the Department of Medical Genetics of University of Pecs. The Medical Faculty, Faculty of Health Sciences and the Faculty of Special Pedagogy are involved in this cooperative project. The experts employed by these faculties come from the fields of medicine, paramedicine, social services and education. This working environment is expected to improve the Hungarian teams’ ability to contribute to the work of European organisations.

All Hungarian Medical Faculties have started to establish their own coordinating centres to harmonise their rare diseases related activities, including research.

Participation in European research projects

Teams from Hungary participate/participated in 9 FP7 rare disease related projects.

E-Rare

Hungary is full partner of E-Rare-2 via the National Rare Disease Research Coordinating Centre at University of Pécs. Hungary did not participate in the 4th Joint Transnational Call in 2012, but it did participate in the 5th Joint Transnational Call in 2013 although no Hungarian teams participate in the selected projects.

IRDiRC

Hungarian funding agencies have not currently committed funding to the IRDiRC, but as part of the E-Rare group of funders there is the possibility for Hungary to participate in the IRDiRC through the University of Pecs.

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6 The summary report of the 2009-2011 program evaluation is available here: [http://www.ett.hu/palyazat/tam_09_11.pdf](http://www.ett.hu/palyazat/tam_09_11.pdf)
7 [http://www.molneur.eoldal.hu/cikkek/english](http://www.molneur.eoldal.hu/cikkek/english)
Orphan medicinal products

The holders of marketing authorisations for orphan medicinal products (or their representatives in Hungary) cooperate with the medical profession and the OEP (The National Health Insurance Fund - Országos Egészségbiztosítási Pénztár).

Orphan medicinal product committee

There is no committee for orphan medicinal products in Hungary, but there is a new committee for the evaluation of reimbursement inclusion decisions in case of highly expensive medicines and care. A new system was established at the National Health Insurance Fund for the evaluation of high valued medicines and care, besides professionals caring for patients with rare diseases where high valued therapy is available, the representatives of HUFERDIS were invited to this expert committee. The committee has issued guidelines on the diagnosis, treatment and care of various rare diseases where therapy is available, those guidelines serve as a basis for the individual decision on the reimbursement of high valued orphan drugs to Hungarian rare disease patients.

Orphan medicinal product incentives

No specific activity reported.

Orphan medicinal product market availability situation

At the beginning of 2011 in Hungary, 69 of the 74 orphan medicinal products were available for sale. In 2012 the situation of orphan medicinal products in Hungary was as follows: 72 products were registered, 4 orphans are not available, 7 are withdrawn from the market for some reason.

Orphan medicinal product market availability situation

The orphan medicinal products on the market in Hungary are: Afinitor™ (Everolimus), Aldurazyme™ (Laronidase), Arzerra™ (Ofatumumab), Atriance™ (Nelarabine), Busilvex™ (Busulfan (Intravenous use), Carbaglu™ (N-carbamyl-L-glutamic acid), Casost™ (Aztreonam lysinate inhalation use), Ceplene (Histamine dihydrochloride), Cystadane™ (Betaine anhydrous), Diacon™ (Striperentol), Dudopa™ (Levodopa/Carbodopa gastrointestinal use), Elaprase™ (Iduronate-2-sulfatase) , Evoltra™ (Clofarabine), Exjade™ (Deferasirox), Fabrazyme™ (α-Galactosidase A), Hirazy™ (Icatibant acetate), Firdapse™ (Amifampridine), Giolan™ (INN-5-aminolevulinic acid hydrochloride), Glivec™ (Imatinib mesilate), Ilaris™, Increlex™ (Mecasermin rinfabate), Inovelon™ (Rufinamide), Ixaro™, Kuvan™ (Sapropterin dihydrochloride), Litak™ (Cladribine), Lysodren™ (Mitotane) , Mepact™ (Muramyl Tripeptide Fosfhatid Etanolamin), Mozobil™ (Plerixafor), Myozyme™ (Recombinant human acid α-glucosidase), Naglazyme™ (N-acetyl galactosamine 4-sulfatase), Nexavar™ (Sorafenib tosylate), Nexavar™ (Sorafenib tosylate) , Nplate™, Nymusa™ (Caffeine citrate), Onsenal™ (Celecoxib) , Orfadin™ (Nitrisinone), Pedea™ (Ibuprofen), Photobarr™ (Porfimer for photodynamic therapy), Prialt™ (α-Galactosidase A), Replagal™ (α-Galactosidase A), Revatio™ (Sildenafil citrate), Revlimid™ (Lenalidomide), Revolade™ (Eltrombopag olamin), Savene™ (Dexrazoxane), Siklos™ (Hydroxyurea), Solliris™ (Eculizumab), Somavert™ ( Pegvisomant), Sprycel™ (Dasatinib), Tasigna™ (Nilotinib), Tasigna™ (Nilotinib), Tepadin™ (Thiotepa), Thalidomide Celgene™ (Thalidomide), Thelin™ (Sitaxentan), Torisel™ (Temsirolimus), Tracleer™ ( Bosantan), Trisenox™ (Arsenic trioxide), Ventavis™ (Iloprost), Vidaza™ (Azacitidin) , Vidaza™ (Azacitidin), Volibris™ (Ambrisentan), Votrient (Patorma)™ (Pazopanib hydrochloride), Wilzin™ (Zinc acetate dihydrate), Xagrid™ (Anagrelide Hydrochloride), Yondelis™ (Ecteinascidin 743), Yondelis™ (Trabectedin), Zavesca™ (Miglustate).

