2013 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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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 Organization
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 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 2012. 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 European Union Committee of Experts 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 2012
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

Each part contains the following description of the methodology, sources and validation process of the entire report, and concludes with 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.
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 is assembling 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 designated a competent, responsible Head of the expert committee, authorised to make decisions, to lead the development of the National Plan.

The former IT centre facilities 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.
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 (HUFERDIS).

At the Europlan Hungarian national conference on rare diseases\(^3\), organised by HUFERDIS 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 Diseases\(^3\) (16-17 November 2012) was organised by HUFERDIS, 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 action plan for rare diseases.

By the end of 2012 the plan was submitted to the Ministry of Health. The legal status of the document and budgetary considerations are still to be considered. The national conference, organised by HUFERDIS with the participation of the Ministry of Health examined the document and the budgetary question. It was hoped that the text of the plan will be finalised by the middle of 2013. The plan includes a proposal for the designation of centres of expertise in accordance with the EUCERD Recommendations. 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 care and healthcare centre records.

**Centres of expertise**

There are currently no officially approved centres of expertise in Hungary, although around 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.

Two main factors are to be considered for the designation of Hungarian national centres of expertise: 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 2013.

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.

**Registries**

The National Register of Congenital Anomalies (VRONY) operates countrywide according to the EUROCAT protocol. The NRDC has initiated the establishment of an overall register for rare diseases. Currently, the

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2. [http://europlan.rirosz.hu/](http://europlan.rirosz.hu/)
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 Urgență 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 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 40 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 is now the 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, 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 reorganization 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, etc. To foster the opinion of patient representatives on future European policies for rare diseases, or to collect their views on

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1 Information extracted from the Orphanet database (December 2012): an update has been provided to the coordinating team which is being entered into the database.
existing ones, HUERFDIS participated on the European POLKA project coordinated by EURORDIS. HUERFDIS started new cooperation with other international organizations as well, including DIA, EPF, EPHA, ECOP, ISOQOL.

**Sources of information on rare diseases and national help lines**

**Orphane activities in Hungary**

Since 2004 there is a dedicated Orphanet team in Hungary, initially hosted by the University of Pécs. 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 language.

**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.

**Other sources of information**

Some websites, maintained by the government ([www.gyemszi.hu](http://www.gyemszi.hu), [www.oefi.hu/aboutus.htm](http://www.oefi.hu/aboutus.htm)), have limited information concerning rare diseases. Scientific societies ([www.mhgt.hu](http://www.mhgt.hu)), non-governmental expert groups ([www.betegmagzat.hu](http://www.betegmagzat.hu)) and market-based organisations ([www.webdoki.hu](http://www.webdoki.hu)) have web based services for patients. The only other significant rare disease-specific website is the homepage of HUERFDIS ([www.rirosz.hu](http://www.rirosz.hu)). Several member associations of HUERFDIS have also detailed specific websites for a given rare disease.

**Good practice guidelines**

Guidelines related to rare diseases have 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 HUERFDIS has thus developed a new rare disease protocol to properly communicate a diagnosis.

**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

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4 [http://www.orpha.net/national/HU-HU/index/honlap/](http://www.orpha.net/national/HU-HU/index/honlap/)
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 2012
HUFERDIS, the Hungarian rare disease alliance, organised a number of events to mark Rare Disease Day in Hungary on 25 February 2012 in Budapest. As usual, many parallel programmes was arranged: expert conference, poster section, games and handicrafting for children, entertainment programmes, “Rare Beauties” Art Exhibition, concerts, press conference, all-day exhibition of the HUFERDIS member associations. Many videos and report are available6). The main breakthrough of the programme was the section dealing with social problems of rare diseases for the first time: four leaders of the Ministry of Social Affairs participated and gave talks6.

For the second time, Rare Disease Day in Hungary was organised in two cities simultaneously. In addition to the events in the capital, Budapest, events were also held in the university town of Pécs, in southwest Hungary. The initiative to organise Rare Disease Day events at multiple locations allows Hungarian rare disease patients to participate in the events nearer to their hometowns. As a joint effort of the Department of Medical Genetics of the University of Pécs, the Éltes Mátyás 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 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 organizations and caregivers from the region. The guest of honour at this year’s scientific program was Daria Julkowska, program coordinator of E-Rare2, the ERA-Net on Rare Disease Research Funding. Hungary joined the E-Rare2 network in 2010, and was able to participate through contributions from the University of Pécs, and the Hungarian National Research Fund. Daria Julkowska gave both a broad overview on E-Rare2 activities and achievements, as well a model of rare disease national coordination, using the example of the French Rare Disease Foundation. The broad interest in rare diseases, and the success of the local events strengthened the organizers in their ambition to continue this tradition, and even inspire other regions in Hungary to organise local events on future Rare Disease Days.

