EUROPLAN
European Project for Rare Diseases National Plans Development
Coordinated by the Italian National Centre for Rare Diseases
Italian National Institute of Health
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RaDiOrg.be
Rare Diseases Organisation

EURORDIS
Rare Diseases Europe

BELGIUM

EUROPLAN NATIONAL CONFERENCE

FINAL REPORT

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EUCERD
Joint Action

FOREWORD

The EUROPLAN National conferences are aimed at fostering the development of a comprehensive National Plan or Strategy for Rare Diseases addressing the unmet needs of patients living with a rare disease in Europe.

These national plans and strategies are intended to implement concrete national measures in key areas from research to codification of rare diseases, diagnosis, care and treatments as well as adapted social services for rare disease patients while integrating EU policies.

The EUROPLAN National conferences are jointly organised in each country by a National Alliance of rare disease patients' organisations and EURORDIS – the European Organisation for Rare Diseases. For this purpose, EURORDIS nominated 10 EURORDIS-EUROPLAN Advisors - all being from a National Alliance - specifically in charge of advising two to three National Alliances.

EUROPLAN National conferences share the same philosophy, objectives, format and content guidelines. They involve all stakeholders relevant for developing a plan/strategy for rare diseases. According to the national situation of each country and its most pressing needs, the content can be adjusted.

During the period 2008-2011, a first set of 15 EUROPLAN National Conferences were organised within the European project EUROPLAN. Following the success of these conferences, a second round of up to 24 EUROPLAN National Conferences is taking place in the broader context of the Joint Action of the European Committee of Experts on Rare Diseases (EUCERD) over the period March 2012 until August 2015.

The EUROPLAN National Conferences present the European rare disease policies as well as the EUCERD Recommendations adopted between 2010 and 2013. They are organised around common themes based on the Recommendation of the Council of the European Union on an action in the field of rare diseases:

1. Methodology and Governance of a National Plan;

2. Definition, codification and inventorying of RD; Information and Training;

3. Research on RD;

4. Care - Centres of Expertise / European Reference Networks/Cross Border Health Care;

5. Orphan Drugs;

6. Social Services for RD.

The themes "Patient Empowerment", "Gathering expertise at the European level" and "Sustainability" are transversal along the conference.

I. General information

Country	Belgium
Date & place of the National Conference	28 February 2014, Brussels
Website	www.Radiorg.be
Organisers	Radiorg
Members of the Steering Committee	• Jean-Jacques Cassiman, Fund Rare diseases and Orphan Drugs • Pol Gerits, Healthcare federal public administration • Ingrid Jageneau, RaDiOrg • André Lhoir, Federal Agency for drugs • Claude Sterckx, Fund Rare diseases and Orphan Drugs • Elfriede Swinnen, Federal Institute for health • Erik Tambuyzer, Fund Rare diseases and Orphan Drugs • Saskia Van den Bogaert, Healthcare federal public administration • Jonathan Ventura, RaDiOrg • Lene Jensen, EUROPLAN Advisor
Names and list of Workshops	• **Workshop 1:** Care, Centres of Expertise and European Reference Networks for Rare Diseases • **Workshop 2** : Definition, Codification and Inventorying of Rare Diseases • **Workshop 3**: Scientific Research on Rare Diseases • **Workshop 4**: Methodology, Governance and Monitoring of the National Plan • **Workshop 5**: Orphan Medicinal Products • **Workshop 6**: Social Services for Rare Diseases
Workshop Chairs (and Rapporteurs, where applicable)	• Bruce Poppe Gent, chair group 1 • Marc Abramovicz, rapporteur group 1 • Herman Van Oyen, Chair Group 2 • Montse Paz-Urbina, rapporteur group 2 • René Westhovens, chair group 3 • Freia Van Hee, rapporteur group 3 • Ingrid Jageneau, co-chair group 4

	• Ilse, Weeghmans, co-chair 4 • Lode De Bot, co-rapporteur group 4 • Jonathan Ventura, co-rapporteur group 4 • André Lhoir, Chair group 5 • Marc Dooms, rapporteur group 5 • Chris Van Hul, Chair group 6 • Claude Sterckx, rapporteur group 6
Annexes	1. Programme in English 2. List of participants

II. Main Report

Plenary Report – Opening Session

National EUROPLAN Conference in Belgium

Rare Disease Day - 28 February 2014 – Brussels, Diamant Conference Centre

The Belgian EUROPLAN-conference was held on Rare Disease Day 2014. EUROPLAN aims to promote and implement the development of national plans for rare diseases in the EU member states.

The goal of the Belgian conference was to examine to which extent the strategic approach of rare diseases in Belgium has already taken shape and which additional measures are necessary to give patients with a rare disease the place in society that they rightly deserve by means of an adjusted healthcare and social care. Furthermore, the question was raised to which extent scientific and clinical research on rare diseases can be promoted and which steps need to be taken with regard to orphan medicinal products, codification, inventorying and registration of rare diseases.

The conference also gave us the opportunity to go over the Belgian Plan for Rare Diseases **for the first time** with a group of multi-stakeholders: health care providers, researchers, patients and carers. The Belgian Plan for Rare Diseases was presented in January 2014 by Ms Laurette Onkelinx, Deputy Prime Minister and Minister of Social Affairs and Public Health. This and other factors drew a huge crowd to the Rare Disease Day/ EUROPLAN conference: 149 people registered, including 45 patients, carers and representatives of patients' organisations.

Explanation of the Belgian Plan for Rare Diseases

During the conference the Belgian Plan for Rare Diseases was explained by Mr Claudio Colantoni, assistant at the cabinet of Ms Laurette Onkelinx, Deputy Prime Minister and Minister of Social Affairs and Public Health. The Belgian Plan for Rare Diseases has a long background: in February 2009 the Belgian Chamber of Representatives unanimously adopted a

resolution to develop an action plan for rare diseases and orphan medicinal products. At the end of 2011, at the request of the Minister of Social Affairs and Public Health, the Fund for Rare Diseases and Orphan Drugs (managed by the King Baudouin Foundation) worked out 42 recommendations and proposed measures for a future Belgian action plan for rare diseases. These 42 recommendations and proposed measures were spread over 11 action domains. The proposed measures were the result of extensive discussions in thematically organised working groups, in which 75 experts from very divergent disciplines and bodies took part as well as representatives of physicians and patients.

