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European Union Committee of Experts on Rare Diseases

# 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 AUSTRIA

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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 Austria. 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 AUSTRIA

**Definition of a rare disease**

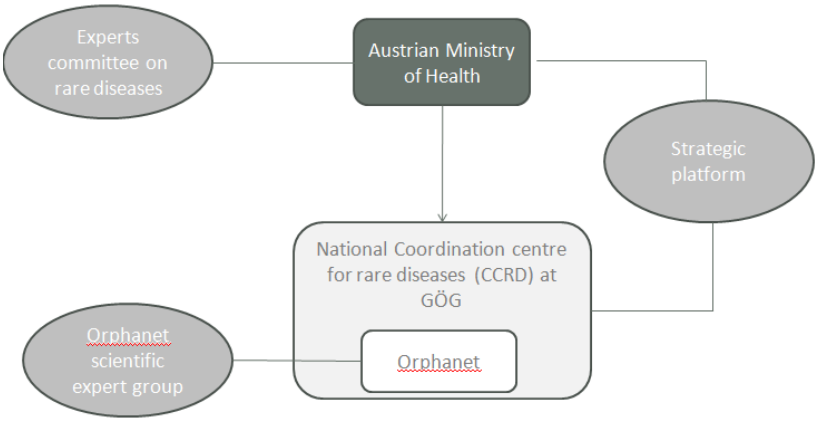
In 2010 there was still no official definition of rare diseases in Austria; on an informal basis, stakeholders in Austria accept the European Regulation on Orphan Medicinal Products definition of a prevalence of no more than 5 in 10'000 persons. In the national plan of action (still under development;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

In response to a petition by health professionals and patient organisation for a national action plan on rare diseases in Feb 2008 and considering the recommendations of the European Council of 8 June 2009 to implement a national action plan on rare diseases until 2013 the highest Austrian Health Advisory board ("Oberster Sanitätsrat") of the Austrian Ministry of Health (BMG) established a subcommittee for rare diseases in May 2009, consisting of 17 members from 13 different organisations or institutions (covering the main stakeholders in the field). This working group was managed by the Austrian Orphanet team and laid the foundation for a national plan of action for rare diseases. It was the first time in Austria that an expert committee of this size, covering a broad spectrum of viewpoints, was working on rare diseases in 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.

However, due to the many topics on the agenda and the lack of resources of the Board Members the Austrian Ministry of Health decided to establish a National Coordination Centre for Rare Diseases (CCRD, Nationale Kontaktstelle für Seltene Erkrankungen, NKSE). The CCRD was established by 1 January 2011 at the Austrian Health Institute (Gesundheit Österreich GmbH, GÖG) and has a team of 1.4 full time equivalents and integrates part of the Austrian Orphanet team. Most members of the subcommittee for rare diseases are still involved in the topic as they kindly accepted their appointment to the Expert committee on rare diseases that was established in mid 2011 by the Ministry of Health (see Figure 1 below). In addition to the previous structure a strategic platform was set up, the is composed of Ministry of Health delegates, academic experts and payers (Austrian provinces and the Main association of the Austrian social security institutions),

*Figure 1: Organisational Chart of the Austrian CCRD in 2011*




The establishment of the CCRD was one of the first steps of the development of a national plan for rare diseases as proposed by the subcommittee for rare diseases in November 2010. The founding of CCRD included a sustained funding of Orphanet as the national information system for rare diseases and the involvement of both, the Medical University of Vienna and the GÖG as partners in the Joint Action Orphanet Europe.

The main activities of the CCRD in 2011, besides setting up its organisational structures and processes, were as follows:

- Acting as the the main driving force in drawing the national plan of action until end of 2013;
- Awareness raising among professionals / experts / doctors / patients on the topic of rare diseases;
- Active participation in the EU-funded project Orphanet as well as other European initiatives in this area (e.g. the EU Working Group on Mechanisms for a coordinated access to orphan medicinal products as part of the platform for Access to Medicines in Europe which is part of the Corporate Social Responsibility Process launched by Commissioner Tajani);
- Information provision:
    - Establishment of Orphanet national scientific advisory board;
    - Establishment of national Orphanet website;
    - Updating of information on rare diseases in Austria ;
- Perform a large scale needs assessment survey regarding rare disease involving academics, stakeholders and – for the first time in Austria – patients and draft a report that is planned to be published in summer 2012;
- Drafting eligibility criteria for the establishment of Centres of Expertise based on EUCERD recommendations and discuss them with the Austrian stakeholders;
- Acting as the communication hub between actors in the field, focussing in the first years on health care professionals and other stakeholders and will contribute to ensuring that the unique challenges faced by people with rare diseases to meet are targeted;
- Acting as the focal point for European activities in the field of rare diseases, i.e. to keep track of developments and trying to draft a landscape of activities involving Austrian institutions.

**Centres of expertise**

Currently, there are no officially designated centres of expertise in Austria; informally, a 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 supports the concept of such centres and has asked the CCRD to work on this topic.

