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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 MALTA

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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 Malta. 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 MALTA

Definition of a rare disease
Stakeholders in Malta 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
There is currently no national plan/strategy for rare diseases in Malta. A Task Force for the implementation of the key requirements for Member States for the Council Recommendations on a European action in the field of rare diseases was set up in October 2010. A detailed framework that will form the basis for a national strategy for rare diseases is being created. The plan is to propose a request for a first budget for the strategy of rare diseases from the National Budget for 2013. The national strategy will have a time span of a number of years with plans to incrementally implement a number of measures that will aim towards increasing the profile and care services tailored for rare diseases in Malta.

Centres of expertise
There are currently no official reference centres of expertise for rare diseases in Malta (see "Pilot European Reference Networks"). Assistance by local government for treatment abroad (namely in the UK) is possible through a bilateral health agreement between the two countries. Further bilateral agreements with other EU Member States are being sought, developed and completed.

Pilot European Reference Networks
Teams from Malta do not currently participate in European Reference Networks for rare diseases. Due to the small size of Malta and its population, participation in the future European Reference Networks will probably be only feasible on the level of individual experts or group of experts managing different cases of rare diseases or groups of rare diseases. Furthermore, there is only one potential centre of expertise which comprises the major acute general hospital; Mater Dei Hospital. This hospital caters for the majority of the secondary and tertiary healthcare provision in Malta. It is a teaching hospital (in conjunction with the University of Malta) and it is a public hospital. In addition, from 2013 onwards it is also planned to house the new Oncology Hospital which is currently under construction on the Mater Dei Hospital site.

Registries
Malta contributes to the EUROCAT European registry as well as the RARECARE and EUROCARE projects through the Malta National Cancer Registry.

Neonatal screening policy
Neonatal screening is available for haemoglobinopathies and hypothyroidism.

Genetic testing
Genetic studies (karyotyping and molecular genetic studies) in foetuses and neonates born with congenital malformations or rare syndromes are available. There are 3 consultant geneticists and 2 genetics laboratories in Malta, the Molecular Genetics Laboratory and Cytogenetics Laboratory. The indicated genetic tests that are not performed in house are referred to a reference centre abroad, mainly to NHS labs in the UK. The funding for these tests is covered by the local health authorities.

National alliances of patient organisations and patient representation
Malta does not currently have an official national alliance of rare diseases patient organisations. However, this role is increasingly being assumed by the Malta Health Network[2] which is a network of health-related voluntary organisations in Malta ().

Sources of information on rare diseases and national help lines
Orphanet activities in Malta
The government of Malta has not designated a national Orphanet team for Malta to date.

[2] http://www.maltahealthnetwork.org/

Official information centre for rare diseases
There is no official information centre on rare diseases in Malta to date.

Help line
Although there is no official help line for rare diseases, the agency Sapport provides support by telephone to all disabled people that request it. This service is funded by the government.

Other sources of information on rare diseases
There were no further developments in the sources of information on rare diseases in 2011.

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

Training and education initiatives
There are currently no initiatives specifically dedicated to rare disease-specific training and education in Malta.

National rare disease events in 2011
Malta did not commemorate RDD in 2011. However, RDD in 2012 was commemorated by the Malta Health Network (MHN) in collaboration with the Ministry for Health, the Elderly and Community Care (MHEC). Funds are being earmarked for Malta's participation in the upcoming Rare Diseases Day in future years.

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

Research activities and E-Rare partnership
National research activities
Funding for research into haemoglobinopathies and other rare genetic disorders is available through various sources (including the European Structural Funds, Ithanet and the University of Malta). According to the Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products*, "measures […] are being taken to promote research and development in Malta. Enterprises carrying out research and development are entitled to various tax credits according to the nature of the specific investments. These tax credits are in addition to the standard 100 % deductions allowed under the Income Tax Act (Cap. 123). These credits are granted under a general framework, which applies to all Research and development initiatives and not exclusively to the pharmaceutical sector[3]"*.

Participation in European research projects
Teams from Malta do not currently participate in a European research projects for rare diseases.

E-Rare
Malta is not currently a partner for the E-Rare project.

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

Orphan medicinal products
Since the start of 2010 Malta has participated in the project "Assessing Drug Effectiveness" (an initiative of the Swedish Presidency) and is currently participating in the project "Mechanism of Coordinated Access to Orphan Medicinal Products" (an initiative of the Belgian Presidency). Malta is also participating in the BBMRI initiative of the EU.

