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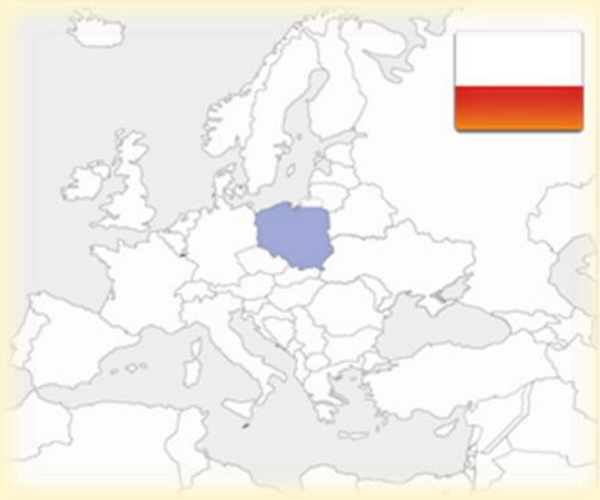
2012 REPORT ON THE STATE OF THE ART OF RARE DISEASE ACTIVITIES IN EUROPE OF THE EUROPEAN UNION COMMITTEE OF EXPERTS ON RARE DISEASES



STATE OF THE ART OF RARE DISEASE ACTIVITIES IN POLAND

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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

General

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 (Iceland, Switzerland, Norway)
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

Pilot European Reference Networks

Dyscerne - European network of centres of expertise for dysmorphology
ECORN-CF - European centres of reference network for cystic fibrosis
Paediatric Hodgkin Lymphoma Network - Europe-wide organisation of quality controlled treatment
NEUROPED - European network of reference for rare paediatric neurological diseases
EUROHISTIONET - A reference network for Langerhans cell histiocytosis and associated syndrome in EU)
TAG - Together Against Genodermatoses – improving healthcare and social support for patients and families affected by severe genodermatoses
PAAIR - Patients' Association and Alpha-1 International Registry Network
EPNET - European Porphyria Network - providing better healthcare for patients and their families
EN-RBD -European Network of Rare Bleeding Disorders
CARE-NMD -Dissemination and Implementation of the Standards of Care for Duchenne Muscular Dystrophy in Europe project
ENERCA - European network for rare and congenital anaemia – Stage 3

GENERAL INTRODUCTION TO THE REPORT ON THE STATE OF THE ART OF RARE DISEASE ACTIVITIES IN EUROPE OF THE EUROPEAN UNION COMMITTEE OF EXPERTS ON RARE DISEASES

The 2012 Report on the State of the Art of Rare Disease Activities in Europe 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 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 2011. 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 either the European Commission, its Agencies or national health authorities.

The report is split into five parts:

Part I: Overview of rare disease activities in Europe
Part II: Key developments in the field of rare diseases in 2011
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

Each part contains a 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 Poland. 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[1].

[1] http://www.eucerd.eu/upload/file/Reports/2012ReportStateofArtRDActivities.pdf

RARE DISEASE ACTIVITIES IN POLAND

Definition of a rare disease

In Poland, there is no official definition for rare diseases; however the definition from the regulation (EC) No. 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products of a prevalence of no more than 5 in 10'000 individuals is widely used amongst stakeholders. In Poland this equates to less than 1'900'000 patients (around 5% of the population).

National plan/strategy for rare diseases and related actions

Although in 2011 a national plan or strategy for rare diseases was not yet adopted, significant steps have been undertaken towards the goal of adopting this important document in order for Poland to meet the EU Council Recommendation, hopefully by the end of 2012.

The existing National Health Program covers some issues of the rare diseases e.g. National Program Against Cancer, which is focused on improving early diagnosis and treatment of cancer, a majority of them being rare diseases. There is currently no specific budget dedicated for rare diseases within the framework of the entire health care system. This does not mean that the treatment of rare diseases is ignored by the Polish health care system. All diagnosed cases of rare diseases are treated in the framework of general health care, and in cases when reimbursement of orphan medicinal products is decided, several therapeutic programs with the use of these drugs are in place. The level of reimbursement of orphan medicinal products in the therapeutic programs on rare diseases in 2011 was around €50 million.

