European Union Committee of Experts on Rare Diseases

2011 REPORT ON THE STATE OF THE ART OF RARE DISEASE ACTIVITIES IN EUROPE

OF THE EUROPEAN UNION COMMITTEE OF EXPERTS ON RARE DISEASES

PART III : ACTIVITIES IN EU MEMBER STATES AND OTHER EUROPEAN COUNTRIES

Joint Action to Support the Scientific Secretariat of the Rare Diseases Task Force/ European Union Committee of Experts on Rare Diseases (N° 2008 22 91)

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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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GENERAL INTRODUCTION

This document was produced by the Scientific Secretariat of the European Union Committee of Experts on Rare Diseases (EUCERD), formerly the Scientific Secretariat of the European Commission's Rare Diseases Task Force (RDTF), through the Joint Action to support the Scientific Secretariat of the former-RDTF/EUCERD (N° 2008 22 91), which covers a three year period (January 2009 – December 2011).

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 drugs up to the end of 2010. A range of stakeholders in each Member State 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 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 or national health authorities

The report is split into three parts:

Part I: Overview of Rare Disease Activities in Europe and Key Developments in 2010
Part II: European Commission and other European activities
Part III: Activities in EU Member States and other European Countries

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.

1. METHODOLOGY AND SOURCES

The main sources of data for this report were are those collected through the systematic surveillance of international literature and the systematic query of key stakeholders carried out in order to produce the OrphaNews Europe newsletter, in addition to data provided by the EUROPLAN associated and collaborating partners in response to the EUROPLAN questionnaire, past reports published by the European Commission (including past reports of the working groups of the Rare Diseases Task Force and EUCERD) and other specialised reports on topics concerning the field of rare diseases and orphan drugs, including the reports of the national conferences organised in the context of the EUROPLAN project. The principal information sources and the collection of data are described in detail here below.

- **European Commission websites and documents**
  Information and documentation from the European Commission was used in order to establish this report, principally accessed through the rare disease information web pages of the Directorate General Public Health[1] and Directorate General Research CORDIS website[2] as well as the site of the European Medicines Agency[3], in particular the pages of the COMP[4] (Committee of Orphan Medicinal Products).

[1] http://ec.europa.eu/health/rare_diseases/policy/index_en.htm
[2] http://cordis.europa.eu/home_fr.html
[3] www.ema.europa.eu
[4] http://www.ema.europa.eu/ema/index.jsp?curl=pages/about_us/general/general_content_000263.jsp&murl=menus/about_us/about_us.jsp&mid=WC0b01ac0580028e30

- **OrphaNews Europe**
  Data from the OrphaNews Europe[5] newsletter for the period 2007-2010 was reviewed and analysed in order to identify initiatives, incentives and developments in the field of rare diseases. The data chosen for analysis and inclusion in the report is mainly information concerning actions of the Commission in the field of rare diseases, the development of rare disease focused projects funded by the Commission and other bodies, and developments in the field of rare diseases at MS level (in particular data concerning the development of national plans and strategies for rare diseases). A similar analysis of the French language newsletter OrphaNews France[6] (which focuses particularly on developments in the field of rare diseases in France) was carried out in order to collect information for the section concerning France.

- **Rare Diseases Task Force publications**
  Various reports of the RDTF have been used as sources of data to collect information on the state of affairs at both EU and Member State levels pre-2010, notably the reports of the RDTF WG on Standards of Care (concerning European Centres of Reference) produced between 2005-2008, including the *RDTF Final Report – Overview of Current Centres of Reference on rare diseases in the EU - September 2005*[7] and the *RDTF Meeting Report: Centres of Reference for Rare Diseases in Europe – State-of-the-art in 2006 and Recommendations of the Rare Diseases Task Force – September 2006*[8], as well as the *RDTF Final Report – State of the Art and Future Directions – March 2008*[9]**.**

- **EUCERD Publications**
  Parts II and III of this report presents an update of the information previously published in the *2009 Report on initiatives and incentives in the field of rare diseases of the EUCERD*[10] (July 2010). The methodology for the production of this previous report is outlined in the introduction. Information on the state of the art of centres of expertise at MS level was also collected during the EUCERD workshop on national centres of expertise and ERNs for rare diseases (8-9 December 2010[11] and 21-22 March 2011[12]).

- **Minutes of the EUCERD**
  The minutes of the first meeting of the EUCERD held on 9-10 December 2011 (and previous minutes of the RDTF meetings) was used in order to identify upcoming initiatives and incentives in the field of rare diseases, and to report on the events held to mark Rare Disease Day 2010.

- **Reports on orphan drugs**
  The information provided for each Member State concerning the state of affairs in the field of Orphan Drugs is taken, when referenced, from the 2005 revision of the *Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products*[13] published in 2006 by the European Commission and produced using data collected by the EMA and Orphanet. This information has been updated when information is available and quoted when still applicable. Another valuable source of information on Orphan Drug policy, at EU and Member State levels was the 2009 KCE 112B report published by the KCE-Belgian Federal Centre of Healthcare Expertise (*Federaal Kenniscentrum voor de Gezondheidszorg/Centre federal d'expertise des soins de santé*) entitled *"Orphan Disease and Orphan Drug Policies" (Politiques relatives aux maladies orphelines et aux médicaments orphelins)*[14]. This report notably provided information for the Member State sections on Belgium, France, Italy, the Netherlands, Sweden and the United Kingdom. The Office of Health Economics Briefing Document *"Access Mechanisms for Orphan Drugs: A*

[5] http://www.orpha.net/actor/cgi-bin/OAhome.php?Ltr=EuropaNews
[6] http://www.orpha.net/actor/cgi-bin/OAhome.php
[7] http://www.eucerd.eu/upload/file/Publication/RDTFECR2005.pdf
[8] http://www.eucerd.eu/upload/file/Publication/RDTFECR2006.pdf
[9] http://www.eucerd.eu/upload/file/Publication/RDTFERN2008.pdf
[10] http://www.eucerd.eu/upload/file/Reports/2009ReportInitiativesIncentives.pdf
[11] http://www.eucerd.eu/upload/file/WorkshopReport/EUCERDWorkshopReportCECERN.pdf
[12] http://nestor.orpha.net/upload/file/EUCERDReport220311.pdf
[13] http://ec.europa.eu/health/files/orphanmp/doc/inventory_2006_08_en.pdf
[14] *Politiques relatives aux maladies orphelines et aux médicaments orphelins*
http://www.kce.fgov.be/index_fr.aspx?SGREF=3460&CREF=13646

Comparative Study of Selected European Countries (No. 52 October 2009)" also provided information on orphan drug availability and reimbursement for the Member State sections on France, Germany, Italy, Spain, Sweden, the Netherlands and the United Kingdom. Information for the overview was also taken from the *Nature Reviews: Drug Discovery* article produced by the COMP/EMA Scientific Secretariat, *European regulation on orphan medicinal products: 10 years of experience and future perspectives*[15].