[3] *Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products (2005 revision)* (pp17-18)

Orphan medicinal product committee

Orphan medicinal products are registered through the centralised procedure and Malta has a member on the Committee for Orphan Medicinal Products and on the Committee for Human Medicinal Products of the European Medicines Agency.

Orphan medicinal product incentives

No specific reported activity.

Orphan medicinal product market availability situation

Information gathered by the Medicines Authority shows that only two orphan medicinal products are purchased and placed on the market and are not provided through the government system for free medicinals): Ecteinascidin 743 (Yondelis) and Sorafenib tosylate (Nexavar). In addition, 38 orphan medicinal products are available within the National Health Scheme (see below).

Orphan medicinal product pricing policy

With regards to reimbursement processes within the National Health Scheme, if an orphan medicinal product is approved through the Exceptional Medicines Treatment Policy, there will be no specific provisions for pricing. However, when a request for introduction into the Government Formulary List is submitted and approved, the pricing policy as for all other new medicines applies. The Pricing Policy for the National Health Scheme was launched in 2010.

Orphan medicinal product reimbursement policy

The Exceptional Medicines Treatment Policy allows for specific provisions for the reimbursement of orphan medicinal products. In 2011, three orphan medicinal products have been introduced into the Government Formulary List and access to treatment has also been granted according to the Exceptional Medicines Treatment Policy.

The following orphan medicinal products are currently being reimbursed[4]: Amifampridine, Anagrelide, Azacitidine, Bosentan, Caffeine citrate, Celecoxib, Cinacalcet, Cladribine, Clofarabine, Colistimethate sodium, Dasatinib, Deferasirox, Dornase alfa, Eptacog Alfa (Recombinant Factor VIIa), Human Cytomegalovirus Immunoglobulin, Iloprost, Imatinib, Lenalidomide, Levamisol hydrochloride, Mercaptopurine liquid, N-Acetylcysteine, Nilotinib, Nitazoxanide, Oxaliplatin, Pegvisomant, Pemetrexed, Plerixafor, Rufinamide, Sildenafil, Stiripentol, Sulfadiazine, Sunitinib, Thalidomide, Thiotepa, Tiopronine, Tobramycin (inhalation solution), Topotecan, Vigabatrin. The drugs available within the National Health Scheme are on the national Government Formulary List and available for dispensing, free of expense to the patients entitled to them.

Other initiatives to improve access to orphan medicinal products

Approval for Compassionate use is a regulatory procedure. It refers to the use of the product which is being considered for approval under the centralised procedure in line with Regulation 726/2004. Off-label use is the use of a product outside its licensed indications. Off-label use is possible at the responsibility of the prescribing physician.

Orphan devices

There are no specific initiatives in place concerning orphan devices in Malta. Indeed, during the open consultation on the Commission Communication "Rare diseases: Europe's challenges", Malta expressed the view that an EU regulation on orphan medical devices "*would neither be necessary nor beneficial"* and that the *"current legal framework already caters for rare diseases"* [5].

Specialised social services

There are limited respite care services and there is an element of co-payment, as with all other residential long-term care services. Therapeutic recreational programmes are also available, and subsidies are available: these services are provided by a private foundation (Inspire Foundation, formerly the Eden Foundation). There is close liaison between health and education authorities to support children in the mainstream schools for the implementation of inclusive education. This includes support to teachers to provide inclusive education at national level. A wide range of services by health care professionals are offered in the community by the health

[4] Source: Directorate of Pharmaceutical Affairs, Ministry of Health the Elderly and Community Care (13 March 2012)

[5] http://ec.europa.eu/health/archive/ph_threats/non_com/docs/r082_en.pdf

care division through Primary Health Care services such as speech Language services and physiotherapy. In addition, there are also social security benefits for those with disabilities.

DEVELOPMENT OF RARE DISEASE ACTIVITIES IN 2011 IN MALTA

National plan/strategy for rare diseases and related actions

A detailed framework that will form the basis for a national strategy for rare diseases is being created. The plan is to propose a request for a first budget for the strategy of rare diseases from the National Budget for 2013. The national strategy will have a time span of a number of years with plans to incrementally implement a number of measures that will aim towards increasing the profile and care services tailored for rare diseases in Malta.

Research activities and E-Rare partnership

IRDiRC

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

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[6] 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.