The Rare Disease Task Force was established via an order of the Minister of Health as his advisory body in 2008. The group consisted of: the Undersecretary of the State as its leader, representatives of some Departments of Ministry of Health, Office for Registration of Medicinal Products, Medical Devices and Biocidal Products, Agency for Health Technology Assessment in Poland, National Health Fund, patient organisations, pharmaceutical industry and scientific experts. The main tasks of the Team were to present opinions on rare diseases policy and reimbursement of orphan medicinal products.

The setup of the Rare Disease Team changed in July 2011. The Minister of Health recognised rare diseases as a much broader problem, not only limited to orphan medicinal products, thus it was necessary to create a better tailored Task Force with competence also in healthcare system organisation and doctors' education. According to the new order of the Minister of Health the new Task Force leader was chosen based on his experience in the healthcare management rather than the official position. The sole purpose of the reorganised Task Force is to prepare a National Plan on Rare Diseases (NPRD). In autumn 2011 a new setup of this advisory body was formally constituted to help with the elaboration of a plan, in collaboration with patient organisations, due to be launched by the end of 2012. Due to the new Order of the Minister of Health, the group was reconstituted, and now it includes the following members chaired by a representative of the Ministry of Health: four experts in the field of rare diseases including representative of umbrella patient organisations, representatives of Poland in EU institutions in the field of rare diseases, Directors of the Departments at the Ministry of Health and representatives of National Health Fund (the Payor).

A National Conference on Rare Diseases[2] organised by the Polish Cystic Fibrosis Foundation MATIO and the National Forum for the Therapy of Rare Diseases on 22 October 2010 in Krakow, using the recommendation of Europlan evaluation model, led in 2011 to the initiation of broad, multi-session work on the draft of national plan for rare diseases.

On 7-8 December 2011 the conference "Partnership for the National Plan for Rare Diseases", organised by National Forum for the Therapy of Rare Diseases was held in Warsaw. It was agreed during this conference that a patient's input to the draft concerning a National Plan on Rare Diseases should be prepared by the end of February 2012, based on data from the working-groups, and in accordance with the Europlan project. The draft will encompass different areas and aspects of rare diseases, such as classification and rare diseases registry; diagnostics; medical care; integrated social support; and information and education. Once prepared, the document will be presented to the Polish Ministry of Health for further discussion and elaboration.

[2] http://www.europlan.org.pl/ and http://download.EURORDIS.org/europlan/2_EUROPLAN_Guidance_Documents_for_the_National_Conference/final_report_poland_europlan.pdf

Based on the above mentioned initiative, with the support of the Chairman of the ministerial Rare Diseases Task Force as well as the Children's Memorial Health Institute, the series of working meetings were organised in order to elaborate the final drafts of each mentioned area of the future national plan. During - these several working sessions held in late 2011, it was decided that the working document "The systemic assumptions for the development of the National Plan for Rare Diseases" (a joint initiative of the National Forum for Therapy of Rare Diseases and broad rare diseases medical/scientific community) will be submitted directly to the Rare Diseases Task Force at the Polish Ministry of Health for further official elaboration of the future governmental RD National Plan[3]. The entire initiative was launched following the EU Council Recommendation on the involvement of patients and their representatives in the political process and promoting the activities undertaken by rare diseases patient groups and associations.

Centres of expertise

In Poland, the health care of patients with rare diseases is not organised in a specific framework and there are no official centres of expertise for rare diseases. Around 10-15 centres have a reputation for expertise in a given field and conducts diagnostics and treatment to different extents. There is a national coordinating centre for metabolic rare diseases at the Children's Memorial Health Institute in Warsaw with links to regional centres. Significant progress has been made as a result of the European Project of Centres of Excellence "PERFECT" *QLG1-CT-2002-90358.* The grant programme included problems associated with rare paediatric diseases in the field of genetics, metabolism, gastroenterology, cardiology, immunology and oncology.

Pilot European Reference Networks

Polish teams participated/participate in the following European Reference Networks for rare diseases: Dyscerne, ECORN CF, ENERCA, EPI/EPNET, EUROHISTIONET, PAAIR, European Network of Paediatric Hodgkin's Lymphoma and Care-NMD.

Registries

In Poland there are number of well constructed registries such as the National Registry of Paediatric Cancers and the Polish Registry of Congenital malformations. Poland takes part in the European registries such as EIMD, ERCUSYN, TREAT-NMD, EUROCARE CF, EUROWILSON, EUROGLYCAN, EURO-WABB, SCNIR, RARECARE, and EUROCAT.

