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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 LATVIA

This work was financed by the EUCERD Joint Action: Working for Rare Diseases N° 2011 22 01

EUCERD European Union Committee of Experts on Rare Diseases
2011-22-01
EUCERD Joint Action

This document has been produced by the Scientific Secretariat of the European Union Committee of Experts on Rare Diseases (EUCERD, formerly the European Commission's Rare Diseases Task Force) through the EUCERD Joint Action: Working for Rare Diseases (N° 2011 22 01, Coordinator: Kate Bushby, University of Newcastle, United Kingdom), within the European Union's Second Programme of Community Action in the Field of Health.

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

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To quote this document:
Aymé S., Rodwell C., eds., *"2012 Report on the State of the Art of Rare Disease Activities in Europe of the European Union Committee of Experts on Rare Diseases"*, July 2012.

ISBN : 978-92-79-25355-3
DOI : 10.2772/50554

http://www.eucerd.eu/wp-content/uploads/2012/09/2012ReportStateofArtRDActivitiesLV.pdf

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 Latvia. 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 LATVIA

Definition of a rare disease

In Latvia, there is no official definition for rare diseases, as stakeholders in Latvia 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

Work has recently been finished on a national plan by the working group, which included health care specialists and representatives from the Ministry of Health. In December 2011, the plan was written and submitted to the Ministry of Health for evaluation. The costs related to rare diseases are currently included in the national health care budget.

A National Cancer Control Programme (2009-2015) was stipulated by regulations No.48 of the Cabinet of Ministers of the Republic of Latvia (adopted on the 29 January, 2009), and included rare cancers. In August 2009, a regulation was introduced which allowed for the compensation of medicines for children with rare diseases.

Centres of expertise

There are currently no official centres of expertise for rare diseases in Latvia, but, for example, the Latvian State University Children's Hospital provides genetics services. The Riga East University Hospital has a specialised clinic (Chemotherapy and haematology clinic) in which haemophilia A, haemophilia B, Factor XII deficiency and von Willebrand disease receive diagnostics and treatment (in this hospital, rare oncological diseases also can be treated, e.g. Burkitt's lymphoma, Langerhans cell histiocystosis, Mantle-cell NHL, multiple endocrinology neoplasia, Erwing's sarcoma, Wilm's tumour, Waldenström macroglobulinemia and others).

A rare cardiovascular diseases network (Poland, Lithuania and Latvia through the P. Stradins Clinical University Hospital, Centre of Cardiology), started in May 2011. This project will last until January 2013.

Pilot European Reference Networks

Latvian teams participate/participated in the following European Reference Networks for rare diseases: Dyscerne and PAAIR.

Registries

The National Health Service of Latvia is the supervising authority and keeper of State Register of patients with specific diseases. There is no separate register for rare diseases, but the register of the patients with specific diseases includes patients with cancer and congenital anomalies (some of these are rare diseases). Latvia contributes to the EUROCARE (Eurocare-5 study) European registry.

Neonatal screening policy

In Latvia, newborns are screened for phenylketonuria and congenital hypothyroidism. All activities connected with the evaluation of these tests and quality control are carried out by the Center for Disease Control and Prevention and under the supervision of the International Society of Neonatal Screening.

Genetic testing

Genetic testing is available in Medical Genetics Clinic of Latvian State University Children's Hospital, Molecular Laboratory, Riga Stradins University, Scientific Laboratory and in Latvian BioMedical Research and Study *Center. No* national guidelines and specific conditions for reimbursement of expenses related to the tests have yet been determined. Genetic testing in other EU and EFTA states is possible with a E112/S2 form if genetic testing is a health care service usually financed from the state budget and this service cannot be provided in the Republic of Latvia or cannot be provided within a reasonable period of time.

Diagnostic tests are registered as available in Latvia for 4 genes and an estimated 4 diseases in the Orphanet database[2].

