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

STATE OF THE ART OF RARE DISEASE ACTIVITIES IN POLAND

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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 activities of the former European Union Committee of Experts on Rare Diseases and the EUCERD Joint Action 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 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 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.

Each year, there are around 15 000 downloads of the different sections of the report combined.
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).

During the several public debates on rare diseases, consideration of the adoption of an additional sub-definition of ultra-rare disease has been suggested as the necessary to be incorporated within the Polish health care system. The preliminary assumption is that the ultra-rare disease definition would be based on a prevalence of no more than 1 in 50 000 individuals.

National plan/strategy for rare diseases and related actions
By the end of 2012 a strategic document “National Plan for Rare Disease – the roadmap” was prepared and handed to the Minister of Health. A broad consultation process started, and it was scheduled for completion by June 2013 in order to meet the recommendations of the EU Council by the end of 2013.

The existing National Health Program 2007-20151 (health policy paper elaborated by the office of the Prime Minister) covers some issues of the rare diseases in broad understanding of public health. Among the 8 strategic goals addressing main causes of mortality in Poland there is the mention of rare diseases, but they are not exclusively designated. There is currently no specific budget dedicated for rare diseases within the framework of the entire health care system, however specific health interventions are reimbursed in project mode (e.g. therapeutic programs). This does not mean that the treatment of rare diseases is ignored. All diagnosed rare disease cases are treated in the framework of health care system. When reimbursement of orphan medicinal products is possible (within the basket of reimbursed services), therapeutic programs are in place, with an annual budget (2012) equivalent to €33,5 million (same as 2011).

The Rare Disease Task Force, the advisory body to the Minister of Health, initially appointed in 2008, was reorganised in late 2011 to meet new expectations of preparing strategic paper “National Plan for Rare Diseases – the roadmap”. Chaired by representative of the Ministry of Health it consists of: four experts in the field of rare diseases including representative of umbrella patient organisation, representatives of Poland in the EU institutions in the field of rare diseases, Directors of the Departments at the Ministry of Health and representatives of National Health Fund (the payer).

The Rare Disease Task Force cooperates closely with the Parliamentary Group on Rare Diseases with joint meetings dedicated either to general issues of rare diseases or solutions of individual patients’ cases.

In summary, in 2013, a total of 42 meetings, consultations, workshops and conferences were held, with active participation of group of ca 400 experts (clinicians, scientists, patients groups, providers, payers, industry, Members of Parliament, Government Officials, health politicians) to work on the document entitled “The National Plan for Rare Diseases – the roadmap”. The final version (no 12.2) was submitted to the Ministry of Health in December 2012, as scheduled. However, the Plan was not adopted in 2013 as it was decided to further develop the defined areas into concrete actions. The revised plan will be submitted for approval in 2014. Work on an organisational, budgetary and legal framework is scheduled, based on the strategic paper “The National Plan for Rare Diseases – the roadmap” which has been accepted by the Ministry of Health and the Government.

Centres of expertise
In Poland, the healthcare provision for 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. For instance there was a national coordinating centre for metabolic rare diseases at the Children’s Memorial Health Institute in Warsaw with links to regional centres. A designation policy for centres of expertise is intended to be established.

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Registries
There is no national committee dealing with registries, however the Centre of Information Systems has the responsibility to facilitate the Parliament Act of April 2011 on information systems in healthcare, which is in force since January 2012. It states that all registries have to comply with strict requirements, including data safety and protection. Registries had to comply with its requirements by June 2012 including measures for data safety and protection. The Minister of Health in the field of monitoring of healthcare needs, patients health status, prophylaxis and monitoring of health programs, may establish a dedicated registry, issuing a particular Regulation, which may also state a public source of funding. Although this legal regulation is in force, there is no designation process for a unified rare disease registry at the moment.

So far a number of registries by Academia and clinicians are in place, such as the National Registry of Paediatric Cancers and the Polish Registry of Congenital malformations.

Poland took part in the European registries such as EIMD, EHDN, ESID, ERCUSYN, TREAT-NMD, EUROCARE CF, EUROWILSON, EUROGLYCAN, EURO-WABB, SCNIR, RARECARE, and EUROCAT.