Neonatal screening policy

There is a national newborn screening program for phenylketonuria, congenital hypothyroidism, cystic fibrosis and congenital deafness. This is coordinated by the Institute for Mother and Child in Warsaw. An additional 19 metabolic disorders diagnosed by tandem mass spectrometry (MS/MS) are available in some regions (mazowieckie, podlaskie, warmińsko-mazurskie, lubelskie, pomorskie, kujawsko-pomorskie, wielkopolskie and lubuskie provinces, around 60% of the Polish population) through a Ministry of Health financed programme (till 2014).

Genetic testing

There are about 35 laboratories (public and non-profit) offering testing for rare diseases. Most of them follow external quality control assessments. Diagnostic procedures which are performed in these labs mainly concentrate on: specific genetic diseases (chromosomal and monogenic disorders), metabolic diseases (selective screening testing for inborn errors of metabolism, lysosomal storage disorders, neuromuscular and haematological diseases, defects in metabolism of carbohydrates, fats, amino acids, purines and pyrimidines, neurotransmitters, as well as disturbances of calcium-phosphate metabolism and energetic processes).

There is also possibility of performing testing abroad. If a specific disease is not diagnosed in Poland the National Health Found may cover such diagnostic procedures, however the regional consultant in genetics must give their approval for such a demand.

Diagnostic tests are registered as available in Poland for 182 genes and an estimated 213 diseases in the Orphanet database[4].

[3] The list of parties involved in the preparation of the systemic assumptions as well as results of their common work may be reached at www.rzadkiechoroby.pl

[3] Information extracted from the Orphanet database (September 2011).

National alliances of patient organisations and patient representation

The National Forum for the Therapy of Rare Diseases – ORPHAN, created in 2005, serves as national alliance for rare disease patients' organisations in Poland. As the umbrella of rare disease associations, the Forum groups together the 22 rare diseases patient organisations and it strengthens the cooperation of rare disease patient organisations at the national level. In autumn 2011 the representative of the National Forum was appointed by Minister of Health as the member of the ministerial Rare Disease Task Force, representing the single unanimous voice and position of Polish rare diseases patient organisations. More information about the goals, membership and activities of The National Forum for the Therapy of Rare Diseases are published online[5].

Sources of information on rare diseases and national help lines

Orphanet activities in Poland

Since 2006 there is a dedicated Orphanet team in Poland, currently hosted by the Children's Memorial Health Institute which in 2010 was designated by the Ministry of Health as a partner for the Orphanet Europe Joint Action. 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 Poland for entry into the Orphanet database.

Since April 2011 the Orphanet Poland team maintains a national Orphanet Poland national website[6]. In order to improve access to information on rare diseases, orphan medicinal products and Orphan in Poland, the Polish Orphanet team has translated the documents concerning Orphanet's activities (leaflets), created lists of Polish associations/expert clinics/diagnostics centres and is developing Polish versions of rare disease abstracts. All this information is available on the Orphanet website.

Official information centre for rare diseases

There is no official information centre on rare diseases in Poland other than Orphanet.

Helpline

There is currently no national help line for rare diseases. Some patient organisations run help lines for specific rare diseases.

Other sources of information

No specific activity currently reported.

Good practice guidelines

No specific activity currently reported.

Training and education initiatives

There are some rare disease specific training sessions for professionals. The best known are dysmorphology meetings organised by the Children's Memorial Health Institute (CMHI) in Warsaw, which have been organised on a regular basis for 4 years now, initially as a part of the Dyscerne project. The Department of Medical Genetics of CMHI have organised the course for paediatricians concerning on advances of molecular biology in paediatrics. In addition, some metabolic rare diseases are also presented and discussed during sessions organised every year by the Medical Centre for Postgraduate Education in Warsaw dedicated to the training of physicians or professionals being trained specific medical speciality (paediatricians, neurologists and child neurologists, clinical geneticists etc.).