The third Hungarian Europlan Conference on Rare Diseases7 (16–17 November 2012) was organised by HUFERDIS, 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 action plan for rare diseases.

Hosted rare disease events in 2012
Amongst the events hosted by Hungary and announced in OrphaNews Europe was the 8th International Society for Newborn Screening European Regional Meeting (4–6 November 2012, Budapest).

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 applications8. 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

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7 In addition, some videos were released by Bach Rezső Bach: http://youtube.be/JKiGdF-fvHg, and Gábor Pogány: http://youtube.be/Z-i8R_Czu4 Hungary (Society of Aiders and People suffering from Neurofibromatosis).
8 http://europlan.rirosz.hu/
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.

The IT centre of the NRDC elaborated the on-line registration system for health care providers, laboratories, research programs and patient groups related to rare diseases. This data collection is in line with the Orphanet data collection standards. The system has been launched and the primary database is used to contribute to the Orphanet database.

**Participation in European research projects**

Hungary participates, or has participated, in European rare disease research projects including: BNE, EUROBONET, EUROGENTEST, EUROPEAN LEUKEMIA NET, EUROSCA, EUROWILSON, GENESKIN, NMD-CHIP, TREAT-NMD, SCRIN-SILICO, OPTATIO, BBMRI and SIOPEN-R-NET.

**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.

**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.

**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 HUFEERDIS 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 according to the Department of Rare Diseases in University of Debrecen. This institution implemented in Hungary the survey of the Rare Disease National Alliances & EURORDIS on Patients’ Access to Orphan Medicinal Products in Europe. Most of orphan medicinal products are available via centres assigned by the National Health Insurance Fund.

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9 http://www.molneur.eoldal.hu/cikkek/english
10 http://semmelweis-egyetem.hu/genomikai-medicina/
11 This section has been written using the Inventory of Community and Member States’ incentive measures to aid the research, marketing, development and availability of orphan medicinal products. 2005 Revision (p15)
The orphan medicinal products on the market in Hungary are: Afinitor™ (Everolimus), Aldurazyme™ (Laronidase), Arzerra™ (Ofatumumab), Atriance™ (Nelarabine), Busilvex™ (Busulfan (Intravenous use), Carbeglu™ (N-carbamyl-L-glumatic acid), Caysston™ (Aztreonam lysinate inhalation use), Ceplene (Histamine dihydrochloride), Cystadane™ (Betaine anhydrous), Diacomit™ (Stri-8methyl), Dudopa™ (Levodopa/Carbidioma gastrointestinal use), Elaprase™ (Iduronate-2-sulfatase), Evoltra™ (Clofarabine), Exjade™ (Deferasirox), Fabrazyme™ (α-Galactosidase A), Fira-zyr™ (Icatiban acetate), Firdapse™ (Amifampridine), Gliolan™ (INN-5-aminovaleric acid hydrochloride), Glivec™ (Imatinib mesilate), Ilaris™, Increlex™ (Mecasermin), Kuvan™ (Sapropterin dihydrochloride), Litak™ (Cladribine), Lyso-dren™ (Mitotane), Mepact™ (Muramyl Tripeptid Fosfatidil 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™ (Nitisinone), Pedea™ (Ibuprofen), Photobarr™ (Porfimerum photodynamic therapy), Prialt™ (α-Galactosidase A), Replagal™ (α-Galactosidase A), Revatio™ (Sildenafil citrate), Revlimid™ (Lenalidomide), Revolade™ (El trombopag olamine), Savene™ (Dexrazoxane), Siklos™ (Hydroxypurea), Soliris™ (Eculizumab), Somavert™ ( Pegvisomant), Sprycel™ (Dasatinib), Tasigna™ (Nilotinib), Tasigna™ (Nilotinib), Tepadina™ (Thiotepa), Thalidomide Celgene™ (Thalidomide), Thelin™ (Sitaxentan), Torisel™ (Temsirolimus), Tracleer™ (Bosentan), Trisenox™ (Arsenic trioxide), Ventavis™ (Iloprost), Vizada™ (Azacamizdin), Vizada™ (Azacitidine), Volibris™ (Ambrisentan), Votrient (Patorma)™ (Pazopanib hydrochloride), Wilzin™ (Zinc acetate dihydrate), Xaglith™ (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 products, 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 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 outpatient’s equity fund. In most cases, support is only available via discretionnal claims. There is a yearly budget for such claims managed by the OEP. The discretionnal procedure takes into account the financial situation of the claimant. In 2009, 289 patients had their discretionnal claims accepted. Around 13 rare diseases receive support within the framework of discretionnal 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 an advisory group to evaluate the applications for expensive medical treatments. The operation rules for this committee have been elaborate. 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.