Neonatal screening policy
There is a national newborn screening program which in its first phase included: phenylketonuria, congenital hypothyroidism, cystic fibrosis and congenital deafness and as of 2013 includes an additional 20 metabolic disorders which can be diagnosed using tandem mass spectrometry. This program is country-wide, regulated and financed by Ministry of Health and coordinated by the Institute of Mother and Child in Warsaw. A screening program for congenital deafness is conducted in collaboration with NGO (The Great Orchestra for Christmas Charity) by Poznań Medical University.

Genetic testing
There are about 35 laboratories (public and private) offering testing for rare diseases. There are no officially designated reference laboratories. Most of them follow external quality control assessments. Diagnostic procedures which are performed in these labs mainly focus 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).

If it is not possible to diagnose a specific disease in Poland the National Health Found (the payer) may reimburse diagnostic procedures on demand, after a referral (second opinion scheme) from the National or Regional Consultant from the appropriate discipline.

Diagnostic tests are registered as available in Poland for 217 genes and an estimated 323 diseases in the Orphanet database².

National alliances of patient organisations and patient representation
The National Forum for the Therapy of Rare Diseases – ORPHAN, founded in 2005, serves as national alliance for rare disease patients’ organisations in Poland. As the umbrella for rare disease associations, the Forum groups together 33 rare diseases patient organisations and it strengthens the cooperation at the national level. The representative of the National Forum was appointed by the Minister of Health as the member of the Rare Disease Task Force, representing the single unanimous voice and position of Polish rare diseases patient organisations during the process of drafting the “National Plan for Rare Diseases – the roadmap”. More information about the goals, membership and activities of the organisation are published online³.

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, in 2010 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.

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² Information extracted from the Orphanet database (January 2014).
³ www.rzadkiechoroby.pl/np
Since April 2011 the Orphanet Poland team maintains a national Orphanet Poland national website. In order to improve access to information on rare diseases, orphan medicinal products and Orphanet 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 and Orphanet Activity Report 2012. Information is available on the Orphanet website.

Orphanet Poland took part in elaborating “The National Plan for Rare Diseases – the roadmap” by participating in Ministry of Health Rare Disease Task Force, and a series of meetings with patient organisations and workshops dedicated to rare diseases which took place in September 2012 in the Children’s Memorial Health Institute. Polish Orphanet Coordinators were group leaders of two workshops “Support activities for the preparation of applications for research projects related to rare diseases” and “Exchange of information to gather knowledge about centers of expertise in Poland; Creation of centers of expertise for group of rare diseases based on currently operating centers treating patients with rare diseases; Support for the creation of reliable registries for rare diseases”.

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.

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 5 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.). Moreover since 2013 a new medical speciality, paediatric metabolic medicine, has been established, what should improve access for the patients with inborn errors of metabolism to well-educated physicians and facilitate setting of reference centres.

National rare disease events in 2013
The International Rare Disease Day (28 February 2013) was organised by National Forum for the Therapy of Rare Diseases – ORPHAN in the Palace of Culture and Science, which is the most recognisable building in Warsaw. Upon this occasion, topics of the draft paper “National Plan for Rare Disease – the roadmap” have been discussed among patients’ families, professionals and parliament representatives and also in several radio, TV and web interviews. The audio-video installation was provided as a platform for patients to share their thoughts and experiences of living with rare diseases. It was a start of a long-term project of “Rare Diseases are Common” campaign. After opening this exhibition appeared in many other places, travelling across Poland. The volunteers “GENE-ius Agents” educated people about rare diseases on Warsaw streets and in public bses.

An awareness raising campaign entitled “Rare diseases are frequent” kicked off at the Polish Europlan Conference on 27-28 September 2013. The European Commission and EURORDIS entrusted to the National Forum for the Therapy of Rare Diseases – ORPHAN to assess the Polish policy towards the issue of rare diseases. On 28 September 2013, during the second day of the Conference a debate and workshop concerning

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4 www.orpha.net/national/PL-PL
5 http://www.eurordis.org/sites/default/files/flags/finalreport-poland.pdf
the next steps in relation to the plan was held. The debate tackled philosophical, scientific, and medical issues. Recognised experts in medicine, bioethics and pharmacotherapy along with representatives of the Polish Parliament (Sejm) and national patient organisations attended. Issues regarding evaluation of effectiveness of technology and orphan drugs (HTA) and regulatory restrictions on access to the therapy and issues regarding the prospects for the implementation of the National Plan for Rare Diseases were discussed in details. The purpose of the debate was to reach an unanimous approach and develop recommendations (included in the report) for healthcare policy makers, to let them include the recommendations developed by prominent participants of the health care system focused on rare diseases in their efforts to improve the scope and therapy standards. The recommendations aim to support the Minister of Health in the correct implementation of the state policy regarding rare diseases.