National rare disease events in 2011

In Poland, Rare Disease Day 2011 was marked by an event at the famous King's Palace on the lake in Lazienki Park in Warsaw. The Organising Committee, created for the event, consisted of 16 Polish rare diseases patients' support groups who created a web page (www.dzienchorobrzadkich.pl) and a Facebook page for the Polish Rare Disease Day. Altogether over 150 people took part: patients, their families and friends, health professionals, parliament and Ministry of Health representatives, the Orphanet-Poland coordinator and the media attended this meeting. The Minister of Health Ms. Ewa Kopacz gave a welcome address and she then presented the government plans for dealing with the issue of rare diseases at national level. The participants heard about help which should be provided for all affected families, the national strategy for rare diseases,

[5] www.rzadkiechoroby.pl/np

[6] www.orpha.net/national/PL-PL

intensive work in this field starting this year and Orphanet activities. After an official session, consisting of a few important speeches by the hosts, all of the participants went outside where many white balloons with the Rare Disease Day logo were released. A private 'wish list' was attached to the string of each balloon. Copies of these wishes were given to the Minister of Health as the patients' petition to the Polish government. The event was reported on in the press, local radio, national radio and TV with a number of interviews and talks.

On 7-8 December 2011 the conference "Partnership for the National Plan for Rare Diseases" was held in Warsaw. This meeting was organised by "Orphan", the National Forum for Rare Disease Therapy, with participation from representatives of the Ministry of Health, families, patient societies, clinical experts, and the Children's Memorial Health Institute's (CMHI) group on rare diseases. The conference was a symbolic, official launch of the work toward developing and adopting a National Plan for Rare Diseases, based on a formula of social consultations. During the first day the current state of Polish patients with rare diseases (RDs) was discussed. The second day took place in the CMHI with the participation of the patient group representatives and the clinical experts who work with rare diseases. The main aim of the discussion was the exchange of experiences and views on the care and treatment of patients and the creation of working-groups, including clinical experts from the Polish reference centers. It was agreed that a patient's input to the draft concerning a National Plan on Rare Diseases will be prepared by the end of February 2012, based on data from the working-groups, and in accordance with the Europlan project. The draft will encompass different areas and aspects of rare diseases, such as classification; diagnostics; medical care; integrated social support; and information and education. Once prepared, the document will be presented to the Polish Ministry of Health for further discussion. On 20 October 2011 a working meeting took place in Warsaw with the participation of the representatives of European paediatric oncology centres and the Ministers of Health of the European countries. It was dedicated to the care of children with cancer, which are all rare diseases. Among other issues, the participants discussed problems related to the establishment of the European Standards of Care for Children with Cancer. The conference was organised by the Polish Ministry of Health in cooperation with the European Society for Paediatric Oncology (SIOPE) in Brussels, and in relation to the Ministry's participation in the European Partnership for Action Against Cancer.

Other rare disease related events included a Dysmorphology meeting in the Children's Memorial Health Institute (Warsaw, 7 June 2011 and Lublin 14 October 2011), V Polish Conference on the Problems of diagnosis, rehabilitation and development of a disabled child", Lublin (13-15 October 2011), "Advances of genetics in pediatric medicine" Course, Warsaw (30 November - 2 December 2011).

Hosted rare disease events in 2011

The following events have been hosted by Poland in 2011: 4th conference of the Eastern European Metabolic Academy (EEMA), Warsaw (26-27 September 2011).

Research activities and E-Rare partnership

National research activities

There is no research programme specifically aimed at rare diseases in Poland. Research on rare diseases is financed within different programmes for state-funded research but there are no specifically allocated funds. Around 10% of projects approved for funding being related to the field of rare diseases.

Participation in European research projects

Polish teams also participate/participated in European rare disease research projects including: EUROGLYCANET, ERNDIM, EUROCARE-CF, EUROGENTEST, EUROPEAN LEUKEMIA NET, EUROWILSON, EUROSCA, EURADRENAL, EURO-GENE-SCAN, MYELINET, NEURO.GSK3, NEUPROCF, RD PLATFORM, TB PAN-NETand SIOPEN-R-NET.

E-Rare

Poland is an observer of the E-Rare 2 project.

IRDiRC

Polish funding agencies are not yet committed members of the IRDiRC.

Orphan medicinal products

Orphan medicinal product committee

There is currently no orphan medicinal product committee in Poland. For details please refer to the section on national plans.

Accordingly to the new Reimbursement Act (in force since July 2011), the Economic Committee within the Ministry of Health takes responsibility to negotiate market conditions for products applying for reimbursement, including orphan medicinal products.

Orphan medicinal product incentives
No specific activity reported.

Orphan medicinal product market availability situation
In 2011, treatment with the following orphan medicinal products has been reimbursed: Cerezyme, Naglazyme, Elapraze, Laronidase and Myozyme (partially)

Specific information on availability of these orphan medicinal products (i.e. number of orphan medicinal products commercialised in 2011) are not available. Information on reimbursement procedures is detailed below.