- **Eurordis website and websites of patient organisation alliances**
  The site of the European Organisation for Rare Diseases[16], and the book *The Voice of 12,000 Patients: Experiences & Expectations of Rare Disease Patients on Diagnosis & Care in Europe* (produced using the results of the EurordisCare[17] surveys), were used to provide information on Eurordis activities and projects and to collect data concerning umbrella patient organisations in each of the European Member States and country-level rare disease events. The websites of national patient alliances were also consulted for information. In addition to this the Rare Disease Day 2010 site[18], maintained by Eurordis, also provided information on events at Member State level[19] concerning Rare Disease Day.

- **EUROPLAN questionnaire to collect information on rare disease activities**
  In the context of the European Project for National Plans Development (EUROPLAN), the partners of the project (who include representatives of national health authorities, expert researchers and clinicians, national alliances of rare disease patient organisations from all MS, and a number of other experts from national health authorities) were addressed a questionnaire and asked to provide detailed information, especially information from sources in their languages, which is more difficultly accessible on the state of rare diseases activities in their country. The structure of the questionnaire (a sample of this questionnaire is included in Annex IV of the *2009 Report on initiatives and incentives in the field of rare diseases of the EUCERD*[20]) followed the structure of the Commission Communication on an action in the field of rare diseases[21]: 19 main questions were formulated in order to collect key data on a number of actions in their country. Since the detail of the answers to these questionnaires varied depending on the information available and the actions specific to the Country, a session of telephone interviews was also carried out to improve the information available, where appropriate. The collection of the information was concluded in October 2009.

- **EUROPLAN national conferences final reports**
  In the context of the EUROPLAN project, 15 national conferences were organised in collaboration with Eurordis and national rare disease patient alliances in 2010 in order to present the Council Recommendation on an action in the field of rare diseases, as well as discuss the Europlan recommendations/guidance document for the development of national plans and strategies in the field of rare diseases[22] and its application at national level. These conferences were attended by a range of stakeholder groups at national level and the final reports[23] of these conferences were presented in a common format for ease of comparison. Information provided in these reports has helped update the information provided in this document. Readers of this report are encouraged to refer to these reports in addition to the present report as they provide further detail of the discussions of national approaches to rare disease policy.

- **Orphanet**
  The Orphanet database was exploited to retrieve data on centres of expertise and the number of genes and diseases tested at Member State level, as well as specific information concerning rare

[15] http://www.ncbi.nlm.nih.gov/pubmed/21532564
[16] http://www.eurordis.org/secteur.php3
[17] http://www.eurordis.org/article.php3?id_article=1960
[18] http://www.rarediseaseday.org/
[19] http://www.rarediseaseday.org/country/finder
[20] http://www.eucerd.eu//upload/file/Reports/2009ReportInitiativesIncentives.pdf
[21] Communication from the Commission to the European Parliament, the Council, the European Economic and Social Committee and the Committee of the Regions on "Rare Diseases: Europe's challenges" (COM(2008) 679 final)
[22] http://www.europlanproject.eu/public/contenuti/files/Guidance_Doc_EUROPLAN_20100601_final.pdf
[23] http://www.eurordis.org/content/europlan-guidance-national-plans-and-conferences#EUROPLAN%20%20National%20Conference%20Final%20Reports

disease research projects, registries, clinical trials and rare disease/orphan drug policies outside of Europe for Part I. Orphanet also provides links[24] to other web-based information services and help-lines which were used to collect information at country-level. The Orphanet Country Coordinators also provided valuable input into the elaboration of information at country level, notably via contributions to OrphaNetWork News. The report produced by the RDPlatform project[25], in particular the report *Rare diseases research, its determinants in Europe and the way forward*[26] was also used as a source for Part I.

- **OrphaNetWork News**
  OrphaNetWork News is the internal newsletter of Orphanet, which communicates information to partners on Orphanet activities in each partner country. The data for this newsletter is collected through a systematic query of Orphanet Country Coordinators and Information Scientists in order to collect information concerning Orphanet country teams' involvement in rare disease meetings and conferences, as well as participation in Rare Disease Day events and partnerships. This surveillance at national level was exploited to provide information for the events section for each Member State.

A selected bibliography and contributions are provided at the end of each Part of the report.

2. REPORT PREPARATION, REVISION AND VALIDATION

The present report provides a compilation of information from the previous report of the EUCERD on the state of the art of rare diseases activities in Europe (*2009 Report on initiatives and incentives in the field of rare diseases of the EUCERD*) elaborated in 2010, which has been updated in 2011 to take into account advances and activities in the field of rare diseases and orphan drugs at EU and MS level in 2010.

Although, in the previous report, information was structured to provide a retrospective of actions at EU level and the state of affairs in the field in each EU Member State (i.e. pre-2009), as well as an inventory of initiatives and incentives undertaken in 2009 at EU and MS level, it was decided in consultation with the EUCERD to take a different approach to this year's report. The current report has merged the information from 'retrospective' and '2009' sections of the previous report and updated it to provide an overview of the state of the art of rare diseases activities in Europe which takes into account the advances up to the end of 2010 whilst providing background information to set these activities in context in order to provide a view of the evolution of activities. The EUCERD also decided that this year's report should include a shorter overview of EU and MS activities in the field of rare diseases (Part I) in addition to the broader 'background' document (Parts II and III).

