

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



STATE OF THE ART OF RARE DISEASE ACTIVITIES IN SERBIA

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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 Serbia. 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 SERBIA

Definition of a rare disease

There is no official definition of a rare disease in Serbia although the generally accepted and used definition is that of the European Regulation on Orphan Medicinal Products of a disease affecting no more than 5 in 10 000 persons.

National plan/strategies for rare diseases and related actions

There is currently no national plan for rare diseases in Serbia. A Republic Expert Committee for a Strategy on Rare Diseases has been established and a proposal for a strategy was developed in 2013 envisaging a number of actions over the years leading up to 2020 including: improvement of diagnostics and treatment, improving availability of orphan drugs, registration of rare disease patients, screening for rare diseases, and increasing the participation in patients' associations. The establishment of centres of expertise has been highlighted as a priority topic. The lack of coordination between different parts of public administration is a problem in the field of rare diseases. The Ministry of Health has yet to give their opinion of the proposals. It is hoped that a stakeholder task force will be established to define objectives, activities and next steps for the implementation of key areas of the proposed National Strategy.

Currently there is a policy to provide treatment for limited number of rare diseases and to define centres of expertise for rare diseases.

A Europlan conference, co-organised by the National Organisation for Rare Diseases of Serbia and the Institute for Molecular Genetics and Genetic Engineering of Belgrade University, under the auspices of the Ministry of Health and Eurordis, was held on 6-7 December 2013 in Belgrade bringing together 170 participants from all stakeholder groups. The conference also drew participation from surrounding countries. At the conference the proposal for a national rare disease strategy was presented and priority topics concerning the field of rare diseases were discussed. The conference report is available online for consultation¹.

Centres of expertise

The proposed strategy recommends that centres of expertise should be formed within existing institutions so that existing resources and expertise can be fully utilised. The Republic Expert Committee for Rare Diseases has already determined that 5 centres should be formed.

Registries

There is currently no national registry for rare diseases, although there are national registries for individual rare diseases (cystic fibrosis, haemophilia and rare coagulopathies).

Neonatal screening policy

Compulsory screening programmes are in place for phenylketonuria and congenital hypothyroidism.

Genetic testing

Diagnostic tests are registered as available in Serbia 31 genes and an estimated 29 diseases in the Orphanet database². Medical laboratories offering diagnostic services for rare diseases are mostly not accredited due to the high costs of the procedure and because accreditation is not officially required Reimbursement is not possible for majority of genetic tests.

National alliances of patient organisations and patient representations

The umbrella organisation the National Organisation of Rare Diseases in Serbia (NORBS³) groups 13 rare disease patient organisations in the country. NORBS organises Rare Disease Day events in Serbia and co-organised the 2013 Europlan conference to discuss a proposal for a national strategy for rare diseases.

¹ <http://www.eurordis.org/sites/default/files/flags/finalreport-serbia.pdf>

² Information extracted from the Orphanet database (January 2014).

³ <http://www.norbs.rs/>

Sources of information on rare diseases and national help lines

Orphanet activity in Serbia

There is no official, rare disease specific information centre on rare diseases in Serbia other than Orphanet. Since 2006 there is a dedicated Orphanet team for Serbia that works on voluntary basis and it is currently hosted by the Institute for Molecular Genetics and Genetic Engineering of Belgrade University. This team is in charge of collecting data on rare disease related services (specialised clinics, medical laboratories, ongoing research, registries, clinical trials and patient organisations) in their country for entry into the Orphanet database. The team also maintains the national Orphanet Serbia website⁴ in the national language.

Official information centre for rare diseases

There is no official information centre for rare diseases in Serbia apart from the services provided by Orphanet Serbia.

Help line

There is no rare disease helpline in Serbia

Other sources of information on rare diseases

NORBS and patient organisations provide information on rare diseases.

Guidelines

No reported information.

Training and education initiatives

No reported information.

National rare disease events in 2013

Rare Disease Day events are organised by NORBS at national level.

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Hosted rare disease events in 2013

No activities reported.

Research activities and E-Rare partnership

National research activities

There are no funding programmes dedicated to rare disease research in Serbia, but there are some research projects involving rare diseases, which are funded by Ministry of Education, Science and Technological Development.

Participation in European research projects The Institute for Molecular Genetics and Genetic Engineering of Belgrade University coordinates one EU FP7-REGPOT project. A team in Serbia participates in 1 bilateral project related to rare diseases

E-Rare

Serbia is not a partner of E-Rare.

IRDiRC

Serbia is not a member of the IRDiRC

⁴ <http://www.orpha.net/national/RS-SR/index/po%C4%8Detna-strana/>

⁵ <http://www.eurordis.org/sites/default/files/flags/finalreport-serbia.pdf>

Orphan medicinal products

Orphan medicinal product committee

The National Health Insurance Fond has a committee for medicinal product for treatment of some inborn metabolic errors

Orphan medicinal product incentives

There are no specific incentives for orphan medicinal products in Serbia.

Orphan medicinal product market availability situation

No reported information.

Orphan medicinal product pricing policy

No reported information.

Orphan medicinal product reimbursement policy

130 million Dinars (around €1.2 million) were allocated to the use of orphan drugs in Serbia with 280 million Dinars (around €2.6 million) planned for this purpose in 2014. This amount goes towards treatments for only a handful of paediatric patients with metabolic diseases requiring enzyme replacement therapies (ERT). A special fund for rare diseases reimburses this treatment due to the high price of the therapy. Other orphan products are reimbursed from the Republic Health Insurance Fund. The Republic Health Insurance Fund can only reimburse orphan medicinal products that are registered in Serbia. Since Serbia is not an EU Member State there is no centralised market authorisation procedure and the registration procedure can take up to one year.

Other initiatives to improve access to orphan medicinal products

Compassionate and off-label use is not recognised by the health insurance system.

Orphan devices

No reported information.

Other therapies for rare diseases

No reported information.

Specialised social services

Despite recent advances in legislation concerning equality and anti-discrimination for persons with disabilities, mechanisms are lacking to enforce these laws and sometimes rare diseases are not recognised in the categories of possible beneficiaries of this legislation.

There are no specific specialised social services for rare diseases currently in Serbia.

DEVELOPMENT OF RARE DISEASE ACTIVITIES IN 2013 IN SERBIA

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⁶ <http://www.eurordis.org/sites/default/files/flags/finalreport-serbia.pdf>

⁷ <http://www.norbs.rs/>

⁸ <http://www.eurordis.org/sites/default/files/flags/finalreport-serbia.pdf>

LIST OF CONTRIBUTIONS⁹

Contributions:

Orphanet Serbia

Marija Joldic (*President, National Organisation for Rare Diseases Serbia*)

SELECTED BIBLIOGRAPHY AND SOURCES¹⁰

- Serbian Europlan national conference report 2013
<http://www.eurordis.org/sites/default/files/flags/finalreport-serbia.pdf>
- National Health Insurance Fund Committees
<http://www.eng.rfzo.rs/index.php/organization/nhif-committees>

⁹ 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.

¹⁰ All websites and documents were last accessed in May 2014.