2013 REPORT ON THE STATE OF THE ART OF RARE DISEASE ACTIVITIES IN EUROPE

STATE OF THE ART OF RARE DISEASE ACTIVITIES IN LATVIA

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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
EUROORDIS - 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 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.
RARE DISEASE ACTIVITIES IN LATVIA

Definition of a rare disease
Stakeholders in Latvia accept the European Regulation on Orphan Medicinal Products definition of a prevalence of no more than 5 in 10,000 individuals and that rare diseases are life-threatening or chronically debilitating diseases.

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 public consultation of the plan was launched in 2012 and the results were analysed by the Ministry of Health. A number of meetings with different stakeholders were held, and as a result, the plan was further elaborated. The plan should be able to be adopted in 2013.

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 designated centres of expertise for rare diseases in Latvia, but, for example, the Latvian State University Children’s Hospital provides genetics services, hospital specialists deal with children with haematological, oncological and endocrinological diseases. 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 histiocytosis, Mantle-cell NHL, multiple endocrinology neoplasia, Erwing’s sarcoma, Wilm’s tumour, Waldenström macroglobulinemia and others). Pauls Stradins University hospital has services in different rare diseases area: cardiology, nephrology, vascular diseases (Arteriovenous vascular malformations, lymphatic disorders, aortic pathology, endarteritis, carotid tumors, etc.), ophthalmology, oncology, gastroenterology, endocrinology, pulmonology. 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.

The Ministry of Health, Orphanet team and experts from 3 University hospitals have been started work on developing national criteria for centres of expertise to be registered in Orphanet database.

Registries
There is no separate registry for rare diseases. The Centre for Disease Prevention and Control is the supervising authority and keeper of Register of patients with particular diseases, including cancers, congenital anomalies (some of these are rare diseases). There is a plan to pilot use the Orpha code for rare diseases in the register of patients with congenital anomalies. Latvia contributes to the EUROCare (Eurocare-5 study) European registry, RARECARENet, Joint Action EUROCAT.

Specialists from university hospital centres collect rare disease patient data, for example, at the Latvian Cardiology Centre is Pulmonary Arterial Hypertension patient’s data base.

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 10 genes and an estimated 9 diseases in the Orphanet database¹.

**National alliances of patient organisations and patient representation**

In 2009 the Latvian Rare Disease Organisation Caladrius² 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. There are plans to create an alliance of rare diseases patient organisations and chronic patient organisations at national level. Until now there were 8-9 organisations who share information and collaborate together in this area. Rare diseases patient organisations lack the capacity to establish an alliance.

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, Pulmonary Hypertension Society and Society of Cystic fibrosis. 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 abroad³.

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

**Orphanet activities in Latvia**

The Ministry of Health of the Republic of Latvia has designated The Centre for Disease Prevention and Control as the representative of the Republic of Latvia to participate in the Joint Action Orphanet Europe since April 2012. The Orphanet team is currently hosted by the Centre for Disease Prevention and Control and 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 Orphanet Latvia country site was launched in April 2012 and regularly updated by the Orphanet team.

**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**

Information on rare diseases is available regarding paediatric rheumatic diseases⁴, lysosomal diseases (Gaucher disease, Fabry disease and Hunter syndrome)⁵, pulmonary hypertension⁶, bleeding disorders⁷, and via PHL Latvia⁸.

**Good practice guidelines**

No best practice guidelines for rare diseases have been produced at national level in 2012.

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¹ Information extracted from the Orphanet database (December 2012).
² www.caladrius.lv
³ http://www.printo.it/pediatric-rheumatology/information/Lettonia/index.htm
⁴ http://lus.dev.zvirbulis.lv/lv/sakums
⁵ http://lus.dev.zvirbulis.lv/lv/sakums
⁶ www.hemofilija.lv
⁷ www.phlatvia.lv
Training and education initiatives
No events reported yet.

National rare disease events in 2012
The Rare Disease Association Caladrius, Latvian Association of Hemophilia, PHA Latvia and the Association Motus Vita all marked Rare Disease Day in Latvia on 29 February 2012 with an event entitled ‘SOLIDARITY’. The event took place in the EU House Conference Hall in Riga. The event served to highlight a number of issues concerning the quality of care for rare disease patients, as well as access to diagnosis and treatment, and aimed to make suggestions concerning what can be done to improve the situation for rare disease patients in Latvia. The Children’s Hospital organised a day to raise awareness of cystic fibrosis dedicated to the parents and primary care providers.

Hosted rare disease events in 2012
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 do not currently contribute funds to 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.”

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.

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9 Inventory of Community and Member States’ incentive measures to aid the research, marketing, development and availability of orphan medicinal products (2005 revision) (pp16-17)
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 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 2012: Aldurazyme, Cystadane, Diacomit, Glialan, INCRELEX, Jakavi, Kuvan, Litak, Mozobil, Myrin, Nexavar, Pedea, Revatio, Sprycel, Tasigna, Ventavis, Volibris, Wilzin, Yondelis. In 2012, compared to 2011, 3 new orphan drugs were launched on the market: Aldurazyme, Jakavi, Yondelis and 3 orphan drugs were no longer available on the market in 2012: Arzerra, Nplate, Revolade. Medicinal product Glivec which was originally designated as an orphan medicine and was placed on the market in 2011 is no longer designated as an orphan drug in Europe.

Orphan medicinal product pricing policy
There are no specific provisions for the pricing of orphan drugs. There have not been any developments in this area.

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: Revatio, Volibris, Nexavar, Cystadane, Diacomit, Mozobil, Thalidomide

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)”

Orphan devices
There were no orphan devices placed on the market in 2012

Other therapies for rare diseases
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.

11 Inventory of Community and Member States’ incentive measures to aid the research, marketing, development and availability of orphan medicinal products (2005 revision) (pp16-17)
DEVELOPMENT OF RARE DISEASE ACTIVITIES IN 2012 IN LATVIA

National plan/strategy for rare diseases and related actions
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Registries
There is a plan to pilot use the Orpha code for rare diseases in the register of patients with congenital anomalies.

Sources of information on rare diseases and national help lines

Orphanet activities in Latvia
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Orphan medicinal products

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Contributions in 2010
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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
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.

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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

12 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.
13 All websites and documents were last accessed in May 2013.