Also a number of meetings for medical students entitled ‘Conversations on rare diseases’ were also organised across Poland in 2013.

Hosted rare disease events in 2013

Amongst the hosted events organised in 2013 was the 9th European inborn errors of metabolism course in Warsaw, Poland (in collaboration with the Children’s Memorial Health Institute) (Warsaw, 25-29 September 2012), Rare diseases and risk of social exclusion (7 October 2013, Warsaw), and the Fourth International Meeting on Primary Central Hypoventilation Syndromes (Warsaw, 13-14 April 2012).

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 are 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. The Polish Ministry of Science and Higher Education usually allocated funding for around ten research projects dedicated to rare diseases in calls for proposals.

Participation in European research projects

Research teams in Poland participate/have participated in 18 FP7 rare disease related projects.

E-Rare

Poland is an observer of the E-Rare 2 project. The Polish partner for E-Rare ERA-NET is the National Centre for Research and Development. Poland joined the 2012 Joint Transnational Call but did not receive funding. Poland participated in the 2013 5th Joint Transnational Call but Polish teams did not participate in the selected projects.

IRDiRC

Polish funding agencies have not committed funding to the IRDiRC.

Orphan medicinal products

Orphan medicinal product committee

There is no specifically dedicated orphan medicinal product committee in Poland.

Accordingly to the new Reimbursement Act (in force since July 2011), the Economics 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 2013 following orphan medicinal products for inborn errors of metabolism have been reimbursed within therapeutic programmes: Cerezyme, Naglazyme, Elaprase, Laronidase, Myozyme, and Cystadane. Other orphan medicinal products (drugs and FSMP) registered outside Poland are available through the individual import.

Orphan medicinal product pricing policy

Since July 2011 Economics Committee (appointed by the Minister of Health) takes responsibility in pricing on negotiation basis with the market authorisation holder. Following their recommendation the Minister of Health
issues public price and reimbursement conditions for the drug. Each medicinal product has to follow HTA requirements of the by Polish Medicinal Health Technology Assessment Agency. In addition to this, the role of HTA has been strengthened.

**Orphan medicinal product reimbursement policy**

The system of drug reimbursement changed on 1 January 2012 accordingly to the Reimbursement of drugs, food for special dietary use and medical devices Act, issued 12 May 2011. The new system is unified and application based. The Minister of Health is not able to introduce reimbursement of a new drug without prior official request from the Marketing Authorisation Holder. The application process is costly and has to be supported with comprehensive data (including reimbursement status, price data in other EU Member States, and health technology analysis).

Currently, drugs for some rare diseases are reimbursed through therapeutic programmes. The Minister of Health every second month (I, III, V, VII, IX, XI) announces the official order containing the list of reimbursed medicinal products. Diseases currently covered include: Crohn disease, Prader-Willi syndrome, cystic fibrosis. Additionally, six drugs for rare diseases are reimbursed for patients with Gaucher disease, MPS I, II and VI, Pompe disease, and hyperhomocysteinemia.

**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. Furthermore, according to the Reimbursement Act, after the recommendation of the national authority in the field of medicine and opinion of the HTA Agency, the Minister of Health can make a reimbursement decision.

**Other therapies for rare diseases**

No specific activity reported.

**Orphan devices**

Since 1 January 2012, according to the Reimbursement Act, the national healthcare package has been continuously supplemented by new medical devices dedicated also to patients with rare diseases. Orphan devices are regularly presented during dysmorphology meetings, national conferences and trainings.

**Specialised social services**

There are no social services specifically designed 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 publicly 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 funds the integration of children with special needs, via “integration classes” in schools.
DEVELOPMENT OF RARE DISEASE ACTIVITIES IN 2013 IN POLAND

Definition of a rare disease
During the several public debates on rare diseases, consideration of the adoption of an additional sub-definition of ultra-rare disease has been suggested as the necessary to be incorporated within the Polish health care system. The preliminary assumption is that the ultra-rare disease definition would be based on a prevalence of no more than 1 in 50,000 individuals.