The next step in the process was the identification of concrete measures and actions. To this end, a steering group for rare diseases was composed of members of the Belgian National Institute for Health and Disability Insurance (RIZIV/INAMI), the Federal Public Service for Public Health, the Scientific Institute of Public Health (WIV-ISP) and the cabinet of the Minister of Social Affairs and Public Health. On the basis of an analysis of the state of affairs and the identification of priority measures, and bearing in mind the available budget, a multi-annual plan was drawn up. This multi-annual plan, based on the proposed measures of the Fund for Rare Diseases and Orphan Drugs, also takes into account the 59 process and outcome indicators, formulated in 2011 by EUROPLAN (European Project for Rare Diseases National Plans Development) and recently endorsed by EUCERD (European Union Committee of Experts on Rare Diseases) in the form of 21 core indicators.

The Belgian Plan for Rare Diseases focusses on four core areas: 1) improved access to appropriate diagnostic tests and information to the patient, 2) optimisation of the care, 3) information management and 4) governance and sustainability. The Plan consists of 20 actions.

At the moment, € 4.7 million is spent on specific measures every year. Once it is at cross speed, an annual budget of € 15.7 is planned. A short overview of the measures in the Plan can be seen below.

Furthermore, patients with a rare disease can also benefit from other initiatives that were taken within the restructuring and the expansion of the healthcare and social services, such as the Cancer Plan, the Plan for Chronic Diseases, programmes on eHealth etc.

The Belgian Plan for Rare Diseases in a nutshell

The measures that were included in the Belgian Plan for Rare Diseases can be summarised as follows:

Domain 1: Improved access to appropriate diagnostic tests and information to the patient

1. Improved funding of tests for the diagnosis and follow-up of rare diseases, carried out in Belgium or abroad – budget of € 1,050,000

2. Quality management in the centres for hereditary metabolic diseases – € 1,200,000

3. Genetic counselling in the existing Centres of Expertise for rare diseases – additional budget of € 100,000 (a budget of € 4,380,000 for genetic counselling was already granted)

4. Multidisciplinary consultation – € 1,300,000

5. Patient-oriented communication – included in action 7

6. Europlan – € 25,000

Domain 2: Optimisation of the care

7. Concentration of the expertise and improvement of the Centres of Expertise: introduction of a care coordinator – € 750,000

8. Centres of expertise for haemophilia – € 1,350,000

9. Function of rare diseases – € 500,000

10. Networks – € 500,000

11. New Centres of Expertise – to be determined later on, on the basis of the available budget

12. Medical food for rare diseases – € 537,532

13. Quick communication between patients, primary health care providers and specialists of the Centres of Expertise: use of the multidisciplinary electronic patient records – budget in action plan eHealth

14. Unmet medical need: quick availability of pharmaceutical specialties – € 3,500,000

15. Inventory of the 'needs that are not covered' – € 0

Domain 3: Information management

16. Central Registry of Rare Diseases – € 317,000

17. Orphanet Belgium – € 75,000

18. Training of health care providers – included in action 7

19. Codification and terminology– budget in action plan eHealth

Domain 4: Governance and sustainability

20. Assessment and monitoring of the Plan – € 200,000

Extended version of the Belgian Plan for Rare Diseases in Dutch: http://www.laurette-onkelinx.be/articles_docs/Belgisch_Plan_voor_zeldzame_ziekten.pdf

Extended version of the Belgian Plan for Rare Diseases in French: http://www.laurette-onkelinx.be/articles_docs/Plan_Belge_pour_les_maladies_rares.pdf

Explanation of the European Union Committee of Experts on Rare Diseases – EUCERD

Pol Gerits of the Federal Public Service for Public Health talked about the activities of EUCERD, which was founded in 2009. The task of this committee of experts was to support the European Commission in preparing and carrying out activities with regard to rare diseases by issuing guidelines and recommendations; mapping out, monitoring and evaluating the activities at European and member state level; stimulating the exchange of information on public health and research; and developing international collaborations. EUCERD had 56 members and was managed by a bureau consisting of 4 members. As from 2014, at the end of the mandate, EUCERD was replaced by the European Commission Expert Group on Rare Diseases, whose mission is similar to the one of the EUCERD.

EUCERD issued 5 sets of recommendations, 1 opinion, a newsletter and an annual overview of the activities. The recommendations related to Centres of Expertise for rare diseases; European reference networks; the clinical added value of orphan medicinal products (CAVOMP); registries and data collection; and core indicators for national strategies. The opinion dealt with the screening of newborns. (Please visit the website EUCERD for more details).

Furthermore, there is also the EUCERD Joint Action (from March 2012 until February 2015). The goals of this Joint Action are to implement and monitor the national plans and strategies (via EUROPLAN and National Conferences), to standardise the rare disease nomenclature (via Orphanet codes) and to map the provision of specialised social services for patients with a rare disease (therapeutic recreation, respite care services, adapted housing etc.).

Finally, Mr Gerits pointed out that in the meantime, 16 member states have worked out a national plan or a national strategy, but that only a few member states have defined a budget for that plan. Belgium is one of those member states and is therefore one of the best students in the European class, dixit Pol Gerits.

Explanation of the European policy on rare diseases

Lene Jensen, EUROPLAN advisor and Chief Executive Officer of Rare Diseases Denmark, emphasised the importance that Europe attaches to rare diseases. As important milestones she mentioned the European regulation on orphan medicinal products (December 1999), the Communication of the European Commission of 11 November 2008 on the challenge of rare

diseases, the Recommendation of the European Council of Health ministers of June 2009 in which all the member states are invited to establish a national plan or strategy before the end of 2013, the EUROPLAN/EUCERD indicators for national initiatives (January 2010 (Madrid) and June 2013 (Luxemburg)) and the European directive on Cross-Border Healthcare (2011, to be implemented before October 2013).