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

National alliances of patient organisations and patient representation

In 2009 the Latvian Rare Disease Organisation Caladrius[3] was launched. The mission of the organisation is to provide patients with the relevant information and support and to represent patients. In 2010 Caladrius established a fund to help rare disease patients who could not otherwise fund their treatments: the organisation had obtained public benefit organisation status to legally collect funds for this action. In 2011 Caladrius (in collaboration with Latvian State University Children's Hospital) organised two visits of the high-qualified cardio surgeons: as a result, high-complicated operations were carried out for 11 children with inborn heart patalogy. In 2011 Caladrius organised 4 seminars about methods of alternative therapy.

In Latvia are a number of other rare diseases and rare diseases-related patient organisations, including the Haemophilia Society, the Society for People with Disabilities Motus Vitae, the Phenylketonuria Society. These organisations often collaborate with each other and in 2011 had many activities, for example, Motus Vitae joined the International ALS/MND Alliance and arranged the international conference VII Nordic ALS Alliance meeting in Latvia "Baltic Bridge": Services for people living with ALS/MND (there participated patients with their assistants, medical professionals, social workers and Health Care Institutions from Denmark, Finland, Estonia, Russia, Iceland and Latvia).

Palidzesim.lv is a non-governmental organisation which financially supports children and families to confirm a diagnosis of rare diseases by sending patients or medical samples abroad[4].

Sources of information on rare diseases and national help lines

Orphanet activities in Latvia

Since 2006, there is a dedicated Orphanet team in Latvia, currently hosted by the Medical Genetics Clinic of the Latvian State at the Children's University Hospital, Riga. 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 Latvia for entry into the Orphanet database. The Ministry of Health of the Republic of Latvia has designated the National Health Service as the representative of the Republic of Latvia to participate in the Joint Action Orphanet Europe since April 2011. The Orphanet Latvia country site is in progress and the site will go on line in April 2012.

Official information centre on rare diseases

There is no information centre for rare diseases in Latvia other than Orphanet. Web based information is available for a limited number of diseases (rare and non-rare) and certain information is maintained by using the state budget.

Help line

There are non-rare disease specific help lines run by the state, some by the state budget, to help patients to access health care and psychological support, but no help line dedicated to rare diseases.

Other sources of information on rare diseases

No information reported yet.

Good practice guidelines

No best practice guidelines for rare diseases have been produced at national level in 2011.

Training and education initiatives

No events reported yet.

National rare disease events in 2011

To mark Rare Disease Day 2011, the Latvian Rare Disease Organisation, Caladrius, organised informative seminar for public and media representatives about Genetic and rare diseases.

The Latvian Hemophilia Society organised meeting for people with rare bleeding disorders on 16-17 April 2011 to celebrate World Haemophilia day: this was most attended meeting in many years, people wore red as recommended by the global dresscode. Many professionals participated and event was mentioned in media.

[3] www.caladrius.lv

Hosted rare disease events in 2011
No hosted events were reported.

Research activities and E-Rare partnership
National research activities
Funding is available for rare disease projects (through state budget, charities and pharmaceutical companies) although funds are not specifically earmarked for rare disease research.

Participation in European research projects
A Latvian team participates in the European Haemophilia Safety Surveillance- EUHASS European research project.

E-Rare
Latvia is not currently a partner of the E-Rare project.

IRDiRC
Latvian funding agencies are not currently committed members of the IRDiRC.

Orphan medicinal products
The State Agency of Medicines of Latvia is responsible for regular collecting and distributing of the information on medicines, including orphan medicinal products, as well as for collecting and compiling the information on the safety, evaluating drug risks and coordinate measures of medicine use risk mitigation, according to Regulations No. 1006 of the Cabinet of Ministers (adopted on December 7, 2004) "State Agency of Medicines Statutes".

Orphan medicinal product committee
A representative of Latvia is a member of the Committee for Orphan Medicinal Products (COMP) of European Medicines Agency.

Orphan medicinal product incentives
The *Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products* reported that in Latvia "*the State Agency of Medicines is entitled, due to considerations of health protection, to make a decision (after discussion with the Minister for Health) regarding the fee exemption or reduction for activities associated with the evaluation, registration or re-registration of a medicinal product if the medicinal product (with or without orphan designation pursuant to Regulation 141/2000) is intended to the treatment of a rare disease*." [5]
Under the centralised procedure, companies submit a single marketing-authorisation application to the European Medicines Agency. Once granted by the European Commission, a centralised (or 'Community') marketing authorisation for Orphan medicinal products is valid in all European Union (including Latvia) and EEA-EFTA states.