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12 Inventory of Community and Member States’ incentive measures to aid the research, marketing, development and availability of orphan medicinal products. 2005 Revision (p15)

13 Inventory of Community and Member States’ incentive measures to aid the research, marketing, development and availability of orphan medicinal products. 2005 Revision (p15)
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.

DEVELOPMENT OF RARE DISEASE ACTIVITIES IN 2012 IN HUNGARY

National plan/strategy for rare diseases and related actions
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 Diseases14 (16-17 November 2012) was organised by HUFERDIS, 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 action plan for rare diseases.

By the end of 2012 the plan was submitted to the Ministry of Health. The legal status of the document and budgetary considerations are still to be considered. The national conference, organised by HUFERDIS with the participation of the Ministry of Health examined the document and the budgetary question. It was hoped that the text of the plan will be finalised by the middle of 2013. The plan includes a proposal for the designation of centres of expertise in accordance with the EUCERD Recommendations. 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.

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 2013.

Sources of information on rare diseases and national help lines
Orphanet activities in Hungary
There is a Orphanet national website launched in 2012, in the Hungarian language15.

Training and education initiatives
The Department of Medical Genetics at the University of Pecs 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.

14 http://europlan.rirosz.hu/
15 http://www.orpha.net/national/HU-HU/index/honlap/
The institutions and clinics participating in rare disease care constitute a Rare Disease Network of the University of Pécs established in February 2012.

National rare disease events in 2012
HUFERDIS, the Hungarian rare disease alliance, organised a number of events to mark Rare Disease Day in Hungary on 25 February 2012 in Budapest. As usual, many parallel programmes was arranged: expert conference, poster section, games and handcrafting for children, entertainment programmes, “Rare Beauties” Art Exhibition, concerts, press conference, all-day exhibition of the HUFERDIS member associations. Many videos and report are available[16]. The main breakthrough of the programme was the section dealing with social problems of rare diseases for the first time: four leaders of the Ministry of Social Affairs participated and gave talks[17]. For the second time, Rare Disease Day in Hungary was organised in two cities simultaneously. In addition to the events in the capital, Budapest, events were also held in the university town of Pécs, in southwest Hungary. The initiative to organise Rare Disease Day events at multiple locations allows Hungarian rare disease patients to participate in the events nearer to their hometowns. As a joint effort of the Department of Medical Genetics of the University of Pécs, the Éltes Mátyás 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 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 organizations and caregivers from the region. The guest of honour at this year’s scientific program was Daria Julkowska, program coordinator of E-Rare2, the ERA-Net on Rare Disease Research Funding. Hungary joined the E-Rare2 network in 2010, and was able to participate through contributions from the University of Pécs, and the Hungarian National Research Fund. Daria Julkowska gave both a broad overview on E-Rare2 activities and achievements, as well a model of rare disease national coordination, using the example of the French Rare Disease Foundation. The broad interest in rare diseases, and the success of the local events strengthened the organizers in their ambition to continue this tradition, and even inspire other regions in Hungary to organise local events on future Rare Disease Days.

The third Hungarian Europlan Conference on Rare Diseases[18] (16-17 November 2012) was organised by HUFERDIS, 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 action plan for rare diseases.

Hosted rare disease events in 2012
Amongst the events hosted by Hungary and announced in OrphaNews Europe was the 8th International Society for Newborn Screening European Regional Meeting (4-6 November 2012, Budapest).

Research activities and E-Rare partnership
E-Rare
Hungary did not participate in the 4th Joint Transnational Call in 2012.

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.

Orphan medicinal products[19]
Orphan medicinal product committee
There is a new committee for the evaluation of reimbursement inclusion decisions in case of high-value 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,

[17] In addition, some videos were released by Bach Rezső: http://youtu.be/KIGdf-fvHg, and Gábor Pogány: http://youtu.be/Z-i8R_CriU4 Hungary (Society of Aiders and People suffering from Neurofibromatosis).
[19] This section has been written using the Inventory of Community and Member States’ incentive measures to aid the research, marketing, development and availability of orphan medicinal products. 2005 Revision (p15)
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 reimbursement policy**
The National Health Insurance Fund established an 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.

**Specialised social services**
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.
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- Hungarian National Conference resources
  http://europlan.rirosz.hu/euroterv-ii-konferencia-1/az-elhangzott-eloadasok

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20 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.

21 All websites and documents were last accessed in May 2013.