It is important that national plans and strategies are based on a comprehensive approach of patients with a rare disease. Thereby, we need to build on the existing national systems for healthcare and social care as much as possible. Furthermore, national plans need to include priority actions, with clear goals and identified mechanisms for follow-up. To this end, the guidelines and the recommendations that were formulated by EUROPLAN are preferably made use of.

According to the above-mentioned Recommendation of the European Council, the National Plans for Rare Diseases need to include the following themes: research on rare diseases, medical and non-medical care for people with a rare disease (via Centres of Expertise and European reference networks), stimulating the access to and development of Orphan Medicinal Products, facilitating social services for patients with rare diseases, adequate mechanisms for the definition, codification and inventory of rare diseases, devising a system for governance and monitoring of the measures that were taken.
As transversal themes is mentioned taking action to ensure the empowerment of patients and their organisations and guaranteeing that these actions include appropriate provisions to safeguard their sustainability over time.

Within this framework, EUROPLAN offers support during the development and implementation of national plans, amongst others via an interactive network of stakeholders but also by organising 24 national conferences, this Belgian conference being one of them. In this respect EUROPLAN should be more considered as a process rather than a project. Every national conference has the same schedule: during the opening session the goals of EUROPLAN and the EU policy with regard to rare diseases are emphasised once more. Then, there are six parallel thematically organised workshops, whereby the evolution on a national level is reviewed and compared to the guidelines and indicators of EUROPLAN. During the closing session, the final conclusions of the workshops are discussed once more in a plenary session and documented by means of an official report.

Report of Workshops

Theme 1 - Methodology, Governance and Monitoring of the National Plan

Actions in the Belgian Plan that relate to this theme: action 6, 20

The intended advantages of the Belgian Plan for Rare Diseases will only be realised if the measures are implemented effectively and efficiently. To that end, a policy model is necessary (governance) that disposes of a sufficient amount of policy-supporting resources, which can

measure on the basis of indicators whether the intended goals were achieved and which – if necessary – can benefit from efficient fine-tuning measures.

Ingrid Jageneau, president of RaDiOrg, recapitulated the process that preceded the definitive Plan for Rare Diseases. She stressed the importance of the contribution of patients' organisations during the activities of the Fund for Rare Diseases and Orphan Drugs. This Fund formulated a series of 42 recommendations, which served as the basis for the final Plan.

Ms Jageneau also talked about the Belgian situation, evaluated by RaDiOrg on the basis of the EUROPLAN indicators. Certain findings were highly remarkable. By publishing its Plan and because of the various legislative initiatives that have been implemented, Belgium complies with the first core indicator, i.e. that every EU member state needs to develop a Plan or Strategy aimed at guiding and structuring relevant actions in the field of rare diseases within the framework of their health and social systems. Core indicators 2 and 3, i.e. the existence of an advisory committee on rare diseases and permanent and official patients' representation in plan development, monitoring and assessment, have not been completely complied with yet. RaDiOrg is also still questioning core indicators 18 and 19, i.e. the existence of a policy/decision to ensure long-term sustainability of the Plan and the amount of public funds allocated to the Plan. Although a dedicated budget exists, the Plan itself is not in force just yet. Furthermore, no funds were reserved for the creation of new Centres of Expertise.

Lene Jensen of Rare Diseases Denmark and representative of EUCERD specified the use of indicators formulated by EUROPLAN and EUCERD. These indicators are process and outcome indicators, rather than health indicators for rare diseases. Concretely, EUROPLAN proposed 22 outcome indicators and 37 process indicators for the national plans in 2010. In 2013, these indicators were partly taken over and completed by EUCERD and reorganised into 21 basic indicators (5 outcome and 16 process). Thereafter, Lene Jensen provided the participants with some background for each of these 21 indicators. She concluded by asking whether additional indicators are necessary for Belgium and whether other forms of monitoring, next to the monitoring by an advisory committee and a coordinating steering group, are desired. She especially wondered to what extent patients participate in the monitoring process.

The latter question was immediately answered by Ilse Weeghmans of the Vlaams Patiëntenplatform (Flemish Patient Platform – VPP). Next to making a strong plea for the importance of patients' organisations and patient participation in the healthcare sector at micro, meso and macro level, Ilse Weeghmans also reviewed the role that patients played in the creation and the further development of the Belgian Plan for Rare Diseases. She especially saw an involvement in actions 5 (call centre), 13 (eHealth), 15 (inventorying of the needs that are not covered) and 20 (governance, as possible partner in the coordination cell and as consultation partner with the Federal Public Service). Also in action 6, the organisation of a national EUROPLAN conference, the patients' organisations are in charge, but what is striking is the absence during the workshops of the authorities that drew up the Plan. Many participants are disappointed because questions about "the how" and "the why" of a series of

specific measures remain unanswered.

Unfortunately, there are a number of actions where the involvement of patients is suppressed, while patients and their representing organisations could offer a clear added value. Examples are action 7 (the function of the care coordinator in the Centres of Expertise), action 8 (creation of Centres of Expertise for haemophilia), action 9 (the expansion of the function 'rare diseases' in official hospitals), action 10 (creation of networks of expertise) and action 11 (the foundation of new Centres of Expertise). In all of these actions, patients and their associations could provide important input on the needs and expectations with regard to the care and information to be received. Furthermore, patients' organisations could play an important role in the assessment of care and quality improvement.