It is therefore expected that future centres of expertise will be identified and suitable ones 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 national legal requirements, taking into account pre-existing structures of the Austrian health care system as will be reflected in the national plan of action for rare diseases,
c) that either the necessary funds have been generated or, which is the preferred option, ways for a cost-neutral establishment of such CoE have been found, and
d) the national plan has been implemented.

The nomination of centres of expertise and related expert clinics constitutes one of the actions in the national plan for rare diseases, covered by the strategic priority “Improving health care pathways”.

**Pilot European Reference Networks**

Austrian teams participate, or have participated, in the following European Reference Networks for rare diseases: EUROHISTIONET, NEUROPED (main partner), European network of paediatric Hodgkin's lymphoma, and PAAIR.

**Registries**

Currently, no nationwide, general, comprehensive registry for rare disease patients exists in Austria. Approximately 10-15 registries for individual rare diseases or groups of rare diseases are run by specialised clinics or networks of experts from different clinics, e.g. a haemophilia registry or one for acromegalia. 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, EIMD, EMSA-SG, EUROCAT and ENRAH. Actions in this area are included in the National Plan for Rare Diseases (“National/Cross-border registry”).

**Neonatal screening policy**

Since the late 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 32 diseases and conditions: adrenogenital syndrome, biotinidase deficiency, carnitine-acylcarnitine translocase deficiency, carnitine palmitoyl transferase IA deficiency, carnitine palmitoyl transferase II deficiency, carnitine uptake deficiency, citrullinemia, argininosuccinic aciduria, congenital hypothyroidism, cystic fibrosis, galactosemia, glutaric acidemia type I, glutaric acidemia type II / multiple acyl-CoA dehydrogenase deficiency, homocystinuria and hypermethionemia, isobutyryl CoA dehydrogenase deficiency, isovaleric acidemia, β-ketothiolase deficiency, long-chain acyl-CoA dehydrogenase deficiency, mitochondrial trifunctional protein deficiency, maple syrup urine disease, medium-chain acyl-CoA dehydrogenase deficiency, methylmalonic aciduria, propionic acidemia, holocarboxylase synthetase deficiency, phenylketonuria and hyperphenylalaninemia, short-chain acyl-CoA dehydrogenase deficiency, tyrosinemia type I, very long-chain acyl-CoA dehydrogenase deficiency, 2-Methyl 3-hydroxybutyryl-CoA dehydrogenase deficiency, 3-Hydroxy-3-methylglutaric aciduria, 3-Methylcrotonyl-CoA carboxylase deficiency, and 3-Methylglutaconic aciduria type I. This screening panel remained unchanged in 2011.

Since 2009, and further expanded in 2010, a scientifically based NBS exists that covers six different lysosomal storage disorders (e.g. Mucopolysaccharidosis (MPS) type 1, Gaucher, Fabry, Pompe, and Nieman-Pick Type A/B). At end of 2011 it had not been decided whether (and when) any of these diseases should be included in the national program.

Detailed information regarding the Austrian NBS is provided by a completely revised homepage[2] that was made available online at the end of 2011 and that is available in three languages (German, English, and Turkish). As additional service, nearly all diseases listed and explained on the NBS homepage are directly linked to the relevant disease entity in the Orphanet database.

**Genetic Testing**

Molecular genetic testing in Austria is regulated by the so-called "Gentechnikgesetz" (GTG), first established in 1994[3] and last revised in 2005[4]. 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").

[2] http://www.meduniwien.ac.at/hp/neugeborenen-screening/
[3] http://www.bmg.gv.at/cms/home/attachments/7/8/8/CH1060/CMS1226929588865/510_1994.pdf
[4] 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 ("Bundesländer") 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 the hospital sector (as inpatient or outpatient) or (b) in the private sector (i.e. by a general practitioner or consultant of a specific medical discipline that has his own practice and a service contract with the relevant health insurance fund). 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 DRG basis. However, hospitals have to make efforts to not exceed the budgets allotted to them for each calendar year. In the second case (private practice), reimbursement is the responsibility of the sickness fund of the patient. In this instance, specific tariffs are calculated by the sickness fund for each type of service and services are reimbursed according to the tariff catalogue. Basically, mainly services that have been successfully negotiated with the sickness fund and integrated into their individual tariff catalogue are eligible for reimbursement. Still, patients/their doctors have always the possibility to apply for individual 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) sickness fund of the patient 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 in- and outpatients, the hospital covers the costs according to the tariffs of the laboratory performing the test;
(c) For patients in the private practice, the respective insurer carries the costs; however, it is possible that certain analyses (depending on the internal regulations of the sickness fund) require an ex-ante approval by the head physician ("Chefarzt") of thesickness 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 need to be authorised by a sickness fund"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 109 genes and an estimated 172 diseases in the Orphanet database[5].

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

3 December 2011 was a milestone for patients suffering from rare diseases in Austria as on this date the umbrella organisation for patient organisations dealing with rare diseases was founded. This national "Allianz für seltene Erkrankungen" is called "Pro Rare Austria" and was founded by Dr. Riedl who is also spokesperson of DEBRA Austria, the Epidermolysis bullosa patient support group. As first broadly recognised activity the new Pro Rare team organised the Rare Disease Day 2012 in Vienna but also participated in a number of rare disease events (e.g. at the Mariazell follow-up Congress on Rare Diseases on 3-4. December 2011 hosted by the Medical University of Vienna).