National plan/strategy for rare diseases and related actions
By the end of 2012 a strategic document “National Plan for Rare Disease – the roadmap” was prepared and handed to the Minister of Health. A broad consultation process started, and it was scheduled for completion by June 2013 in order to meet the recommendations of the EU Council by the end of 2013.

In summary, in 2013, a total of 42 meetings, consultations, workshops and conferences were held, with active participation of group of ca 400 experts (clinicians, scientists, patients groups, providers, payers, industry, Members of Parliament, Government Officials, health politicians) to work on the document entitled “The National Plan for Rare Diseases – the roadmap”. The final version (no 12.2) was submitted to the Ministry of Health in December 2012, as scheduled. However, the Plan was not adopted in 2013 as it was decided to further develop the defined areas into concrete actions. The revised plan will be submitted for approval in 2014. Work on an organisational, budgetary and legal framework is scheduled, based on the strategic paper “The National Plan for Rare Diseases – the roadmap” which has been accepted by the Ministry of Health and the Government.

Neonatal screening policy
As of 2013 the newborn screening programme includes an additional 20 metabolic disorders which can be diagnosed using tandem mass spectrometry. This program is country-wide, regulated and financed by Ministry of Health and coordinated by the Institute of Mother and Child in Warsaw.

Training and education initiatives
Since 2013 a new medical specialty, paediatric metabolic medicine, has been established, what should improve access for the patients with inborn errors of metabolism to well-educated physicians and facilitate setting of reference centres.

National rare disease events in 2013
The International Rare Disease Day (28 February 2013) was organised by National Forum for the Therapy of Rare Diseases – ORPHAN in the Palace of Culture and Science, which is the most recognisable building in Warsaw. Upon this occasion, topics of the draft paper “National Plan for Rare Disease – the roadmap” have been discussed among patients’ families, professionals and parliament representatives and also in several radio, TV and web interviews. The audio-video installation was provided as a platform for patients to share their thoughts and experiences of living with rare diseases. It was a start of a long-term project of “Rare Diseases are Common” campaign. After opening this exhibition appeared in many other places, travelling across Poland. The volunteers “GENE-ius Agents” educated people about rare diseases on Warsaw streets and in public buses.

An awareness raising campaign entitled “Rare diseases are frequent” kicked off at the Polish Europlan Conference on 27–28 September 2013. The European Commission and EURORDIS entrusted to the National Forum for the Therapy of Rare Diseases – ORPHAN to assess the Polish policy towards the issue of rare diseases. On 28 September 2013, during the second day of the Conference a debate and workshop concerning the next steps in relation to the plan was held. The debate tackled philosophical, scientific, and medical issues. Recognised experts in medicine, bioethics and pharmacotherapy along with representatives of the Polish Parliament (Sejm) and national patient organisations attended. Issues regarding evaluation of effectiveness of technology and orphan drugs (HTA) and regulatory restrictions on access to the therapy and issues regarding

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6 http://www.eurordis.org/sites/default/files/flags/finalreport-poland.pdf
the prospects for the implementation of the National Plan for Rare Diseases were discussed in details. The purpose of the debate was to reach an unanimous approach and develop recommendations (included in the report) for healthcare policy makers, to let them include the recommendations developed by prominent participants of the health care system focused on rare diseases in their efforts to improve the scope and therapy standards. The recommendations aim to support the Minister of Health in the correct implementation of the state policy regarding rare diseases.

Also a number of meetings for medical students entitled ‘Conversations on rare diseases’ were also organised across Poland in 2013.

Hosted rare disease events in 2013
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Research activities and E-Rare partnership
E-Rare
Poland participated in the 2013 5th Joint Transnational Call but Polish teams did not participate in the selected projects.
LIST OF CONTRIBUTIONS

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\[7\] 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.
SELECTED BIBLIOGRAPHY AND SOURCES

- National Forum on the Treatment of Orphan Diseases
  http://www.rzadkiechoroby.pl/
- Rare Disease Day website
  www.dzienchorobrzadkich.pl
- A social media campaign entitled “Hope. It’s in your genes”
  www.nadziejawgenach.pl
- Orphanet Poland national website
  http://www.orpha.net/national/PL-PL/index/strona-g%C5%82%C3%B3wna/
- Final Report of the 2010 Polish Europlan Poland National Conference
- Final Report of the 2013 Polish Europlan Poland National Conference
  http://www.eurordis.org/sites/default/files/flags/finalreport-poland.pdf

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