After a thorough discussion the participants provided the working group with the following recommendations:

1. Indicators – In the execution of the Belgian Plan for Rare Diseases the proposed measures need to be accompanied by process and outcome indicators as recommended by EUCERD. Only in this way a concrete and objective monitoring of the Belgian Plan for Rare Diseases is possible.
2. Participation of all the stakeholders – The steering group, as described in measure 20 of the governance model, has to communicate with all the stakeholders involved and has to take into account their perspectives, expertise and identity, but at the same time their limitations and restrictions (e.g. it cannot be expected from a patients' organisation working with unpaid volunteers that it participates in tens of meetings, scientifically maps the needs of its patients etc. This requires the necessary professional support).
3. Building on existing structures – The governance model, as described in measure 20, needs to be based on and take into account existing groups, representations and bodies as much as possible. The steering group needs to keep close contact with the Observatory for Chronic Diseases, the Fund for Rare Diseases and Orphan Drugs etc. However, the significance and scope of each of these organisations need to be specified again and the interaction between the organisations has to be structured by means of clear agreements.
4. Feedback – The governance model, as described in measure 20, needs to include a permanent feedback between the authorities and the stakeholders involved, in all the phases: planning, identification of new measures, preparatory activities for execution etc. The participants in the workshop had the feeling that, in the time span between the proposed measures and recommendations by the Fund for Rare Diseases and Orphan Drugs (King Baudouin Foundation) and the publication of the current Belgian Plan for Rare Diseases, the stakeholders and the patients (organisations) were practically not consulted. Many consider this a missed opportunity. No feedback was given about the reason why certain measures from the publication of the Fund were

not included in the Plan. Furthermore, a number of additional measures were incorporated in the Plan, although they did not explicitly appear in the proposals of the Fund. The rationale for this is not really transparent and was not sufficiently explained during the EUROPLAN conference.

Theme 2 - Definition, codification and inventorying of RD

Actions in the Belgian Plan that relate to this theme: action 16, 17, 18, 19

Patients with a rare disease have to become visible in the Belgian healthcare system by means of a suitable codification and the creation of a central registry of rare diseases. After all, the care policy for rare diseases is heavily obstructed by a lack of epidemiological and longitudinal data about patients and the absence of an overview of the care structures involved.

Prof. Bettina Blaumeiser of the Centre for Medical Genetics of the University of Antwerp discussed the problematic nature of applying the European definition of rare diseases too strictly (5 people in 10,000), due to which some patients are in the 'twilight' zone and run the risk of being excluded. Prof. Blaumeiser put her finger on the sore spot by showing that there is not much certainty about the correctness of the existing prevalence and incidence data. Furthermore, the differences between the regions are significant and have not been mapped out properly.

Dr. Ingrid Mertens of the Federal Public Service for Public Health gave a rough sketch of how the department for data management wants to promote the use of ORPHA codes (developed by Orphanet) in addition to the ICD10 and SNOMED classification. At the moment the Belgian Centres of Expertise use different codification systems or no codification systems at all. For the incorporation of the ORPHA codes in the central registry, the department for data management cooperates with the WIV-ISP (Orphanet Belgium and the Central Registry of Rare Diseases), the European Orphanet coordinator (Inserm, France), the WHO (for ICD) and IHTSDO (for SNOMED).

Prof. Viviane Van Casteren of the Scientific Institute of Public Health (WIV-ISP) talked in detail about the initiatives that are in the pipeline in order to collect data about patients with a rare disease. In Belgium there are various patient registries and other databases related to a rare disorder or a group of rare diseases. On a Belgian level the WIV-ISP has plenty of experience with disease-specific registries (e.g. mucoviscidosis and neuromuscular diseases). The WIV-ISP is also responsible for the development of the Central Registry of Rare Diseases. At the moment a trial run of the Central Registry is done in two genetic centres and later on this year other genetic centres will follow. This Central Registry however does not contain any data of genetic tests yet. This will be worked out in the future as part of the Plan for Rare Diseases.

Finally, Dr. Séverine Henrard of the Institute of Health and Society (IRSS-UCL) presented her study on the willingness of patients to participate in clinical trials, registries and scientific research.

During the discussions a lot of attention was paid to privacy aspects and the possibility/challenge of coupling Belgian registries to European patient registries. The discussion yielded the following conclusions and recommendations:

1. Definition of rare diseases – At first sight, the European (EC) definition seems to be uncomplicated with its prevalence of 1/2000, as it guarantees a focus on rare diseases. Nevertheless, there is quite some uncertainty as to the incidence and prevalence figures, which are different according to the region and also change over time (e.g. as a consequence of prenatal screening or because patients live longer). As this can change the status of diseases, a review and if necessary an adjustment needs to take place on a regular basis. At the moment Orphanet is the most reliable source for incidence and prevalence data, even though regional differences are rarely shown. In the future a registration by means of a central registry will be a minimum requirement to dispose of correct data.
2. Registration obligation – In order to optimise the data collection it will be important that the Centres of Expertise for rare diseases are obliged to register their patients in the Central Registry of Rare Diseases.
3. Information to patients – Within the care system the possibility of informing patients' needs to be created (e.g. with regard to registration and participation in studies). To this end, a specific budget should be allocated.
4. Privacy – it is important that an opt-out procedure is possible for patients who do not want to be registered. However, practice (on the basis of the current experience of the WIV-ISP) has shown that very few patients make use of this possibility.
5. ORPHA codes – The traditional disease codes are not refined enough to be used for rare diseases. Although the ORPHA codes are sufficiently specific, they are not integrated in the current software. The participants in the workshop think that the use of the ORPHA codes by the centres for rare diseases should be obliged. In a later phase these codes should be included in SNOMED, a system that does not compel physicians to enter the codes themselves but automatically generates the right code on the basis of certain keywords.
6. Completeness of the registration – The centres for rare diseases have to cover the entire area, otherwise the registration remains incomplete. If we look at the current registry of neuromuscular diseases for example, there are – relatively speaking – many more patients that are registered in the north of Belgium than in the south. This is probably due to the difference in the degree of penetration of the neuromuscular centres.

Theme 3 - Research on RD

Actions in the Belgian Plan that relate to this theme: none

Prof. René Westhovens, rheumatologist at the University Hospital of Leuven, talked about the needs, the priorities, but also the possibilities of fundamental, clinical and translational

research on rare diseases on a Belgian and European level. He based his plea on the case of systemic sclerosis. As clinician and researcher, but just as well as president of the RIZIV/INAMI college for Orphan Medicinal Products, he stated that the efficacy and the safety of orphan medicinal products should be constantly evaluated, even after it has been introduced on the market. Questions about dosage, therapy loyalty, outcomes, expansion to sub-indications and quality of care have to be raised continuously. Furthermore, he also mentioned that ethical and social bottlenecks with regard to rare diseases and Orphan Medicinal Products are not researched sufficiently and that the transparency of the cost price of the treatment should be high on the agenda.