Orphan medicinal product pricing policy
The Ministries of Health and of Finance decide on maximum public prices and reimbursement status on the opinion of the Medicines Management Team and a HTA recommendation from the Agency for Medical Technology Evaluations[7]. However, following the implementation of the new Reimbursement Act (July 2011), a few changes have been introduced complying with Transparency Directive. The Medicines Management Team was disbanded and the new Economic Committee takes responsibility in the negotiation process. In addition to this, the role of HTA has been strengthened.

Orphan medicinal product reimbursement policy
At the moment reimbursement of drugs in outpatient settings is based on a reasoned application of the market authorisation holder. Reimbursement of drugs in hospitals is decided through the special procedure initiated by the Minister of Health (requiring a recommendation of the President of the Agency for Health Technology Assessment in Poland).

The abovementioned system will change from 1 January 2012 according to the Act of 12 May 2011 on reimbursement of drugs (in force since July 2011), foods for special dietary use and medical devices. The new system will be unified, application based. The Minister of Health will not be able to introduce reimbursement of a new drug without prior application from the marketing authorisation holder. The application will have to contain among others HTA analyses, information on reimbursement status and pricing in other EU Member States.

Currently, drugs for some rare diseases are reimbursed through therapeutic programmes. Each year the National health Fund selects a priority list for funding through this mechanism: to be included orphan medicinal products must show budget impact and clinical effectiveness[8]. Diseases currently covered include Crohn disease, Prader-Willi syndrome, cystic fibrosis). Five drugs for ultra rare diseases are reimbursed for patients with Gaucher, MPS I, II and VI, and Pompe disease. However, in 2009, Ministry of Health decided to limit the existing available therapy for Pompe disease due to lack of proven clinical effectiveness. Unfortunately, regardless of the new clinical data proving the effectives are available since May 2010 and after many interventions made by patient community and physicians the newly diagnosed late on-set Pompe patients do not qualify for the therapy reimbursement as the National Health Fund as well as Ministry of Health were not willing to consider the change of this unjustified limitation. This has led to unequal treatment of citizens, when people diagnosed before 2009 have reimbursement granted as compared with those patients diagnosed after the changes to the therapeutic programme.

The Minister of Health issues a regulation containing a list of chronic diseases for which some drugs are available in pharmacies free of charge, for a flat-rate or partial co-payment. This list includes among others epidermolysis bullosa, phenyloketonuria and amyotrophic lateral sclerosis.

Other initiatives to improve access to orphan medicinal products
There is no official compassionate use policy. Life-saving treatment with drugs registered outside of Poland is subject to individual decisions of the Minister of Health and might be reimbursed by the President of the National Health Fund under his consent on a named-patient basis. If a company donates a drug, it is subject to taxation, which further limits potential compassionate use.

[4] *Orphan drugs in Europe : Pricing, Reimbursement, Funding and Market Access Issues,* Donald Macarthur (2011) p 113
[5] *Orphan drugs in Europe : Pricing, Reimbursement, Funding and Market Access Issues,* Donald Macarthur (2011) p 114

Orphan devices
Orphan devices are regularly presented during dysmorphological meetings, national conferences and trainings.

Specialised social services
There are no social services designed specifically for patients for rare diseases, though respite care exists in general and educational centres can provide day care for children and education for patients: these are both privately and publically funded initiatives, provided on an application basis. Some official programmes require for patients to be qualified as disabled in order to participate. Therapeutic recreational services such as camps are eligible for co-funding by the state social care (usually 30% patient co-payment). Patient organisations sometimes provide services which are financed from private funds specifically for rare diseases patients. The state finances the integration of children with special needs, via integration classes in schools.

DEVELOPMENT OF RARE DISEASE ACTIVITIES IN 2011 IN POLAND

National plan/strategy for rare diseases and related actions
Although in 2011 a national plan or strategy for rare diseases was not yet adopted, significant steps have been undertaken towards the goal of adopting this important document in order for Poland to meet the EU Council Recommendation, hopefully by the end of 2012.