Pro Rare Austria demands:

- Official recognition of defined rare diseases
- Public acknowledgment of the special status of patients suffering from rare diseases, namely
    - Exemption from any co-payments or cost-sharing for any treatment related to their rare disease
    - Unrestricted access to medicinal investigations, tests and diagnosis in Austria, and if not available nationally, within the EEA
    - Unrestricted access to all available therapies and medicines especially orphan drugs in Austria, and if not available nationally, within the EEA

[5] Information extracted from the Orphanet database in September 2011.

- Austrian-wide uniform regulations with regards to long term care, care-support and childcare
- Improvement of medical care by establishment or designation of regional and/or national reference clinics/reference centres
- Promotion of scientific research aimed at developing therapies

Delegates of Pro Rare and a few other patient organisations were also invited to become member of the newly established Expert Committee for rare diseases (see Figure 1).

Apart from Pro Rare general alliances of patient organisations (both for rare and non-rare diseases) do exist on the province level (ARGE Selbsthilfe Carinthia, Upper Austria, Lower Austria, Salzburg, Styria, Tyrol, Vorarlberg, and Vienna). They are united under the supra-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. The Austrian Health Institute supports Pro Rare by providing meeting rooms and optional funding for further education in the field.

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".

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

***Orphanet activities in Austria***

With the establishment of the National Coordination Centre for Rare Diseases (CCRD) the dedicated Austrian team that was in charge for Orphanet for almost ten years also expanded to include GÖG staff members. Part of the staff is still 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 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 2011, the Orphanet-Austria[6] national website which provides an entry point to the Orphanet database was launched based on the self-developed country website which was launched back in 2008. The objectives are 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. The team reports about major events and activities organised either by Orphanet Austria itself or by other stakeholders.

***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 (Nationale Koordinationsstelle für Seltene Erkrankungen, NKSE) was established at the Austrian Health Institute Gesundheit Österreich[7] (as part – and first structural measure – of the national plan of action under development). NKSE is funded by the Austrian Ministry of Health (Bundesministerium für Gesundheit) and shall act in the mid-term as information provider next to its other functions. Orphanet Austria was integrated into this coordination centre to enable maximum synergy between the two structures. In the first phase the CCRD launched the Austrian orphanet website[8] and provided information to health professionals, e.g. by presenting on rare disease specific congresses and events.

***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.

***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

[6] http://www.orpha.net/national/AT-DE/index/startseite/
[7] http://www.goeg.at/de/Bereich/Koordinationsstelle-NKSE.html
[8] www.orpha.net/national/AT-DE/index/startseite

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)[9].

- A number of medical departments or patient registries also host websites with comprehensive and useful information on those rare diseases they are focusing on[10].
- In 2011 also rare disease specific information was provided on the official governmental health platform for Austria. Examples of information provided are excerpys regarding the patient, payer and stakeholder survey performed by the Austrian National Coordination Body for Rare Diseases (NKSE)[11] as well as information regarding the establishment of the NKSE[12].

**Good practice guidelines**

No specific information reported.

**Training and education initiatives**

The Academy of the Epidermolysis Bullosa House hosts training workshops for epidermolysis bullosa on a regular basis. In addition, the Department of Dermatology of the Paracelsus Medical University Salzburg organised the "Fostering Courses for Genodermatoses" under the auspices of the European Academy of Dermatology and Venerology in 2009, 2010 and 2012.

**National rare disease events in 2011**

A day dedicated to rare diseases was held on 26 February 2011 at the Paracelsus Medical University Salzburg to celebrate the 4th Rare Disease Day. Also on 26 February 2011, the "walk for rare diseases" took place in Vienna, again featuring more participants than in previous years.

On 2 and 3 December 2011, the Medical University of Vienna and the National Action Platform for Rare Diseases (NASE) held the Austrian Congress on Rare Diseases 2011 in Vienna. The conference was planned as a follow-up event from the 2010 congress in Mariazell and once again was attended by a large range of stakeholders to encourage discussion, to raise awareness and to look for solutions.

In addition to these events, the Second Regional Forum for Rare Diseases was held on 20-21 May 2011 in Innsbruck.

**Hosted rare disease events in 2011**

In addition to above mentioned events, a symposium pre-congress symposium to the International Congress on Prevention of Congenital Diseases, entitled Combating Rare Genetic Diseases - Clinics and Networking in Science, was held on 12 May 2011.

Rare diseases were also a topic at the 3rd International Congress for Quality Management in Villach that was held in the frame of the EU InterReg Project between Italy, Austria and Slovenia (Presentation: Management seltener Erkrankungen: Erfahrungen in Österreich - Entwicklungen in Österreich).