Prof. Eric Legius of the Department for Human Genetics of the University of Leuven talked about how the fundamental and translational research can be bridged. According to Prof. Legius we need to pay more attention within education and training to the combination of basic biology and clinical medicine. The interaction between clinical activities and research activities is important: research leads to better clinical performances whereas clinical input leads to better research. The best way to bridge research and clinic is to set up integrated departments in which both clinical activities as well as basic research take place. Furthermore, being integrated in multidisciplinary networks at a local, national and international level is a must. The bottleneck is still the financial vulnerability of most research groups on rare diseases, due to their size.

Dr. Arnaud Goolaerts of the National Fund for Scientific Research (FNRS/NFWO) and Irene Norstedt of the European Commission– DG Research & Innovation - entered at length into the Belgian and European mechanisms for the financing of research and zoomed in on the financing of the research on rare diseases.

The discussion yielded the following recommendations:

1. Incorporation of research in the Plan – The participants were astonished to find that there was not a single research component to be found in the current version of the Belgian Plan for Rare Diseases, even though it seems to speak for itself that research is the foundation for better diagnosis and treatment of patients with a rare disease in the future. The participants in the workshop consider this a serious shortcoming in the Plan.
2. Systematic framework – We need a framework for a better cooperation with patients and patients' organisations with regard to participation in clinical studies on the one hand and the identification of priorities in research on the other. The registries (the Central Registry of Rare Diseases and the Genetic Registry) might already be a first important instrument in order to enable participation in clinical studies in a more systematic way and to determine research priorities.
3. Fragmentation – The cooperation between the Belgian research group within and across the language borders dealing with fundamental and clinical research on rare diseases has to be improved. We need to break through the fragmentation that is still

far too frequent. Developing a collaborative framework would already be a step in the right direction. At the same time one should also look across the borders and develop platforms for European and international research on rare diseases, as various disorders are so rare that it is definitely useful to coordinate the research on a European level. It goes without saying that the mechanisms for financing need to be defragmented as well.

4. Funds – The financial support of Belgian translational research projects should be improved (e.g. via the E-RARE research programme). Belgium has various research groups dealing with rare diseases that do very well at a European and international level. Still, the financing that they get is inadequate, which limits further expansion.
5. Integration – On all levels we should think about how we can better integrate clinical activities with research activities. The participants mentioned aspects such as education and training, financing, quality of care etc. A possible method for integration is the creation of 'health campuses'.
6. Care assessment – Finally, it was argued that making resources available for the scientific assessment of care-oriented and patient-oriented outcomes is highly necessary.

Theme 4 – Care for RDs – Centres of Expertise and European Reference Networks for Rare Diseases

Actions in the Belgian Plan that relate to this theme: action 1, 2, 3, 4, 5, 7, 8, 9, 10, 11, 15

Centres of Expertise and networks of expertise are the cornerstones of each integrated approach for rare diseases. All the experts, physicians and patients agree on this. Only by organising the expertise into multidisciplinary teams the quality of care can be guaranteed.

The Belgian Plan for Rare Diseases states that it is expected of the current reference centres ('mucoviscidosis', 'metabolic disorders', 'refractory epilepsy' and 'neuromuscular diseases'), which now work with the Belgian National Institute for Health and Disability Insurance (RIZIV/INAMI) on the basis of conventions (contracts), that they will evolve into Centres of Expertise under the same recognition system as the new Centres of Expertise. Furthermore, the creation of new Centres of Expertise for haemophilia is planned.

The creation of other Centres of Expertise for the upcoming years has also been announced. These new centres will have to comply with certain characteristics, conditions established by Royal Decree. It will depend on the expertise present in Belgium, the needs (established by the working group on rare diseases of the Observatory) and a selection tool developed by an ad hoc working group of the College of Physicians-Directors and the scientific department of the Observatory for which diseases Centres of Expertise are created. Depending on the available budgets, this will start as from 2015.

Actions have also been planned with regard to networking and care coordination functions as well as with concerning the reinforcement of the input of genetic centres in diagnostics,

counselling and information provision. Finally, a function 'rare diseases' is planned in hospitals that supply a whole range of activities which support the treatment and the care of patients.

During the well-attended workshop the role of the Centres of Expertise was explained from both the patients' and the experts' point of view by Claude Sterckx of the Belgian Mucoviscidosis Association and Prof. Martine Cools, coordinator of the multidisciplinary Disorders of Sex Development (DSD) Team of the University Hospital of Ghent, respectively.

Claude Sterckx talked about the influence that the centres for mucoviscidosis have had on the treatment and support of patients with mucoviscidosis for the past 15 years. He described the advantages of Centres of Expertise on the basis of the experience of the reference centres for mucoviscidosis and the impact that they have had. Mr Sterckx also emphasised the importance of the 'network': a network between people and institutions for specialised care, both within and outside of Belgium, and for medical, paramedical, psychological as well as social care. However, the network also has to make connections with the primary and secondary health care. After all, it is these health care providers that are involved in the daily care of the patient. Furthermore, the contact with and the approach of the patients need to be comprehensive, with attention to all of their needs and providing them with concrete answers.

From the point of view of the patients an approach to care in centralised and specialised centres was advocated on the one hand, but on the other hand the importance of an open and transparent network of health care services with attention for an optimal referrals was stressed as well. Furthermore, Sterckx emphasised the fact that not all rare diseases require the same approach. In other words, it is necessary to meet the specific needs of the patients. We have to progressively incorporate a number of rare diseases and treat them in official and financially supported Centres of Expertise. But at the same time a lot of useful work can be done for *all* rare diseases by improving and accelerating the diagnosis, even unrelated to the Centres of Expertise. That is precisely why it is important that the patients are given a participative role in the creation, the functioning and the assessment of the Centres of Expertise. Unfortunately, this is not included in the current Belgian Plan.