Once this information was merged and updated using the sources cited above, a draft of each country section was sent in April to a range of key stakeholders in each respective country for their input along with a guidance document providing an explanation of the type of information to include if available for each category. The stakeholders identified for each country included: the MS representatives at the EUCERD and their alternates, the Orphanet Country Coordinators, National Alliances of rare disease patient alliances, the partners of the E-Rare consortium, MS representatives on the COMP, representatives of national competent authorities and other rare diseases experts identified at national level. The collected feedback was integrated to the country reports to elaborate the final drafts which were sent at the end of May 2011 to the EUCERD MS representatives for their final validation, to the best of their knowledge, of the information concerning their respective country.

Part II of the report on activities at European Union level was sent for validation, to the best of their ability, by the representatives at the EUCERD of the European Commission Directorate Generals for Health, Research and Innovation, Enterprise and Industry, as well as the EMA: this process was carried out in May/June 2011 by the Scientific Secretariat of the EUCERD. The European Commission is not responsible, however, for the completeness and the correctness of the information presented in this report.

[24] http://www.orpha.net/consor/cgi-bin/Directory_Contact.php?lng=EN
[25] http://www.rdplatform.org/
[26] http://asso.orpha.net/RDPlatform/upload/file/RDPlatform_final_report.pdf

Part I was the last part of the report to be elaborated: the overview of the state of the art of rare diseases activities in Europe and key developments in 2010 is the result of an analysis of the information collected for Parts II and III. Part I was drafted by the Scientific Secretariat of the EUCERD before validation by the Bureau of the EUCERD acting as the Editorial Board for the present report.

3. REPORT STRUCTURE

The report is structured into three main parts: Part I consists of an overview of the activities in the field of rare diseases in Europe at EU and MS level as well as a short summary of key developments at EU and MS level in 2010; Part II concerns activities at EU level; Part III concerns activities at EU MS level, as well as five other non-EU European countries where information was available. Each part is followed by a selected bibliography outlining the sources used to produce the part of the report, which includes a list of the European Commission documents referred to in the report and a list of web addresses by country listing national sources of information on rare diseases and links to documents concerning national plans or strategies for rare diseases when in place. Each part is also followed by is a list of contributors the report, organised by country with mention of the validating authority in each country, and stating their contribution to the current and/or previous report. A list of frequently used acronyms has also been included in each part to ease reading.

Part I provides an overview of the state of the art of rare disease activities in the field of rare diseases in Europe and key developments in 2010 at EU and MS level. This part thus serves as a summary to highlight key areas of the Parts II and III, which serve to provide more detailed background information at EU and MS level. The overview is structured into a number of topics: political framework, expert services in Europe research and development, orphan drugs and therapies for rare diseases, patient organisations and information services.

Part II of the report on activities at EU level is organised slightly differently to the last edition of the report where activities were presented in sections corresponding to the European Commission Directorates General (DG) of the European Commission implicated in the field of rare diseases. In the present report, activities concerning rare diseases and orphan drugs at EU level are split into four sub-sections:

1. EC activities related to rare diseases in the field of public health
2. EC activities related to rare diseases in the field of research
3. EC activities in the field of orphan drugs and therapies for rare diseases
4. Other European rare disease activities (i.e. meetings at European level and selected transversal EU activities).

The sub-section concerning the EC activities actions in the area of Public Health is divided into three parts: an overview of EC DG Health and Consumers' activities in the field of public health, activities in the field or rare diseases funded by DG Health and Consumers, and activities of DG Health and Consumers indirectly related to rare diseases.

The sub-section concerning the EC activities in the field related to research in the field of rare diseases presents information concerning DG Research and Innovation's 5th, 6th and 7th framework programmes for research, technological development and demonstration activities related to rare diseases, as well as information concerning the International Rare Disease Research Consortium (IRDiRC) and Open Access Infrastructure for Research in Europe (OpenAire) initiatives.

The sub-section concerning EC activities in the area of orphan drugs and advanced therapies for rare diseases is organised accordingly: European legislation concerning orphan medicinal products and related activities, European Medicine Agency's (EMA) activities in the field of orphan medicinal products and therapies for rare diseases, EMA Committee for Orphan Medicinal Products' activities, EMA Committee on Human Medicinal Products' activities, European legislation and activities in the field of clinical trials, European legislation and activities in the field of advanced therapies, European legislation and activities in the field of medicinal

products for paediatric use, other EMA activities and initiatives relevant to rare diseases and orphan drugs, EU-USA collaboration in the field of orphan medicinal products and other EC activities and initiatives in the field of orphan drugs.

The sub-section concerning other European rare disease activities provides information on transversal rare disease activities and initiatives at EU-level and includes information on the High Level Pharmaceutical Forum, actions undertaken in the scope of recent European Union presidencies, the E-Rare ERA-Net for rare diseases and outcomes of European and International rare disease congresses and conferences in 2010.

Part III concerns the activities in the field of rare diseases in each of the 27 Member States plus Norway and Switzerland as EEA countries, Croatia and Turkey as candidates for EU membership, and Israel: Iceland has chosen to not contribute a country report this year. These sections are organised in alphabetical order by country.

The information on each country is clearly divided into a number of categories:

- Definition of a rare disease
- National plan/strategy for rare diseases and related actions
- Centres of expertise[27]
- Registries
- Neonatal screening policy
- Genetic testing[28]
- National alliances of patient organisations and patient representation;
- Sources of information on rare diseases and national help lines;
- Best practice guidelines
- Training and education initiatives
- Europlan national conference
- National rare disease events in 2010[29]
- Hosted rare disease events in 2010[30]
- Research activities and E-Rare partnership
- Participation in European projects[31]
- Orphan drugs (Orphan drug committee, Orphan drug incentives, Orphan drug availability[32], Orphan drug reimbursement policy, Other initiatives to improve access to orphan drugs, Orphan drug pricing policy)
- Orphan devices
- Specialised social services

The choice of categories of information for inclusion in this year's report were discussed by the EUCERD at their first meeting (9-10 December 2010): categories new to this year's edition include genetic testing, Europlan national conferences, orphan devices, other initiatives to improve access to orphan drugs and orphan drug pricing policy. The categories for which information is provided depends wholly on the information available following data collection from the described sources and contact with stakeholders. If no detail has been given for a topic, the mention "no specific activity/information reported" has been added.