**Research activities and E-Rare partnership**

***National 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, harbouring the risk (in times of restricted research budgets) of 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, several 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

[9] Some examples (non-exhaustive) are http://www.mps-austria.at, http://www.klinefelter.at, http://www.rett-syndrom.at/index.html, http://www.debra-austria.org, http://www.pah-info.at

[10] Some (non-exhaustive) examples are: www.genodermatosen.at, http://www.akromegalie-register.at/wDeutsch/akromegalie/index.php?navanchor=1110006, https://www.studienregister.at/web/guest/home (select info folder for haemophilia).

[11] www.gesundheit.gv.at/Portal.Node/ghp/public/content/aktuelles/seltene-erkrankungen-befragung.html

[12] www.gesundheit.gv.at/Portal.Node/ghp/public/content/aktuelles/koordinationsstelle-seltene-erkrankungen.html

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").

***Participation in European research projects***

Austrian teams participate, or have participated, in around 50 European research projects or research networks for rare diseases – with a leading role in eight – including for instance: BNE, CLINIGENE, DIRECT, EDEN, EMSA-SG, EFACTS, EMINA, ENRAH, ENCE-PLAN, EURIPFNET, EUROTRAPS, EURO-IRON1, EURO-LAMINOPATHIES, EUROPEAN LEUKEMIA NET, EUROWILSON, GENEGRAFT, GENESKIN, GENOMIT, IMMOMEC, IntReALL, LEUKOTREAT, LYMPHANGIOGENOMICS, MYELINET, NEUTRONET, NEUROPRION, PERXISOMES, PNSEURONET, PROTHETS, PULMOTENSION, PWS, RARE-G, RHORCOD, RD-Connect[13], RD PLATFORM, WHIPPLE'S DISEASE, SARS/FLU-VACCINE and TUB-GENCODEV. Austria is part of the SIOPEN-R-NET research network and networks resp/registries such as ERCUSYN, EUROCAT, IDR, PRINTO, SCNIR and RARECARE.[14]

***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)[15] 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 participated in the 3rd Joint Transnational Call in 2010/11 and Austrian teams will participate in all together seven of the funded projects.

***IRDiRC***

Austrian funding agencies are not currently a committed members of IRDiRC.

**Orphan medicinal products**

***Orphan medicinal product committee***

There is currently no committee for orphan drugs in Austria.

***Orphan medicinal product incentives***

The Austrian Medicines Law (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)[16]".*

***Orphan medicinal product market availability situation***

As soon as marketing authorisation is provided, orphan drugs are available quite quickly in Austria. Actions 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 still improve availability.

In 2011, 51 of the 62 orphan drugs with existing EU marketing authorisation were available in Austria and were used at least once. For the remaining orphans no out-patient use was documented, but it is possible that they were used in-patient, i.e. during hospital stay. Only Plenadren is not marketed for sure in Austria. Regarding reimbursement status and the meaning of "boxes" please see the section below. It is important to note, however, that the label "no box" does not mean that patients have no access or that it is not publicly funded, it just means that it is not included in the Austrian out-patient positive list.

*Table 1. Availability of authorised orphan drugs in Austria in 2011/2012*

| *Active Ingredient* | *Brand name* | *Company* | *Outpatient reimbursement* |
|---|---|---|---|
| **Pegvisomant** | **Somavert** | **Pfizer** | **N** |
| **Clofarabin** | **Evoltra** | **Genzyme** | **N** |
| **Imatinib Mesilat** | **Glivec** | **Novartis Europharm** | **N** |
| **Mercaptopurin** | **Mercaptopurin Nova Labo** | **Nova Laboratories** | **n.a.** |

[13] Positive evaluation but contract not yet concluded.
[14] Based on information provided by the national EU database team PROVISIO.
[15] http://www.fwf.ac.at/
[16] *Inventory of Community and Member States' incentive measures to aid the research, marketing, development and availability of orphan medicinal products. Revision 2005* (p7).