Prof. Martine Cools, coordinator of the multidisciplinary DSD Team of the University Hospital in Ghent, explained by means of a number of concrete examples how everything goes in a specialised multidisciplinary centre (in her case a multidisciplinary team focusing on sex development disorders in children and adolescents) and which patients' needs the team tries to fulfil. Prof. Cools stated that in reality there is hardly any support for the time investment and the level of specialisation that is required by each case, for paramedic and psychological care, for coordination functions and for the provision of information to patients and society. Furthermore, the legal basis should be re-adjusted (with respect to orphan medicinal products, data collection and compulsory referral). Flexibility as to the structural framework is still problematic as well. Prof. Cools looks at the Belgian Plan for Rare Diseases from an expert's and specialised physician's point of view and uttered various concerns, for example about the underestimation of the current situation, the financial needs as well as the late and limited

consultation of the professional health care providers. She also criticised the fact that there is no information available about which new Centres of Expertise are to be founded and that no budget has been put aside, and fears that the selection process will lack all transparency.

Prof. Marc Abramowicz talked about the contribution of the genetic centres to the diagnosis and treatment of patients with a rare disease. In the Belgian Plan for Rare Diseases these centres are given a central role. The financing for diagnostic and follow-up tests will be improved, as well as the quality control in the centres. Furthermore, a genetic counselling will be organised in the current reference centres and Centres of Expertise.

After each presentation the participants in the workshop had a thorough discussion and the following conclusions were drawn:

1. Networking – With regard to networking, it is important that the networks between experts and Centres of Expertise are developed. The interaction between the Centres of Expertise and the actors in the primary and secondary health care may not be forgotten either. The involvement of the primary and secondary health care needs to be further elucidated in the Belgian Plan.
2. Criteria for Centres of Expertise – The criteria that will be used to recognise Centres of Expertise or to grant 'expertise' to centres is unclear. Furthermore, there is some concern with regard to the institution that will formulate these criteria, which procedures will be followed and how the criteria will be applied in practice. The participants in the workshop had many questions with regard to this matter, which remained unanswered. They are therefore very concerned in this respect.
3. Which diseases – There is not much transparency at the moment with regard to which diseases will be incorporated in the Belgian Plan for Rare Diseases, just as little as about how the priorities will be determined.
4. Budget – The funding that is put aside needs to be sufficient in order to enable the rollout of the Plan. This does not seem to be the case with the budget that is set aside at the moment. There is no budgetary perspective for the development and recognition of additional Centres of Expertise (in spite of the fact that this is a cornerstone of the Plan) and various measures in the Plan were assigned no or a very tight budget and will therefore be hard to realise. Furthermore, the existing bottlenecks are not resolved, i.e. the reimbursement of psychosocial services, the intellectual performances of physicians.
5. Registries and databases – It is unclear whether and how the various initiatives regarding registries, databases, electronic exchange platforms and electronic patient records will be connected to one another or be integrated. Within the initiatives in eHealth an electronic patient record is mentioned; within the measures for chronic diseases a multidisciplinary patient record is spoken of; in the genetic centres a genetic registry is being developed and in the Plan for Rare Diseases a Central Registry of Rare Diseases is in the pipeline. To which extent can it be avoided that these evolutions take

place separately without being geared to one another?

6. Communication – The government needs to communicate clearly on the future developments and the implementation of the Plan. The current version lacks a structured and detailed step-by-step plan about how and when the different measures will be realised and which process and outcome indicators are connected to these measures. Furthermore, nothing was said about the step of the recommendations published by the Fund for Rare Diseases and Orphan Drugs in the current Plan and many important stakeholders had the feeling that they were not consulted enough.

Theme 5 – Orphan Medicinal Products

Actions in the Belgian Plan that relate to this theme: action 12, 14, 15

Although there are thousands of rare diseases, only over 80 Orphan Medicinal Products have a market authorisation in the EU since 2001. In other words, there is a gigantic unmet medical need in patients with a rare disease. Furthermore, the access to Orphan Medicinal Products with an EU market authorisation is different depending on the specific member state. One of the indicators suggested by EUCERD is therefore to look into the access to authorised Orphan Medicinal Products in every member state. Furthermore, EUCERD wants to check to which extent the members states have worked out programmes in order to enable the access to medicinal products that have not been authorised yet (type 'compassionate use programmes').

In addition to this, measures need to be taken in order to optimise the (continuous) data collection regarding the functionality, safety and efficacy of the medicinal products. Furthermore, additional supporting mechanisms need to be devised in order to enhance the chance of success of small and medium-sized enterprises that develop Orphan Medicinal Products, as these companies often take big risks while they are hardly supported or protected.

Dr. pharm. Greet Musch of the Federal Agency for Medicines and Health Products (FAGG) talked about the problem of the unmet medical need in Belgium for patients with a rare disease and about the measures that are currently there and that are worked out. She mentioned an initiative of the government and the sector to organise a 'bioplatform' for clinical studies in an early stage, which should promote the accessible participation of patients and act as a kind of stimulus to companies to carry out clinical studies in Belgium.

Belgium has had 'Compassionate Use' and 'Medical Need' programmes for a number of years now. Furthermore, Belgium is working on a new procedure to enable a financial compensation of the medical insurance for innovative medicines filling an unmet medical need (project 'unmet medical need') for patients with a serious disease. Medicinal products that are not authorised yet should be made available and covered by the medical insurance more quickly, even before they are registered at a European level. For medicinal products with an authorisation this can be done before a new indication is recognised. However, there has to be

an unmet medical need each time.

Dr. Daniel Brasseur of the Federal Agency for Medicines and Health Products (FAGG) emphasised that the data collection for medicines for rare diseases is an enormous challenge. For Orphan Medicinal Products, criteria such as functionality, efficacy and safety need to be dealt with just as carefully as it is the case with other medicines. However, for Orphan Medicinal Products, it is imperative – even more so than for other medicines – that during the commercialisation new evidence about the medicines is collected. This requires a new approach, both as to market authorisation ('adaptive licensing') and reimbursement. To this end, new ideas need to be formed in which both the government, the industry, the medical-scientific world and the patients work together as partners.