[27] The term "official centre of expertise" used in this report means officially designated via a (ministerial) procedure.
[28] This section contains data extracted in May 2011 from the Orphanet database of the number of genes for which there is a diagnostic test registered in Orphanet and the estimated number of diseases for which diagnostic tests are registered in Orphanet (the term 'estimated' is used as the concept of a single disease is a variable one).
[29] As announced in OrphaNews Europe.
[30] As announced in OrphaNews Europe.
[31] Past and ongoing participation in pilot European Reference Networks, DG Research and Innovation financed projects, EUROPLAN and European registries. Some countries have added information on additional European projects.
[32] Contacts were asked to provide information on availability of orphan drugs (i.e. which drugs are registered/marketed at national level): some countries instead provided information on which drugs are accessible (i.e. reimbursed, on a positive drug list etc.). It is explicitly explained in each case to which of these concepts is being referred.

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 (Iceland, Switzerland, Norway)
EMA - European Medicine's 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 Rare Diseases Patient Organisation
FDA - US Food and Drug Administration
HLG - High Level Group for Health Services and Medical Care
HTA - Health Technology Assessment
JA - Joint Action
MA - Market Autorisation
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

1. EUROPEAN UNION MEMBER STATES

1.1. AUSTRIA

Definition of a rare disease

In 2010 there was still no official definition of rare diseases in Austria; on an informal basis, stakeholders in Austria accept the European Orphan Drug Regulation definition of a prevalence of no more than 5 in 10'000 individuals. In the national plan of action (still under development; see below) it is foreseen to officially adopt the European Orphan Drug Regulation definition of a prevalence of no more than 5 in 10'000 individuals.

National plan/strategy for rare diseases and related actions

In May 2009, the highest advisory board ("Oberster Sanitätsrat") of the Austrian Ministry of Health (BMG) established a subcommittee ("Unterkommission") for rare diseases, consisting of 17 members from 13 different organisations or institutions (covering the main stakeholders in the field). This working group was headed and managed by the Austrian Orphanet team and was in charge to set the grounds for a national plan of action for rare diseases in Austria. It was the first time ever that an expert committee of this size, covering a broad spectrum of viewpoints, was working on rare diseases in such a comprehensive manner, with topics ranging from the description of the situation of rare diseases in general to legal and ethical aspects, equality in legal and practical terms, the identification of concrete problems, bottlenecks and restrictions that patients, relatives, physicians and scientists are confronted with, and, finally, the identification of possible measures and strategies aiming to improve the situation, to combat (structural) deficits, to optimise health care pathways, and to minimise disease burden wherever possible.

In its first sessions, the working group decided to adopt a step-by-step working strategy with the following deliverables:

- A general text document (the "framework" of the action plan, deliverable D1) containing:
  - i. a comprehensive introduction into the topic;
  - ii. the definition of approximately nine to ten strategic priorities covering the most relevant needs in Austria (titles only);
  - iii. a final section defining the general mode of monitoring.
- Detailed chapters for the key strategic priorities previously defined in the general text document (deliverables D2-D10/11).

It was further decided that, as soon as a draft for any of the chapters/deliverables is finished, this draft will be reviewed by the highest advisory board (see above) and, after approval, referred to the political decision process. This strategy was developed to ensure that:

- The final document fulfils the standards of a comprehensive plan of action;
- Individual priorities can be implemented as soon as possible without any delay caused by the elaboration of the other deliverables.

In the few following meetings, the committee worked on the content and the details of deliverable D1.

In the first meeting in 2010, the working group adopted the major parts of deliverable D1, i.e. the introductory part and the final definition of the strategic priorities covered by the national plan. While most parts were decided unanimously, single aspects with regard to the order of the strategic priorities were adopted with a qualified majority. The nine strategic priorities of the Austrian national plan of action are as follows:

i) Recognition of the specificity of rare diseases;
ii) Improving health care pathways by:
  a) Defining and establishing specialised centres of expertise and centres of competence for groups of related rare diseases (see also chapter on "Centres of Expertise" below);
  b) Establishing a national coordination centre for rare diseases (to administer and accompany, amongst other activities, the certification of specialised centres, as well as to establish and coordinate a national network of these centres);

c) Establishing and sustaining support for a comprehensive information system for rare diseases (i.e. Orphanet in Austria);
d) Establishing a central office for rare diseases;

iii) Improving the diagnostic pathway and extending diagnostic capabilities/possibilities (including the development and clinical implementation of screening tests);
iv) Improving therapeutic options/procedures and the access to therapy;
v) Establishing selective research programmes specifically addressing rare diseases;
vi) Improving awareness and knowledge about rare diseases (addressees: general public, health care personnel, professionals);
vii) Improving the epidemiological knowledge on rare diseases (establishment of a national/cross-border registry);
viii) Establishing a permanent expert group for rare diseases at the Austrian Ministry of Health;
ix) Recognition of the attainments of patient support groups.

All members of the subcommittee agreed that out of these nine strategic priorities, the chapter on "Improving health care pathways" constituted the priority with the highest relevance, and that this chapter should therefore be elaborated first. Accordingly, in the three final meetings, the working group focussed on:

(a) The adaption of the RDTF criteria for centres of expertise to the specific needs of a small to medium-sized country like Austria, considering in parallel the framework requirements (for instance pre-existing structures) in the healthcare system;
(b) The development of a modular system of criteria defining expert clinics with outstanding 'pure clinical', as well as 'combined clinical and scientific' expertise;
(c) The definition of a possible certification process for these types of expert centres;
(d) The establishment of a national coordination centre with well-described first duties and responsibilities, as well as additional (optional) modules that might be relevant – and then implemented – in the future;
(e) The functional integration of Orphanet Austria into this coordination centre in order to maximise the synergies between the two structures.