The Rare Disease Task Force was established via an order of the Minister of Health as his advisory body in 2008. The setup of the Rare Disease Team changed in July 2011. The Minister of Health recognised rare diseases as a much broader problem, not only limited to orphan drugs, thus it was necessary to create a better tailored Task Force with competence also in healthcare system organisation and doctors' education. According to the new order of the Minister of Health the new Task Force leader was chosen based on his experience in the healthcare management rather than the official position. The sole purpose of the reorganised Task Force is to prepare a National Plan on Rare Diseases (NPRD). In autumn 2011 a new setup of this advisory body was formally constituted to help with the elaboration of a plan, in collaboration with patient organisations, due to be launched by the end of 2012. Due to the new Order of the Minister of Health, the group was reconstituted, and now it includes the following members chaired by a representative of the Ministry of Health: four experts in the field of rare diseases including representative of umbrella patient organisations, representatives of Poland in EU institutions in the field of rare diseases, Directors of the Departments at the Ministry of Health and representatives of National Health Fund (the Payor).

On 7-8 December 2011 the conference "Partnership for the National Plan for Rare Diseases", organised by National Forum for the Therapy of Rare Diseases was held in Warsaw. It was agreed during this conference that a patient's input to the draft concerning a National Plan on Rare Diseases should be prepared by the end of February 2012, based on data from the working-groups, and in accordance with the Europlan project. The draft will encompass different areas and aspects of rare diseases, such as classification and rare diseases registry; diagnostics; medical care; integrated social support; and information and education. Once prepared, the document will be presented to the Polish Ministry of Health for further discussion and elaboration.

Based on the above mentioned initiative, with the support of the Chairman of the ministerial Rare Diseases Task Force as well as the Children's Memorial Health Institute, the series of working meetings were organised in order to elaborate the final drafts of each mentioned area of the future national plan. During these several working sessions held in late 2011, it was decided that the working document "The systemic assumptions for the development of the National Plan for Rare Diseases" (a joint initiative of the National Forum for Therapy of Rare Diseases and broad rare diseases medical/scientific community) will be submitted directly to the Rare Diseases Task Force at the Polish Ministry of Health for further official elaboration of the future governmental RD National Plan[9]. The entire initiative was launched following the EU Council

[9] The list of parties involved in the preparation of the systemic assumptions as well as results of their common work may be reached at www.rzadkiechoroby.pl

Recommendation on the involvement of patients and their representatives in the political process and promoting the activities undertaken by rare diseases patient groups and associations.

Sources of information on rare diseases and national help lines

Orphanet activities in Poland

Since April 2011 the Orphanet Poland team maintains a national Orphanet Poland national website[10]. In order to improve access to information on rare diseases, orphan drugs and Orphan in Poland, the Polish Orphanet team has translated the documents concerning Orphanet's activities (leaflets), created lists of Polish associations/expert clinics/diagnostics centres and is developing Polish versions of rare disease abstracts. All this information is available on the Orphanet website.

National rare disease events in 2011

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Research activities and E-Rare partnership

E-Rare

Poland is an observer of the E-Rare 2 project.

[10] www.orpha.net/national/PL-PL

IRDiRC

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Orphan medicinal products

Orphan medicinal product committee

Accordingly to the new Reimbursement Act (in force since July 2011), the Economic Committee within the Ministry of Health takes responsibility to negotiate market conditions for products applying for reimbursement, including orphan medicinal products.

Orphan medicinal product pricing policy

Following the implementation of the new Reimbursement Act (July 2011), a few changes have been introduced complying with Transparency Directive. The Medicines Management Team was disbanded and the new Economic Committee takes responsibility in the negotiation process. In addition to this, the role of HTA has been strengthened.

Orphan medicinal product reimbursement policy

The reimbursement system will change from 1 January 2012 according to the Act of 12 May 2011 on reimbursement of drugs (in force since July 2011), foods for special dietary use and medical devices. The new system will be unified, application based. The Minister of Health will not be able to introduce reimbursement of a new drug without prior application from the marketing authorisation holder. The application will have to contain among others HTA analyses, information on reimbursement status and pricing in other EU Member States.

LIST OF CONTRIBUTIONS[11]

Contributions in 2010
Jolanta Sykut-Cegielska *(Children's Memorial Health Institute, Warsaw)*
Miroslaw Zielinski *(Polish National Forum on the Treatment of Rare Disorders)*
Jakub Adamski *(Ministry of Health)*

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[11] 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.
[12] All websites and documents were last accessed in May 2012. A more detailed list of sources is available in the full report: http://www.eucerd.eu/upload/file/Reports/2012ReportStateofArtRDActivities.pdf