Orphan medicinal product market availability situation
The State Agency of Medicines of Latvia's includes the medicinal products registered in the Republic of Latvia and the centrally registered medicinal products (including orphan medicinal products) in a register[6] of medical products of the Republic of Latvia (according to Regulations No. 376 of the Cabinet of Ministers (adopted on May 9, 2006) "Procedures for the Registration of Medicinal Products").

The following orphan medicinal products were marketed in Latvia in 2011:Arzerra, Cystadane, Diacomit, Gliolan, Glivec, INCRELEX, Kuvan, Litak, Mozobil, Nexavar, Nplate, Pedea, Revatio, Revolade, Sprycel, Tasigna, Ventavis, Volibris, Wilzin, Myrin. In 2011, compared to 2010, 6 new orphan medicinal products were launched on the market: Kuvan, Litak, Mozobil, Nplate, Revolade, Tasigna and 4 orphan medicinal products are no longer available on the market: Atriance, Evoltra, Exjade, Orfadin.

[5] Inventory of *Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products (2005 revision)* (pp16-17)
[6] http://www.zva.gov.lv/index.php?id=112&top=112&large=

Orphan medicinal product pricing policy
No specific policy is in place.

Orphan medicinal product reimbursement policy
Since 2009, some orphan medicinal products for children are available as a part of the special programme "Medical treatment of rare diseases for children" for Children's University Hospital, Riga. Within this programme, there are provided some orphan medicinal products like Elaprase, Cystadane, Increlex, Kuvan.

Orphan medicinal products are partially available via the reimbursement system. Imatinibum, Dasatinibum, Nilotinibum are included in the positive reimbursement list.

2% of reimbursement budget is intended to individual reimbursement with limitation up to 10 000 LVL/year for a single patient. Within this individual reimbursement, the following orphan medicinal products are provided: Exjade, Revatio, Volibris, Nexavar, Atriance, Cystadane, Wilzin, Diacomit.

Other initiatives to improve the availability of orphan medicinal products
The *Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products* reported that in Latvia
"The State Agency of Medicines may issue [...] distribution authorisation for medicinal products not registered in Latvia if the medicinal product is intended for treatment of a rare disease (for an individual patient on the basis of prescription or for use in a health care institution on the basis of a written request)."[7]

Orphan devices
No specific information reported.

Specialised social services
Respite care services are available and the categories of patients eligible for reimbursement are described in the "Procedures for the Organisation and Financing of Health Care" (Regulations of the Cabinet of Ministers No. 1046, adopted on 19 December 2006). Therapeutic recreational programmes are also available and costs are included in the national health care budget. There are existing government-run services promoting social integration of those with handicaps, including the school environment and work place.

DEVELOPMENT OF RARE DISEASE ACTIVITIES IN 2011 IN LATVIA

National plan/strategy for rare diseases and related actions
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[7] *Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products* (2005 revision) (pp16-17)

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Research activities and E-Rare partnership

IRDiRC

Latvian funding agencies are not currently committed members of the IRDiRC.

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Contributions in 2011
This report has been compiled in collaboration with members of the Ministry of Health Centre of Health Economics, Health Payment Centre, State Agency of Medicines and Medical Genetics Clinic of the University Children's Hospital, Riga, including:
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Contributions in 2012
This report has been compiled in collaboration with members of the Ministry of Health, the National Health Service, the State Agency of Medicines, the Riga East University Hospital, the Paula Stradins Clinical University Hospital, Caladrius (The Latvian Rare Disease Organisation), the Society for People with Disabilities Motus Vitae, the Haemophilia Society and the Pulmonary Hypertension Association.

Validated by*:* Antra Valdmane *(EUCERD Representative Latvia, Ministry of Health of the Republic of Latvia)*

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  http://www.orpha.net/national/LV-LV/index/homepage/
- Latvian Rare Disease Organisation - Caladrius
  www.caladrius.lv
- Palidzesim.lv
  www.palidzesim.lv

[8] 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.
[9] 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