While the work on the criteria for the different types of expert clinics could not be finished within 2010, the establishment of a national coordination centre for rare diseases, and the functional integration of Orphanet into this centre, was eventually approved at the last meeting of the working group in November 2010.

The coordination centre was officially established on 1 January 1011 at the Austrian Health Institute; it is thus the first measure of the national plan that has been implemented (including the sustained funding of Orphanet as the national information system for rare diseases). In parallel, it is intended to be the main driving force to finish the elaboration of the national plan of action within the coming year.

In December 2010, the subcommittee for rare diseases was dissolved (as part of the *Oberster Sanitätsrat* that had finished its regular three-years-period). It will be replaced in 2011 by a new expert committee, affiliated directly to the Ministry of Health.

Centres of expertise

Currently, there are no officially designated centres of expertise in Austria; informally, few well-recognised centres exist with an outstanding expertise in their field, the best known probably being the "Spezialambulanz Genodermatosen" for Epidermolysis bullosa in Salzburg. The Austrian Ministry of Health strongly supports the concept of such centres. It is therefore expected that further centres of expertise will be identified and that all these centres will be officially designated as soon as (a) the final criteria for European centres of expertise have been developed by the European Union Committee of Experts on Rare Diseases, (b) these criteria have been adapted to meet the legal requirements, and to take into account pre-existing structures of the health care system, in Austria and integrated into the national plan of action for rare diseases, and (c) this national plan has been implemented successfully.

The nomination of centres of expertise and related expert clinics (potentially called "centres of competence") constitutes one of the main actions in the national plan for rare diseases, covered by the strategic priority "Improving health care pathways".

Registries

Currently, no nationwide, general, comprehensive registry for rare disease patients exists in Austria. Approximately 13 registries for individual rare diseases or groups of rare diseases are run by specialised clinics or networks of experts from different clinics. These registries are primarily maintained on a private/institutional

basis, in many instances "in kind" by the expert teams; some registries are additionally supported by corresponding patient support groups. Some of the European registries Austrian teams participate in are EUROCARE CF, AIR, EMSA-SG, EUROCAT and ENRAH. Actions in this area are included in the National Plan for Rare Diseases ("National/Cross-border registry"). The Austrian Ministry of Health did not have any budget in 2010 to financially support selected individual patient registries.

Neonatal screening policy

Since the early 1960s, Austria has a well-established, nationwide newborn screening (NBS) program that is carried out for practically all newborns in one screening centre, operated by the University Children's Hospital of the Medical University of Vienna. The Austrian NBS program is one of the most comprehensive programs worldwide and screens for the following diseases and conditions: adrenogenital syndrome, carnitine-acylcarnitine translocase deficiency, carnitine palmitoyltransferase I deficiency, carnitine palmitoyltransferase II deficiency, carnitine transporter defect, citrullinemia / argininosuccinate lyase deficiency, congenital hypothyroidism, cystic fibrosis, galactosaemia, glutaric acidemia type I, glutaric acidemia type II / multiple acyl-CoA dehydrogenase deficiency, homocystinuria and hypermethionemia, isobutyryl CoA dehydrogenase deficiency, isovaleric academia, β-Ketothiolase deficiency, long-chain 3-hydroxy acyl-CoA dehydrogenase deficiency / trifunctional protein deficiency, maple syrup urine disease, medium-chain acyl-CoA dehydrogenase deficiency, methylmalonic aciduria / propionic academia, multiple carboxylase (holocarboxylase) deficiency, phenylketonuria and hyperphenylalaninemia, short-chain acyl-CoA dehydrogenase deficiency, tyrosinemia type I (II), very long-chain acyl-CoA dehydrogenase deficiency, 2-Methyl 3-hydroxy butyryl-CoA dehydrogenase deficiency, 3-Hydroxy-3-methylglutaryl-CoA lyase deficiency, 3-Methylcrotonyl-CoA carboxylase deficiency, and 3-Methylglutaconic aciduria type I. This screening panel remained unchanged in 2010.

The scientifically based NBS for lysosomal storage disorders has been further developed since 2009 and now includes Mucopolysaccharidosis (MPS) type 1 disease in addition to the previous disorders (Gaucher, Fabry, Pompe, Nieman-Pick Type A/B). At end of 2010 it had not been decided whether (and when) any of these diseases should be included in the national program.

Genetic Testing

Molecular genetic testing in Austria is regulated by the so-called "Gentechnikgesetz" (GTG), first established in 1994[33] and last revised in 2005[34]. The Gentechnikgesetz covers all legal, ethical and (bio-) safety aspects regarding diagnostics and research in the field of molecular genetics (including generation and handling of genetically modified organisms). In the chapter on human molecular genetic testing, genetic tests are subdivided into the following four types:

(a) Type 1 comprises tests to identify either concrete somatic changes in the number, structure, or sequence of chromosomes, genes or DNA fragments or concrete chemical modifications in chromosomes, genes or DNA fragments in patients suffering from a clinically manifested and diagnosed disease (for instance, the search for a somatic mutation or altered methylation status in a tumour tissue sample);
(b) Type 2 covers tests searching for germline mutations in patients suffering from a clinically manifested and diagnosed disease;
(c) Type 3 comprises tests to establish the genetic risk/predisposition of a healthy individual to potentially develop a genetic disease in the future, or to establish the carrier status of a healthy individual for a certain genetic disease, in disorders where prophylaxis or treatment are available;
(d) Type 4 covers tests to establish the genetic risk/predisposition of a healthy individual to potentially develop a genetic disease in the future, or to establish the carrier status of a healthy individual for a certain genetic disease, in disorders where prophylaxis or treatment do not exist.

While for genetic tests of categories 1 and 2 no authorisation is necessary, tests of categories 3 and 4 can only be performed in laboratories officially authorised by the Austrian Ministry of Health. Institutions seeking authorisation have to register their activity and apply with a detailed description of their laboratories, equipment, technical procedures, quality schemes, and experience in genetic testing. The formal authorisation for the respective genetic test is granted after an evaluation process, which includes consultation of the scientific board of the Committee on Gene Technology ("Gentechnikkommisson").