By means of the example of rare cancers, Prof. Dominique Bron of the Institut Bordet stressed the indispensable character of a national and international networking for the diagnosis, prevention and treatment of rare diseases. In order to be able to treat patients with a rare form of cancer better, Prof. Bron put forward four important foundations: diagnostic assessment, treatment/clinical research, translational/fundamental research, research on quality of life and outcomes. National and international networking both significantly contribute to each of these phases. For the diagnostic assessment a central review by pathologists and molecular biologists might reduce the number of misdiagnoses, as was stated recently in a KCE report that argued in favour of Centres of Expertise for rare cancers (KCE is the Belgian Health Care Knowledge Centre). For the treatment and the clinical research the European cooperation is necessary. This is shown by the success of current studies that are coordinated by platforms for cooperation such as the EORTC, ESMO, EHA etc. These types of collaborations are indispensable, especially to guarantee that patients get access to clinical studies and even more so if we are dealing with rare cancers or personalised treatments.

With regard to translational and fundamental research, developing and sharing biobanks is an important added value. Finally, national and international networks of patients and social media platforms for patients (type www.Esperity.com) can also provide important information about the quality of life of patients and about patient-related outcomes. In short, cooperation and networking are important keys in order to make progress in the approach of rare diseases, and in particular rare cancers.

Dr. Wilfried Dalemans and Dr. Eric Halioua from Tigenix and Promethera Biosciences respectively discussed into detail the problems that small and medium-sized enterprises (SMEs) have to cope with when they accept the challenge of developing new Orphan Medicinal Products or orphan treatments. For all the phases of the development process (research, the regulatory phase (market access), price setting and reimbursement phase), SMEs have specific requests in order to make the sometimes difficult cooperation with other partners (academic centres, EMA and national authorities respectively) go more smoothly. Especially a clear framework, both for ATMP (advanced therapy medicinal products) and Orphan Medicinal Products at the level of EMA is required to enable a quicker access to the market.

Furthermore, it is necessary that a harmonisation of the reimbursement is achieved in all the member states. With regard to support of research by the government, an extension of the principle of the tax shelter to biomedical SMEs is put forward.

The following recommendations were given by the participants in this workshop:

1. Involvement of the patients – The patients need to be involved more quickly and to a higher extent in the development of Orphan Medicinal Products. Examples were given of Orphan Medicinal Products that were available on the market but were very hard to administer, and medicines that were used off-label and for which the company itself was not even aware of this off-label use. In short, patients need to be involved more quickly and to a higher extent.
2. Support to SMEs – Small and medium-sized enterprises (SMEs) are often the pioneers in the research on Orphan Medicinal Products. They depart from a brilliant idea but have to realise it with very limited resources: they have revenues and only restricted access to financing and subsidies. These pioneer companies deserve to be better protected, for example by means of an extension of the tax shelter principle.
3. Rare cancers – More than half of the Orphan Medicinal Products have rare cancers as indication. Rare cancers are an important domain within the group of rare diseases. Both in the Belgian Plan for Rare Diseases as well as in the Cancer Plan not much attention is spent on rare cancers. However, important initiatives need to be developed with regard to biobanks, clinical studies, creating Centres of Expertise and networks for rare cancers. The Belgian Health Care Knowledge Centre (KCE) recently published a study showing the importance of these initiatives. The study formulates a number of recommendations similar to the ones that were already put forward for other rare diseases. Also on a European level attention will be paid to the approach of rare cancers in the near future.
4. Harmonisation and simplification – Clinical studies for rare diseases are complex, because patient populations are spread over different hospitals across national borders. This often means that for an Orphan Medicinal Products, only cross-border clinical studies are possible. But each member state has its own legislation, its own system and its own procedures with regard to clinical research, obliging every country to hand in a separate and adjusted approval file. These differences, and often contradictions, significantly impede the execution of international clinical studies with small groups of patients. That is why a harmonisation and simplification of the procedures between the different member states is argued for. This plea is not only applicable to the execution of European clinical studies, but also to the reimbursement of Orphan Medicinal Products.
5. Unmet medical needs – The procedures that are set up for unmet medical needs may under no circumstances stand in the way of the development of new Orphan Medicinal Products.

Theme 6 –Social Services for Rare Diseases

Actions in the Belgian Plan that relate to this theme: action 5, 7, 13, 15

EUCERD wants to include a sufficient amount of initiatives in national plans or strategies in order to support people with a rare disease (and the people that are closest to them as well as carers) in everyday life, so they are better integrated in society, at school and/or on the labour market. In the EUCERD report of November 2012 about the 'Need for special services and integration into special policies' specialised social services are mentioned as a tool to promote integration. That is why a lot of attention is spent on the foundation of Centres of Expertise within a global network and especially the role of care coordinators (although they are no health care providers). In addition to this, new ways of information provision need to be found which fulfil the individual needs in a more suitable way. Finally EUCERD refers to respite centres as an indicator of the policy on rare diseases. We want to know which specific role such like respite centres can play and which conditions they would have to comply with.

By means of a practical example – the" Plateforme Régionale d'Information et d'Orientation" sur les maladies Rares des Pays de la Loire (PRIOR) – Magali Colinet of the project PRIOR (France) and Dominique Le Berre of the Alliance for Rare Diseases (Alliance Maladies Rares – France) showed how patient-oriented care coordination can be set up. PRIOR serves an area of 165,000 inhabitants in the Loire region. The organisation keeps close contact with three target groups: patients and their circle of acquaintances, professional health care providers and patients' organisations. The platform maps out the needs of patients on the basis of questions and individual cases and guides these patients in everyday life. The emphasis hereby is on valorising existing competences and integrating the patient in society as well as possible. Furthermore, the platform promotes and shares good practices.

André Gubbels of the Federal Public Service for Social Security talked about the 'labyrinth of information provision' in the social sector. He argued that the social sector is shattered and divided along social classes. Nowadays, we have specific services for children, adolescents, disabled people, the elderly etc. The services are categorised according to the sector: health, social matters, education, labour, justice etc. and work under different powers: the federal state, the communities or the regions, and the local municipal level. This affects all the parties involved – clients, helpers (e.g. social workers) and the government. Although everyone recognises the problem, only traditional solutions are worked out: the creation of another information point, contact centre, coordination service etc. According to André Gubbels, the information provision to the citizen needs to be completely reviewed. The care-providing organisation should no longer be the central point from which the information departs, but the information needs to be centred around the citizen asking for care and information. Although this total turnaround might seem utopian, it is not. After all, there are internet applications available today that would easily enable this kind of adjustment.