Orphan medicinal product pricing policy

The OEP does not have a direct impact on pricing.

Orphan medicinal product reimbursement policy

According to the Inventory of Community and Member States’ incentive measures to aid the research, marketing, development and availability of orphan medicinal product¹⁰, “the Hungarian Government promotes the use of orphan medicinal products for specific patients by means of special financial arrangements .

“Some rare conditions (such as Fabry disease or adult-type chronic myeloid leukaemia - CML), the National Health Insurance Fund (OEP) provides standard price-support for the medicinal products in a predetermined

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¹⁰ Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products. 2005 Revision (p15)
manner. In this case the patient’s contribution is negligible or 0%. In other cases, support for the orphan medicinal products imported for patients’ treatment can be provided on application under a special equity procedure laid down by law. The OEP pays the price-support for the necessary medicinal products from earmarked resources in the outpatients’ equity fund”\(^{11}\). In most cases, support is only available via discretionary claims. There is a yearly budget for such claims managed by the OEP. The discretionary procedure takes into account the financial situation of the claimant. In 2009, 289 patients had their discretionary claims accepted. Around 13 rare diseases receive support within the framework of discretionary claims. 33 orphan medicinal products are 100% reimbursed in Hungary. The re-regulation of pharmaceutical reimbursement inclusion decisions started in 2011. The National Health Insurance Fund established the above mentioned advisory group to evaluate the applications for expensive medical treatments. The operation rules for this committee have been elaborated. A significant proportion of applications are submitted by rare disease patients’ physicians.

**Other initiatives to improve access to orphan medicinal products**

Off-label use is possible, provided that the benefits of the drugs for a certain disease are certified, but the process is highly bureaucratic.

**Other therapies for rare diseases**

No specific activity reported.

**Orphan devices**

No specific activity reported

**Specialised social services**

There are good and high quality programs in the field of early development and respite care which support patients and their families. However, these programmes do not cover the whole country. Legislation exists on care, training, integration, work help for special needs children (i.e. extra home care), however these initiatives are not available to all rare disease patients. The change of this legislation has started in favour of rare disease patients. There are measures in place to support patients who need to travel inland to access health care through an assessment of needs by the Health Insurance Fund.

The Ministry of Human Resources started to work together with HUFERDIS for a project establishing the National Habilitation, Development and Service Centre of Rare Disorders to help the social integration of rare disease patients. Several health care institutions started to change care profile during the reorganisation within the Semmelweis Plan. Some of them will be able to offer more rehabilitation and social care.

HUFERDIS joined the therapeutic recreational programs of a member association (Hungarian Williams Syndrome Association) and organised programmes for capacity building and training, networking, awareness raising, exchange of information and best practices, during a special development family camp and after. HUFERDIS is also participating in the EUCERD Joint Action activities concerning Specialised Social Services.

\(^{11}\) Inventory of Community and Member States’ incentive measures to aid the research, marketing, development and availability of orphan medicinal products. 2005 Revision (p15)
DEVELOPMENT OF RARE DISEASE ACTIVITIES IN 2013 IN HUNGARY

National plan/strategy for rare diseases and related actions
By the end of 2012 the National Plan for Rare Diseases was submitted to the Ministry of Health. The fourth Hungarian Europlan conference on 25-26 October 2013, organised by with the participation of the Ministry of Health, examined the document and the budgetary question: the conference was a lively one thanks to the signing of National Plan for Rare Diseases by the Minister of Human Resources. The National Plan for Rare Diseases has now been approved (at the end of 2013)\(^{12}\) and there is an elaborated budgetary plan for the 7 year strategy. The approval of the budget proposal is expected in 2014 in order to finalise the plan. The National Plan for Rare Diseases 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 National Plan for Rare Diseases, including the allocation of a dedicated budget. The implementation of National Plan for Rare Diseases is jeopardized by some uncertainty caused by the prospective MP election, therefore the enhancement of national and international advocacy work is continuously necessary. Beside the National Plan for Rare Diseases the Ministry plans rare disease specific communications in project “Development of public health communication” supported by Cohesion Fund.