[33] http://www.bmg.gv.at/cms/home/attachments/7/8/8/CH1060/CMS1226929588865/510_1994.pdf
[34] http://www.bmg.gv.at/cms/home/attachments/7/8/8/CH1060/CMS1226929588865/gtg-nov._11-05.pdf

Laboratories performing genetic testing in Austria are listed in a special registry ("Genanalyseregister") administrated by the Ministry of Health. Of note, the designation "reference laboratory" as an official term does (currently) not exist in Austria.

In Austria, reimbursement is primarily a responsibility of the individual states and not centrally regulated (of note, some exceptions exist). This responsibility is further split between two different types of institutions, depending on whether the patient had been treated (a) in a hospital as an inpatient or (b) in an outpatient clinic or private practice (general practitioner or consultant of a specific medical discipline). In the first case, the costs of any type of diagnostic test or treatment have to be paid from the budget of the hospital. The hospital, in turn, is indirectly reimbursed by the health fund of the respective state ("Landesgesundheitsfonds") on the basis of an average daily rate calculated for one patient in this specific hospital and department/clinic. However, hospitals have to make efforts to not exceed the budgets allotted to them for each calendar year. In the second case (outpatient clinic, private practice), reimbursement is the responsibility of the health insurance fund of the patient. In this instance, specific tariffs are calculated by the insurance fund for each type of service and services are reimbursed according to the tariff catalogue. Of note, only services that have been successfully negotiated with the insurance fund and integrated into their individual tariff catalogue are eligible for reimbursement.

Taking into account this dual reimbursement system with all its regulations, the reimbursement of genetic testing is as follows:

(a) As an obligatory prerequisite, all tests have to be officially accepted/approved by the (local) insurance fund and integrated into their tariff catalogue (either as a specific single test, or on the basis of average calculations for long versus short genes, number of exons, complexity of the analysis, or other criteria);
(b) For inpatients, the hospital covers the costs according to the tariffs of the laboratory performing the test;
(c) For outpatients, the respective insurance company carries the costs; however, it is possible that certain analyses (depending on the internal regulations of insurance fund) require an ex-ante approval by the head consultant ("Chefarzt") of the insurance fund, even if the analysis is requested/recommended by a specialist for human genetics (in Tyrol, for instance, all genetic determinations that cost more than €1'000 have currently to be authorised by the "Chefarzt").

Genetic testing abroad is possible as soon as the test is strongly indicated for an individual patient and cannot or not easily be performed within the country (again, the same rules apply as above and the determination has obligatorily to be approved ex-ante either by the respective insurance fund or – for inpatients - by the medical director of the hospital).

Diagnostic tests are registered as available in Austria for 110 genes and an estimated 173 diseases in the Orphanet database[35].

National alliances of patient organisations and patient representation

To date, there is still no specific national alliance of patient organisations for rare diseases in Austria. Nevertheless, the situation has developed significantly within the past year.

Initiated and organised by the Austrian Orphanet team, representatives of the individual patient organisations participated in common activities and events during the past few years. Some highlights were the Rare Disease Day events and the first national congress for Rare Diseases in Mariazell (see below). This led to an increased confidence between the individual groups, as well as the growing awareness, that a common effort will be necessary to approach the general problems of rare diseases.

At the conference in Mariazell, patients and representatives of different patient organisations had the opportunity to experience the advantages, perspectives, and development potential that would be brought about by an active umbrella organisation using the example of EURORDIS, and to discuss this topic extensively. At the end of the conference, about 20 groups made an informal declaration of intent to establish a national alliance of rare disease patient organisations. Of note, the Pharmig, one of several associations of the Austrian pharmaceutical industry, stated their intention to participate as one possible partner, amongst others, in the core funding of this national alliance.

Apart from this group effort dedicated specifically to rare diseases, general alliances of patient organisations (both for rare and non-rare diseases) do exist on the state level (ARGE Selbsthilfe Carinthia, Upper Austria, Lower Austria, Salzburg, Styria, Tyrol, Vorarlberg, and Vienna). They are united under the supra-

[35] Information extracted from the Orphanet database in May 2011.

umbrella *Arbeitsgemeinschaft (ARGE) Selbsthilfe*, which is located in Vienna. The *ARGE Selbsthilfe* can provide limited funding (up to €900 for a period of 6 months with repeat applications possible) for all patient organisations (including those in the rare diseases field), however, funding is confined to support the formation of a new patient organisation or to provide interim aid for an existing one bridging a limited time gap. Very recently, the Austrian Ministry of Health decided to structurally support the *ARGE Selbsthilfe* by providing an office (including one secretary position) from pre-existing ressources of the Austrian Health Institute (GÖG). Apart from that, for the near future, no further specific support of rare disease patient organisations is planned by the ministry.

Further institutions supporting patient organisations for non-rare, as well as rare diseases (all located in Vienna) are the *Selbsthilfe-Unterstützungsstelle für gesundheitsbezogene Selbsthilfegruppen (SUS)* and the *Martha-Frühwirt-Zentrum*. The SUS (as part of the Fonds Soziales Wien) provides all kinds of administrative support, but does so without funding; similarly, the Martha-Frühwirt-Zentrum offers administrative support and rooms or offices for the activities of patient organisations, but again without direct funding.

Thematically restricted support for patient organisations will possibly be part of the future National Plan for Rare Diseases, integrated into the priority "Improving awareness and knowledge about rare diseases".

Already in 2009, representatives of two rare disease patient organisations were mandated - during a meeting of active and interested patient organisations in the rare disease field – to represent patients in the subcommission for rare diseases and to take part in the development of the national plan of action. When the subcommission will be replaced in 2011 by a new expert committee which will be directly located at the Ministry of Health (see above), the number of patient representatives will be increased (in parallel to the number of medical specialists).

Sources of information on rare diseases and national help lines

Orphanet activities in Austria

Since 2002 there is a dedicated Orphanet team in Austria, currently hosted by the Institute of Neurology at the Medical University of Vienna. This team is in charge of collecting data on rare disease related services (specialised clinics, medical laboratories, ongoing research, registries, bio-banks, clinical trials and patient organisations) in their country for entry into the Orphanet database. The team was designated in 2010 as the national Orphanet team for Austria by the Austrian Ministry of Health, as was a second new team at the Austrian Health Institute (Gesundheit Österreich GmbH, GÖG). The strategy behind this "two team approach" is to structurally integrate Orphanet (on a long run) into the Austrian health care system. Therefore, the teams are no rivals but cooperate very closely.