| | | | |
|---|---|---|---|
| **Histamin Dihydrochlorid** | **Ceplene** | **EpiCept GmbH** | **N** |
| **Arsentrioxid** | **Trisenox** | **Cell Therapeutics (UK)** | **N** |
| **Tafamidis** | **Vyndaqel** | **Pfizer** | **N** |
| **Aztreonamlysin** | **Cayston** | **Gilead** | **N** |
| **Tobramycin** | **TOBI Podhaler** | **Novartis Europharm** | **Y** |
| **Ofatumumab** | **Arzerra** | **GlaxoSmithKline** | **N** |
| **Nilotinib** | **Tasigna** | **Novartis** | **N** |
| **Dasatinib** | **Sprycel** | **Bristol-Myers Squibb** | **N** |
| **Rilonacept** | **Rilonacept Regeneron** | **Regeneron** | **n.a.** |
| **Ibuprofen** | **Pedea** | **Orphan Europe** | **N** |
| **Trabectedin** | **Yondelis** | **Pharma Mar S.A.** | **N** |
| **Deferasirox** | **Exjade** | **Novartis Europharm** | **N** |
| **Dexrazoxan** | **Savene** | **SpePharm** | **N** |
| **Icatibant** | **Firazyr** | **Jerini AG** | **Y** |
| **Betain** | **Cystadane** | **Orphan Europe** | **N** |
| **Carglumsäure** | **Carbaglu** | **Orphan Europe** | **N** |
| **Pirfenidon** | **Esbriet** | **InterMune** | **R** |
| **Romiplostim** | **Nplate** | **Amgen** | **Y** |
| **Eltrombopag** | **Revolade** | **GlaxoSmithKline** | **Y** |
| **Busulfan** | **Busilvex** | **Pierre Fabre** | **N** |
| **Thiotepa** | **Tepadina** | **Adienne S.r.l.** | **N** |
| **Amifampridin** | **Firdapse** | **Eusa Pharma SAS** | **n.a.** |
| **Sorafenib** | **Nexavar** | **Bayer HealthCare** | **N** |
| **Rufinamid** | **Inovelon** | **Eisai** | **N** |
| **Bosentan** | **Tracleer (Ftbl)** | **Actelion Registration** | **Y** |
| **Iloprost** | **Ventavis** | **Schering AG** | **Y** |
| **Sildenafil** | **Revatio (Ftbl)** | **Pfizer** | **Y** |
| **Ambrisentan** | **Volibris** | **GlaxoSmithKline** | **Y** |
| **Nelarabin** | **Atriance** | **GlaxoSmithKline** | **N** |
| **5-Aminolaevulinsäure** | **Gliolan** | **Medac** | **n.a.** |
| **Plerixafor** | **Mozobil** | **Genzyme** | **N** |
| **Miglustat** | **Zavesca** | **Actelion Registration** | **Y** |
| **Velaglucerase alfa** | **VPRIV (1 ST)** | **Shire** | **Y** |
| **Alglucosidase alfa** | **Myozyme** | **Genzyme Europe** | **N** |
| **Zinkacetat Dihydrat** | **Wilzin** | **Orphan Europe** | **N** |
| **Laronidase** | **Aldurazyme** | **Genzyme Europe** | **N** |
| **Idursulfase** | **Elaprase** | **Shire** | **N** |
| **Galsulfase** | **Naglayzme** | **BioMarin** | **n.a.** |
| **Lenalidomid** | **Revlimid** | **Celgene Corporation** | **N** |
| **Thalidomid** | **Thalidomide** | **Celgene Corporation** | **N** |
| **Azacitidin** | **Vidaza** | **Celgene Corporation** | **N** |
| **Stiripentol** | **Diacomit** | **Laboratoires Biocodex** | **n.a.** |
| **Hydrocortison** | **Plenadren** | **Duocort Pharma** | **Not marketed** |
| **Mitotan** | **Lysodren** | **Laboratoire HRA** | **Y** |
| **Temsirolimus** | **Torisel** | **Wyeth** | **N** |

| | | | |
|---|---|---|---|
| **Cladribin** | **Litak** | **Lipomed** | **N** |
| **Mifamurtid** | **Mepact** | **IDM Pharma** | **N** |
| **Levodopa/Carbidopa** | **Duodopa Gel** | **Abbot Products GmbH** | **N** |
| **Eculizumab** | **Soliris** | **Alexion Europe** | **N** |
| **Sapropterin** | **Kuvan** | **Merck Serono** | **Y** |
| **Coffeincitrat** | **Peyona** | **Chiesi Farmaceutici** | **N** |
| **Mecasermin** | **Increlex** | **Tercica** | **Y** |
| **Ziconotid** | **Prialt** | **Eisai Limited** | **N** |
| **Hydroxycarbamid** | **Siklos** | **Addmedica** | **n.a.** |
| **Porfimer Natrium** | **Photobarr** | **Axcan Pharma** | **n.a.** |
| **Everolimus** | **Votubia** | **Novartis Europharm** | **N** |
| **Anagrelid-Hydrochlorid** | **Xagrid** | **Shire** | **n.a.** |
| **Nitisinon** | **Orfadin** | **Swedish Orphan** | **n.a.** |

*N = No box, R = Red box, Y = Yellow Box, n.a. = non information on use available*
*Source: Austrian PPI service 2012*

***Orphan medicinal product pricing policy***

In case a marketing authorisation holder applies for reimbursement by the Austrian Social Insurance in case of out-patient treatment, i.e. inclusion in the positive list "Erstattungskodex", the product falls under statutory price regulations. Thus its maximum ex-factory price may not exceed the EU-24 average price. The final decision on the reimbursement price is made by Association of Austrian Social Security Institutions after some negotiation with the company. The way of the application and the decision process is regulation by a specific regulation called VO-EKO in German.