In 2012 an expert group was also established at the Ministry of Health to identify the technical specification for a pilot study concerning the introduction of Orphacodes into hospital and healthcare centre records and the study started in 2013.

The formation of a RD help line system, together with the development of rare disease emergency cards was also discussed in the 4\(^{th}\) Europlan conference. However, no steps have been made yet.

Centres of expertise
NRDC initiated a collaboration with the National Health Insurance Fund for the listing and transparent accreditation of centres of expertise, hospitals, and laboratories working in the field of rare diseases taking into account existing resources and their concentration, as well as eliminating parallelism and formalising existing informal relations and determining patients’ pathways. The research project final report is expected to be published in 2014.

The legal base for establishing rare diseases expert centres had been set up at the end of 2013 by the Ministry of Health. The details of the designation process are to be elaborated.

Registries
The National Register of Congenital Anomalies (VRONY) operates countrywide according to the EUROCAT protocol. The former case definition of VRONY (congenital anomalies diagnosed from conception to the end of the first year of the newborn) has been extended by eliminating the age limit. Consequently, all the diagnosed congenital anomalies are to be reported from 2013 in an obligatory manner. The NRDC has initiated the establishment of an overall register for rare diseases.

National alliances of patient organisations and patient representation
During 2013 HUFERDIS played an important role in of the establishment of Hungarian National Patient Forum, resulting in the election of its president as the Coordinator of the Forum. The federation was also a funder member of the new Hungarian Alliance of Patient Organisations (HAPO).

Sources of information on rare diseases and national help lines

\textit{Helpline}

HUFERDIS started a process to establish the necessary help line system, by applying for a Norwegian grant.

National rare disease events in 2013
HUFERDIS, the Hungarian rare disease alliance, organised a number of events to mark Rare Disease Day in Hungary in both Budapest and Pecs.

In the central event, there were parallel professional meetings in Budapest. A professional conference brought together patient associations, healthcare professionals, pharmacists, technical diagnostic support specialists and also decision makers to share their opinions and ideas on how to act in the field. There was a roundtable discussion about the National Plan for Rare Diseases, updating the authorisation procedures concerning rare diseases. An overview of diagnostic opportunities and new horizons was given, summarising the experiences and finished projects from last year’s efforts. There were several useful programmes for families as well, including patient organisation presentation booths, poster sessions, a press conference, playground, crafts and entertainment. HUFERDIS also organised a Rare Beauties exhibition to mark the Day in an artistic fashion. A Solidarity Walk of 1.7 km through the City Park was also organised bringing together patients and those supporting them.

As a joint effort of the Department of Medical Genetics of the University of Pécs, the Éltes Mátýás School for Children with Special Needs, and the Pécs Gallery at the Zsolnay Cultural Center, an information day for all rare disease patients in the region was held on 23 February 2013. The Zsolnay Cultural Centre, located on the site of the famous Zsolnay porcelain factory in Pécs, proved to be an attractive location for the patients, families, caregivers, and local people interested in rare diseases. The backbone of the program consisted of musical performances by the students of the Éltes Mátýás School, many of whom are affected by rare diseases themselves, and information stands of various Hungarian rare disease patient organisations and caregivers from the region. Speeches given by the Dean of the Medical Faculty, the Director of the Clinical Centre, and the Vice Rector of the University, stressed the role of the University and its academic resources in the region’s rare disease care and research.

As previously mentioned, the fourth Hungarian Conference on Rare Diseases was organised by HUFERDIS on 25 October 2013 as part of the Europlan initiative, when the Hungarian National Plan for Rare Diseases was publicly presented for the first time. It is a strategy of health policy from 2014-2020 for RD.

Research activities and E-Rare partnership
E-Rare
Hungary participated in the 5th Joint Transnational Call in 2013 although no Hungarian teams participate in the selected projects.
LIST OF CONTRIBUTIONS

Contributions in 2010
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Gabor Pogany (President of HUFERDIS, member of EUCERD)

Contributions in 2011
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- Europlan Hungarian National Conference Final Report
  http://europlan.rirosz.hu/eurotery-iv-konferencia/dokumentumok/I_Europlan%20report%20of%20Hungary.pdf?attredirects=0&amp;d=1
- Hungarian National Conference resources
  http://europlan.rirosz.hu/home
- Hungarian National Plan for Rare Diseases (in English)

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13 The contributors and validators of the report have contributed information which is accurate to the best of their knowledge. However, readers should take note that the contents of this report are illustrative and not exhaustive.

14 All websites and documents were last accessed in May 2014.