In 2010, the Austrian country team continued to finance and maintain a self-developed country website, launched back in 2008, in order to provide basic information on the Orphanet database and the local Orphanet team, as well as to raise public awareness on rare diseases in general. To this end, the team reported about major events and activities, like the rare disease day 2010 or the national congress on rare diseases in Mariazell (see below), organised either by Orphanet Austria itself or by other stakeholders. As a new web tool, an interactive forum on rare diseases was developed and integrated into the country website intended as one national discussion platform on rare diseases; however, this tool has been used only sporadically despite several attempts to motivate in particular patients to exchange their knowledge, views and questions about (specific) rare diseases. In 2011, a new Orphanet country site, adapted to the new common layout of Orphanet country websites, will be developed as soon as the Joint Action Orphanet Europe has started.

Official information centre for rare diseases

Until 2010, Orphanet was the only official source of information specific to rare diseases in Austria. In January 2011, the national coordination centre for rare diseases was established at the Austrian Health Institute (as part – and first structural measure – of the national plan of action), financed by the Austrian Health Ministry. Orphanet Austria was integrated into this coordination centre to enable maximum synergy between the two structures.

Help line

There is currently no official nation-wide national helpline for rare diseases in Austria. In April 2010, a more regional helpline was established in Salzburg, focusing primarily on rare genetic skin disorders (genodermatoses) and metabolic disorders. The helpline is operated by two physicians and can currently be contacted on one afternoon per week. Although announced locally, it can of course be contacted from all around the country. According to the information available, it is funded on a private basis.

Other sources of information

Further sources of information on rare diseases include:

- Disease-specific websites of patient organisations. A number of patient organisations for specific rare diseases – or groups of rare diseases – exist in Austria that host excellent websites providing extensive and very detailed information on "their" rare disease/group of rare diseases (including information on the medical background, symptoms, diagnostics and treatment/care of patients);
- Some medical departments also host websites with comprehensive and useful information on those rare diseases they are focussing on;
- Further websites, under development in 2010, will go online during the next year.

Best practice clinical guidelines

No specific information reported.

Training and education initiatives

The academy of the Epidermolysis Bullosa (EB) House hosts training workshops for epidermolysis bullosa on a regular basis. In addition, the Department of Dermatology of the private Paracelsus Medical University in Salzburg participated in the GENESKIN project in 2010, organising courses on rare genetic skin diseases. The 1st Mariazeller Gesundheitsdialog (see below) covered rare diseases from several medical disciplines (dermatology, immunology, haemato-oncology, haemophilia, infectiology, cardiology, nephrology/andrology, pulmonology, and paediatrics/metabolic disorders) and was approbated by the Austrian Medical Association as a 7 hour training-seminar.

Europlan national conference

Austria did not hold a Europlan national conference in 2010.

National rare disease events in 2010

The central rare disease event in Austria was the 2nd Austrian March for Rare Diseases held in Vienna on February 27, 2010. Approximately 350 participants - patients, physicians, researchers, supporters, representatives of the biomedical industry - came together to raise awareness of rare diseases. This year, the route led through the inner city of Vienna from the Opera house to the Hofburg castle. The march was followed by a closing event with lunch buffet and speeches. The event was widely covered by the media.

On February 28, the 2nd Rare Diseases Information Day took place in Salzburg. It was organised and supported by several patient organisations.

On June 11, 2010, the first Regional Forum on Rare Diseases took place in Salzburg, organised by the university departments of dermatology and paediatrics and adolescent medicine, the International Forum Gastein, along with the newly founded Institute for inherited metabolic disorders. It comprised lectures on the interdisciplinary challenge of rare metabolic disorders, the orphan drug legislation, and Orphanet as an information platform. Several experts, primarily from the private Paracelsus Medical University Salzburg, reviewed their specialties with a particular focus on selected rare diseases or groups of rare diseases. The meeting finished with a lively panel discussion on the situation of rare diseases in Austria, focusing on current difficulties and potential solutions with a particular focus on the establishment of certified centres of competence and on reimbursement policies.

On June 12, 2010 a regional symposium was held in Vienna with the topic "10 years of enzyme replacement therapy for Anderson-Fabry disease".

The Mariazeller Gesundheitsdialog under the scientific auspices of Orphanet Austria (Mariazell, October 15-16, 2010) was entirely dedicated to the field of rare diseases. It was the first national congress on rare diseases in Austria that brought all the different stakeholders in the rare disease field (physicians and scientists, patients and patient organizations, politicians, health care and social welfare representatives, pharmaceutical industry) together for a common dialogue. There were three key aspects: 1) for physicians: a training program on rare diseases from different medical disciplines (see above); 2) for patients: apart from lectures on patients' rights, fund raising, and conflict management, the most important part was the introduction of EURORDIS as an excellent example of a well organised umbrella organisation, followed by an intensive discussion; and 3) introduction of the EU-initiative for a national action plan and the state of play regarding its implementation in Austria; the highlight was a mixed panel and plenary discussion with all participants.

Hosted rare disease events in 2010
Austria did not host any international rare disease related events in 2010.

Research activities and E-Rare partnership
Research activities
Currently, there is no specific and explicit funding policy for rare diseases in Austria. In theory, funding is available through grant applications at different funding bodies (for instance, the *Fonds zur Förderung der wissenschaftlichen Forschung (Austrian Science Fund; FWF)*, the *Nationalbank*, or minor resources such as the *Fonds des Bürgermeisters der Bundeshauptstadt Wien*); however, funding follows a bottom-up approach, meaning that applications from all medical disciplines and, in some instances, totally unrelated medical, as well as non-medical, research fields compete each other in a peer-review selection process, eventually resulting in a selection bias towards projects addressing more common diseases.