The Austrian Social Insurance Fund reported to have received 14 submissions for orphan drugs between 2006 and 2011, whereby:

- 2 submissions included 1 clinical trial
- 7 submissions included 2 clinical trials
- 5 submissions included 3 clinical trials (maximum allowed)
- All but one submissions included at least 1 Randomised Clinical Trial
- In half of the submissions placebo was used as the only comparator, although therapeutic options were available

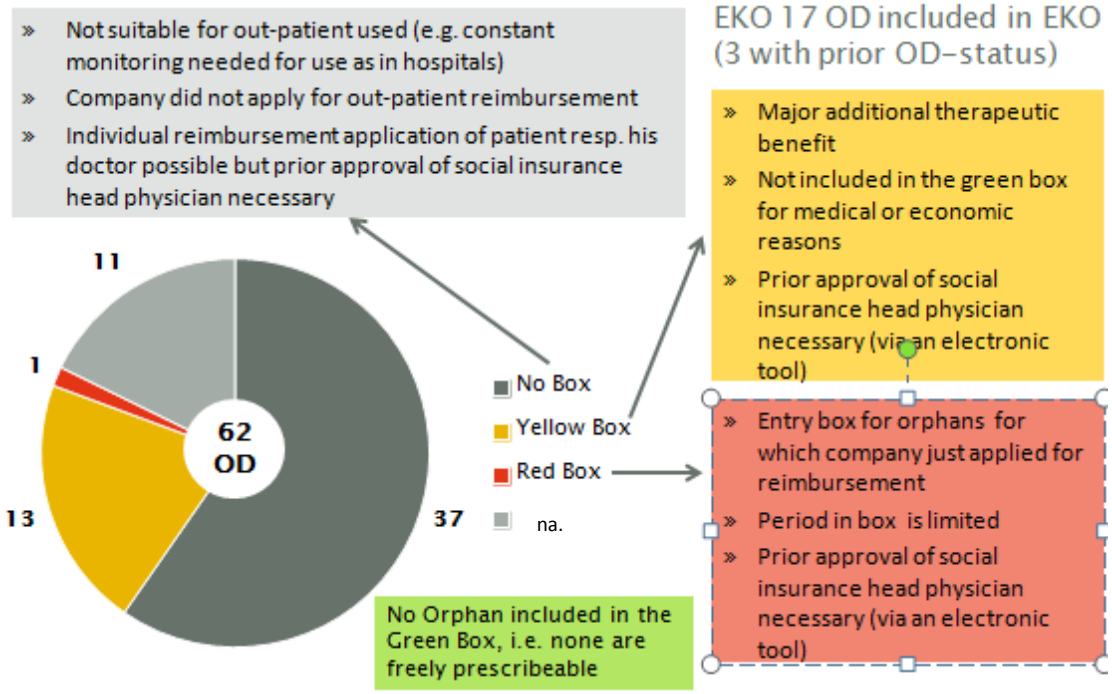
If the orphan drug is not reimbursed its price may be determined by the manufacturer alone. Unauthorised orphan drugs may be imported on case-by-case decisions. The vast majority of orphan drugs are dispensed in hospitals

***Orphan medicinal product 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[17]. Contract physicians are entitled to prescribe all medicines included in the Austrian Reimbursement Code (EKO)[18] - 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 electronic online tool. A reply is sent within 30 minutes latest. 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, see Figure 2 below.

[17] Art. 133 ASVG 1955, regulating the extent of medical treatment [Art. 133 ASVG 1995; BGBl. No. 189/1955]
[18] Art. 31.3(12) ASVG, on the publication of the Reimbursement Code EKO (Art. 31.3(12) )

*Figure 2: Out-patient reimbursement status of all authorised orphan medicines with an active orphan designation in Austria, 2011/2012*[19]




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 four 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 to the CCRD team 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 2011 public expenditure per prescription for orphan drugs amounted to around €2,700.- which is a small decrease compared to €2,771.- in 2010. Altogether the Austrian Social Insurance spent €85.3 million on orphan drugs in 2010, and €93.2 in 2011[20]. In 2010 3.3% of all pharmaceutical expenditure was caused by orphan drugs where as expressed by number of prescriptions the share was only 0.03%.

[19] Source: CCRD 2012 based on EKO 1/2012 and Warenverzeichnis 1/2012.
[20] See table 2.

*Table 2: Expenditure of Austrian Social Insurance for medicines and orphan drugs used out-patient, 2009 and 2010*[21]

| Indicator | 2009 | 2010 |
|---|---|---|
| **Expenditure** | | |
| Total PE by Social Insurance | 2.575.279.455 | 2.595.067.053 |
| Social Insurance Expenditure for Orphan Drugs* | 74.600.543 | 85.296.165 |
| OD share on Total PE | 2,9 % | 3,3 % |
| **Medicines Prescriptions** | | |
| Total number of prescriptions | 117.080.832 | 118.021.978 |
| No. Of OD-prescriptions * | 27.085 | 30.778 |
| OD share on total number of prescriptions | 0,02 % | 0,03 % |
| **Ø Cost per prescription** | | |
| All medicines | 22,00 | 21,99 |
| Orphan Drugs * | 2.754,30 | 2.771,30 |

*PE = Pharmaceutical Expenditure, OD = Orphan Drug*

***Other initiatives to improve access to orphan medicinal products***

Any kind of off-label use is not well accepted by public authorities in Austria.

**Orphan devices**

No specific information reported.