An alternative source of funding is provided by occasional project calls launched by the Austrian Ministry of Science. In the past 5 years, one of these calls was dedicated to rare diseases. Moreover, some fundraising patient organisations finance rare disease research projects. One strategic priority in the Austrian national plan will be the implementation of a defined, separate funding budget in the main existing research bodies, which will be specifically dedicated for research on rare diseases, as aforementioned in the National Plans segment ("Establishing a selective funding for research on rare diseases").

E-Rare
Austria was not an official partner in the E-Rare consortium before 2009 and did not participate in the first E-Rare Joint Transnational Call in 2007. The Fonds zur Förderung der wissenschaftlichen Forschung (Austrian Science Fund)[36] joined the second E-Rare Joint Transnational Call in 2009, and around €580,000 of funding was granted for Austrian teams participating in 3 projects. Austria will participate in the 3rd Joint Transnational Call in 2011.

Participation in European projects
Austrian teams participate, or have participated, in the following European Reference Networks for rare diseases: EUROHISTIONET, NEUROPED (main partner), Paediatric Hodgkin Lymphoma Network and PAAIR. Austrian teams participate, or have participated, in European research projects for rare diseases including: BNE, CLINIGENE, EMSA-SG, EMINA, ENRAH, ENCE-PLAN, EURIPFNET, EUROTRAPS, EURO-LAMINOPATHIES, EUROPEAN LEUKEMIA NET, EURO-IRON1, GENESKIN, LYMPHANGIOGENOMICS, MYELINET, NEUTRONET, NEUROPRION, PERXISOMES, PNSEURONET, PROTHETS, PULMOTENSION, PWS, RHORCOD, RD PLATFORM, SIOPEN-R-NET and SARS/FLU-VACCINE. Austrian teams contribute to the following European registries: AIR, EUROCARE CF, EMSA-SG, EUROCAT and ENRAH. Austria contributes to the EUROPLAN project. Austria is part of the SOPEN-R-NET research network. In addition, Austrian experts are represented in initiatives on the field of orphan drugs lead by the European Commission DG Enterprise.

Orphan drugs
Orphan drug committee
There is currently no committee for orphan drugs in Austria.

Orphan drug incentives
According to information collected for the publication of the first *"Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products"* the Austrian Drugs Act (2001) provides for *"the waiving of fees (e.g. for marketing authorisation or variations) for orphan drugs authorised through the national procedure (applicable until 20 November 2005, date from which the centralised route of marketing authorisation of designated orphan medicinal product became mandatory)[37]".*

Orphan drug availability
As soon as marketing authorisation is provided, orphan drugs are available quite quickly in Austria (regarding possible delays in the provision of orphan drugs see below in the chapter on reimbursement policy). Actions

[36] http://www.fwf.ac.at/
[37] *Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products. Revision 2005* (p7).

are foreseen by the National Plan for Rare Diseases in this area ("Recognition of the specificity of rare diseases" and "Improving equal access to established therapies") to improve availability. However, any kind of off-label use is not well accepted by public authorities in Austria.

Orphan drug reimbursement policy

According to the Austrian Social Insurance Law (ASVG) insured patients must be granted all necessary forms of medical treatment in a sufficient and appropriate way as long as adequacy of resources used is reasonable[38]. Contract physicians are entitled to prescribe all medicines included in the Austrian Reimbursement Code (EKO)[39] - considering specific rules (e.g. second-line therapy) - on behalf of the sickness funds (general reimbursement). Specific medicines require ex-ante or ex-post approval of a head physician ("Chefarzt") of the contracting sickness fund. The same is true for exceptional cases where a pharmaceutical is not listed in the Reimbursement code. To obtain the approval the prescribing physician needs to send a written request to the sickness fund via an online tool. A reply is sent within 30 minutes. Decisions of the sickness fund's head physicians depend on medicinal and pharmacological necessities as well as economic criteria. In practice, orphan medicines usually belong to a group requiring prior approval.

If it is determined that a medicine is best applied in a hospital setting, e.g. because of the complexities of administration (as it is for instance the case for "Elaprase", a drug for an enzyme replacement therapy), then there is no need for reimbursement in the outpatient setting. In exceptional cases, reimbursement may be still approved, however, if the administration is done on an outpatient basis and this is medically justified. For orphan medicines not included in the EKO, the attending physician may still seek approval from the sickness fund (e.g. requesting administration of the orphan as out-patient treatment).

In case a patient is seeking to obtain approval for treatment outside of Austria, the same procedure as described above applies (i.e. ex-ante approval by the head physician). In the last three years no treatment with orphans taking place outside of Austria has been approved, however, several patients underwent diagnostic testing in other countries, e.g. in Germany.

Interviewed national experts explained that patients could experience delays in the provision of orphan drugs due to fragmented funding responsibilities. The public payer of medicines in Austria depends on the place of treatment, i.e. the owners of hospitals having to pay for intramural care whereas the regional sickness funds cover medicines prescribed in out-patient care. Sickness funds pay a lump sum for the provision of in-patient care for their insured to the regional hospital funds.

In 2009 public expenditure per prescription for orphan drugs amounted to €2,754 and in 2010 to €2,771, which is a 0.67% rise. Altogether the Austrian Social Insurance spent €85 million on orphan drugs in 2010.

Other initiatives to improve access to orphan drugs

No specific information reported.

Orphan drug pricing policy

In case a marketing authorisation holder applies for reimbursement in Austria, the product falls under statutory price regulations. This means that the maximum reimbursement price may not exceed the EU-25 average price; in most cases this price is subject to negotiations between the main Association of Austrian Social Security Institutions and the marketing authorisation holder. In case the product is not reimbursed the price of the product can freely be set by the manufacturer. Unauthorised orphan drugs may be imported on case-by-case decisions. The majority of orphan drugs are dispensed in hospitals.

Orphan devices

No specific information reported.

Specialised social services

No specific activity reported.

[38] Art. 133 ASVG 1955, regulating the extent of medical treatment [Art. 133 ASVG 1995; BGBl. No. 189/1955]

[39] Art. 31.3(12) ASVG, on the publication of the Reimbursement Code EKO (Art. 31.3(12))

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