# DEVELOPMENT OF RARE DISEASE ACTIVITIES IN 2011 IN AUSTRIA

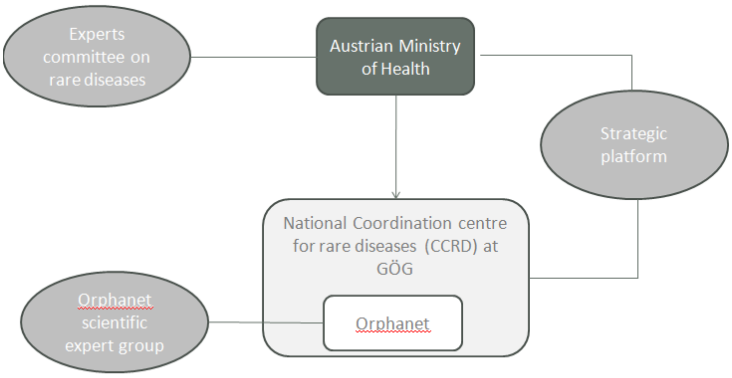
**National plan/strategy for rare diseases and related actions**

As response to a petition by health professionals and patient organisation for a national action plan on rare diseases in Feb 2008 and considering the recommendations of the European Council of 8 June 2009 to implement a national action plan on rare diseases until 2013 the highest Austrian Health Advisory board ("Oberster Sanitätsrat") of the Austrian Ministry of Health (BMG) established a subcommittee for rare diseases in May 2009, consisting of 17 members from 13 different organisations or institutions (covering the main stakeholders in the field). This working group was managed by the Austrian Orphanet team and laid the foundation for a national plan of action for rare diseases. It was the first time in Austria that an expert committee of this size, covering a broad spectrum of viewpoints, was working on rare diseases in 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.

However, due to the many topics on the agenda and the lack of resources of the Board Members the Austrian Ministry of Health decided to establish a National Coordination Centre for Rare Diseases (CCRD, Nationale Kontaktstelle für Seltene Erkrankungen, NKSE). The CCRD was established by 1 January 2011 at the Austrian Health Institute (Gesundheit Österreich GmbH, GÖG) and has a team of 1.4 full time equivalents and integrates part of the Austrian Orphanet team. Most members of the subcommittee for rare diseases are still involved in the topic as they kindly accepted their appointment to the Expert committee on rare diseases that was established in mid 2011 by the Ministry of Health (see Figure 1 below). In addition to the previous structure a strategic platform was set up, the is composed of Ministry of Health delegates, academic experts and payers (Austrian provinces and the Main association of the Austrian social security institutions),

[21] Note: All medicines expenses below the prescription fee (around € 5,-) are not included in these figures. Source: Austrian Social Insurance 2012 (Maschinelle Heilmittelabrechnung).

*Figure 1: Organisational Chart of the Austrian CCRD in 2011*




The establishment of the CCRD was one of the first steps of the development of a national plan for rare diseases as proposed by the subcommittee for rare diseases in November 2010. The founding of CCRD included a sustained funding of Orphanet as the national information system for rare diseases and the involvement of both, the Medical University of Vienna and the GÖG as partners in the Joint Action Orphanet Europe.

The main activities of the CCRD in 2011, besides setting up its organisational structures and processes were as follows:

- Acting as the the main driving force in drawing the national plan of action until end of 2013;
- Awareness raising among professionals / experts / doctors / patients on the topic of rare diseases;
- Active participation in the EU-funded project Orphanet as well as other European initiatives in this area (e.g. the EU Working Group on Mechanisms for a coordinated access to orphan medicinal products as part of the platform for Access to Medicines in Europe which is part of the Corporate Social Responsibility Process launched by Commissioner Tajani);
- Information provision:
    - Establishment of Orphanet national scientific advisory board;
    - Establishment of national Orphanet website;
    - Updating of information on rare diseases in Austria;
- Perform a large scale needs assessment survey regarding rare disease involving academics, stakeholders and – for the first time in Austria – patients and draft a report that is planned to be published in summer 2012;
- Drafting eligibility criteria for the establishment of Centres of Expertise based on EUCERD recommendations and discuss them with the Austrian stakeholders;
- Acting as the communication hub between actors in the field, focussing in the first years on health care professionals and other stakeholders and will contribute to ensuring that the unique challenges faced by people with rare diseases to meet are targeted;
- Acting as the focal point for European activities in the field of rare diseases, i.e. to keep track of developments and trying to draft a landscape of activities involving Austrian institutions.

**Neonatal screening policy**

Detailed information regarding the Austrian NBS is provided by a completely revised homepage[22] that was made available online at the end of 2011 and that is available in three languages (German, English, and Turkish). As additional service, nearly all diseases listed and explained on the NBS homepage are directly linked to the relevant disease entity in the Orphanet database.

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

3 December 2011 was a milestone for patients suffering from rare diseases in Austria as on this date the umbrella organisation for patient organisations dealing with rare diseases was founded. This national "Allianz für seltene Erkrankungen" is called "Pro Rare Austria" and was founded by Dr. Riedl who is also spokesperson

[22] http://www.meduniwien.ac.at/hp/neugeborenen-screening/

of DEBRA Austria, the Epidermolysis bullosa patient support group. As first broadly recognised activity the new Pro Rare team organised the Rare Disease Day 2012 in Vienna but also participated in a number of rare disease events (e.g. at the Mariazell follow-up Congress on Rare Diseases on 3-4. December 2011 hosted by the Medical University of Vienna).

Pro Rare Austria demands:

- Official recognition of defined rare diseases
- Public acknowledgment of the special status of patients suffering from rare diseases, namely
  - Exemption from any co-payments or cost-sharing for any treatment related to their rare disease
  - Unrestricted access to medicinal investigations, tests and diagnosis in Austria, and if not available nationally, within the EEA
  - Unrestricted access to all available therapies and medicines especially orphan drugs in Austria, and if not available nationally, within the EEA
  - Austrian-wide uniform regulations with regards to long term care, care-support and childcare
- Improvement of medical care by establishment or designation of regional and/or national reference clinics/reference centres
- Promotion of scientific research aimed at developing therapies

Delegates of Pro Rare and a few other patient organisations were also invited to become member of the newly established Expert Committee for rare diseases (see Graph 1).

Apart from Pro Rare general alliances of patient organisations (both for rare and non-rare diseases) do exist on the province level (ARGE Selbsthilfe Carinthia, Upper Austria, Lower Austria, Salzburg, Styria, Tyrol, Vorarlberg, and Vienna). They are united under the supra-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. The Austrian Health Institute supports Pro Rare by providing meeting rooms and optional funding for further education in the field.

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".

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

***Orphanet activities in Austria***

In 2011, the Orphanet-Austria[23] national website providing a national entry point to the Orphanet database was launched based on the self-developed country website which was launched back in 2008. The objectives are 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. The team reports about major events and activities organised either by Orphanet Austria itself or by other stakeholders.

***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 (Nationale Koordinationsstelle für Seltene Erkrankungen, NKSE) was established at the Austrian Health Institute Gesundheit Österreich[24] (as part – and first structural measure – of the national plan of action under development). NKSE is funded by the Austrian Ministry of Health (Bundesministerium für Gesundheit) and shall act in the mid-term as information provider next to its other functions. Orphanet Austria was integrated into this coordination centre to enable maximum synergy between the two structures. In the first phase the CCRD launched the Austrian Orphanet Website[25] and provided information to health professionals, e.g. by presenting on rare disease specific congresses and events.

***Other sources of information***

In 2011 rare disease specific information was also provided on the official governmental health platform for Austria. Examples of information provided are excerpts regarding the patient, payer and stakeholder survey

[23] http://www.orpha.net/national/AT-DE/index/startseite/
[24] http://www.goeg.at/de/Bereich/Koordinationsstelle-NKSE.html
[25] www.orpha.net/national/AT-DE/index/startseite

performed by the Austrian National Coordination Body for Rare Diseases (NKSE)[26] as well as information regarding the establishment of the NKSE[27].

**National rare disease events in 2011**

A day dedicated to rare diseases was held on 26 February 2011 at the Paracelsus Medical University Salzburg to celebrate the 4th Rare Disease Day. Also on 26 February 2011, the "walk for rare diseases" took place in Vienna, again featuring more participants than in previous years.

On 2 and 3 December 2011, the Medical University of Vienna and the National Action Platform for Rare Diseases (NASE) held the Austrian Congress on Rare Diseases 2011 in Vienna. The conference was planned as a follow-up event from the 2010 congress in Mariazell and once again was attended by a large range of stakeholders to encourage discussion, to raise awareness and to look for solutions.

In addition to these events, the Second Regional Forum for Rare Diseases was held on 20-21 May 2011 in Innsbruck.

**Research activities and E-Rare partnership**

***E-Rare***

Austria participated in the 3rd Joint Transnational Call in 2010/11 and Austrian teams will participate in all together seven of the funded projects.

***IRDiRC***

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

[26] www.gesundheit.gv.at/Portal.Node/ghp/public/content/aktuelles/seltene-erkrankungen-befragung.html
[27] www.gesundheit.gv.at/Portal.Node/ghp/public/content/aktuelles/koordinationsstelle-seltene-erkrankungen.html

# LIST OF CONTRIBUTIONS[28]

Contributions in 2010
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Brigitte Blöchl *(Medical University Vienna)*
Claudia Habl/Christine Leopold *(Gesundheit Österreich GmbH/ Austrian Health Institute)*

Contributions in 2012
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Main Association of Austrian Social Security Institutions *(HVB)*
Austrian Ministry of Health (BMG), Ministry of Science and Research *(BMWF)*
Patient Organisation for Rare Diseases *(Pro Rare)*
Austrian Pharmaceutical Association *(Pharmig)*
DEBRA Austria
Medical University Vienna *(MUW)*
Gesundheit Österreich GmbH *(GÖG)*
Pharmaceutical Pricing and Reimbursement Information Network *(PPRI)*

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  http://www.meduniwien.ac.at/hp/neugeborenen-screening/


[28] 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.
[29] All websites and documents were last accessed in May 2012. A more detailed list of sources is available in the full report: http://www.eucerd.eu/upload/file/Reports/2012ReportStateofArtRDActivities.pdf