To conclude, Danielle Huse of Villa Rozerood in De Panne and Thijs Verbruggen of the Zeepreventorium in De Haan made a warm plea in favour of respite centres. They should more explicitly and to a higher extent be involved in the treatment of patients with a rare disease. Of course these respite centres need to comply with a number of quality criteria and have to closely cooperate with Centres of Expertise.

The following recommendations were made by the participants in this workshop:

1. Information – Structure the provision of information around the people who should receive the information and not around who gives the information (i.e. around the citizen instead of around the administration/health care provider). In other words, the roles need to be turned around. If the patient types in a keyword on his or her computer, tablet or smartphone, he has to be able to access all the services immediately. The technology to realise this is already available.
2. Access to information – The citizen/patient owns the data, so he should be able to gain access to them and consult them. The technology to realise this is already available, so it needs to be implemented now.
3. Care coordination – It is important that the person (or the team) coordinating the care from the Centres of Expertise also pays attention to the coordination of the care close to the patient, e.g. by identifying the competences in the region and making them known (by means of a registry for example) and by visiting the patient at home so it is clear what his/her needs are.
4. Integration and participation – The interventions of the care coordinator have to go beyond just care, sensu stricto. We need to have an integrated care pathway, with attention to everyday life and the integration in society (school/work, adjustments in the environment etc.)
5. Respite centres – Specialised rehabilitation and respite formulas for certain rare diseases need to be given an equal place within the holistic care, complementary to the reference centres and Centres of Expertise.

Additional Workshops (*optional*)

None

Report of the Closing Session - Conclusions

This session was moderated by Peter Raeymaekers, who invited a representative from each workshop to report the most important findings and suggestions to the plenary. Peter Raeymaekers also concluded the Conference:

At the moment of the EUROPLAN National Conference the Belgian Plan for Rare Diseases had only been published for a couple of weeks. The Plan consists of 20 measures in 4 domains and is partly based on the 42 recommendations and proposed measures that were issued by the Fund for Rare Diseases earlier. This Fund is a broad platform managed by the King Baudouin Foundation in which more than 75 individual stakeholders, including representatives of patients, participated. Furthermore, other measures in the current Belgian Plan for Rare

Diseases are based on initiatives that were already in the pipeline.

Some of the measures of the Plan are currently being implemented; others will be carried out shortly. For some measures, one is still waiting for additional budgets. At the moment € 4.7 million is spent on specific measures every year. Once it is at cross speed, an annual budget of € 15.7 is planned.

Various measures of the Plan were extensively discussed during the workshops (see report of the individual workshops). However, at this stage it is still too early to make a 'comprehensive' analysis and to draw conclusions on the Belgian Plan for Rare Diseases with regard to the EUROPLAN/EUCERD indicators. There are still too many uncertainties. For some measures the description in the current version of the Plan is still too brief and no underlying policy documents have been released yet. A number of Royal Decrees are being prepared, but the texts were not yet available at the moment of the National Conference.

Furthermore, important elections are taking place in Belgium in the near future. While in the whole of the EU citizens will be in the voting booth around 25 may 2014, electing the new members of the European Parliament, the Belgian people will be choosing their representatives for the Federal Parliament and the federated entities (communities and regions) at the same time. This means that after 25 May new governments will be formed at various levels in Belgium, which will undoubtedly have an influence on the implementation of the Belgian Plan for Rare Diseases.

Annexe I – Programme in English

EUROPLAN NATIONAL CONFERENCE– 28 FEBRUARY 2014
Diamant Conference Center, Brussels

<table>
<tr><td>08.30
–
09.15</td><td colspan="3">Welcome</td></tr>
<tr><td>09.15
–
10.15</td><td colspan="3">Plenary session: Introduction

Opening
Ingrid Jageneau, President of RaDiorg.be

The national plan for rare diseases
Ms Laurette Onkelinx, Minister of Social Affairs and Public Health

European guidelines and EUROPLAN joint action
Pol Gerits, EUCERD representative for Belgium / Federal Public Service for Public Health
Lene Jensen, Chief Executive Officer of Rare Disorders Denmark and EURORDIS-EUROPLAN advisor for Belgium</td></tr>
<tr><td>10.30
–
12.00</td><td>Workshop 1: Care for RDs – Centres of expertise and European reference networks for rare diseases

Chair: Bruce Poppe, Ghent
Rapporteur: Marc Abramovicz, VUB

1. The role of centres of expertise from the patients' point of view
Claude Sterckx, Belgian Mucoviscidosis Association

2. The role of genetic centres
Elfriede De Baere, College for Genetics

3. The role of centres of expertise from the experts' point of view
Martina Cools, DSD team of the University Hospital of Ghent</td><td>Workshop 2: Definition, codification and inventorying of rare diseases

Chair: Herman Van Oyen, WIV-ISP
Rapporteurs: Elfriede Swinnen & Montse Urbina- Paz, ISP-WIV

1. Definition
Bettina Blaumeiser, CMG – University Hospital of Ghent

2. Codification
Ingrid Mertens, Federal Public Service for Public Health, Data Management

3. Inventorying
Viviane Van Casteren, ISP
Séverine Henrard, IRSS - UCL</td><td>Workshop 3: Current needs and priorities with regard to fundamental, clinical and translational research on rare diseases

Chair: Pr. René Westhovens, University Hospital of Leuven / president of the RIZIV/INAMI college for orphan drugs
Rapporteur: Freia Van Hee, NFWO

1. How to reduce the gap between fundamental and translational research
Erik Legius, Centre of Human Genetics – University of Leuven

2. Financing of research in Belgium
Arnaud Goolaerts, NFWO Belgium

3. Financing of research in Europa
Irene Norstedt, European Commission – DG Research & Innovation</td></tr>
<tr><td>12.30
–
13.45</td><td colspan="3">Lunch break</td></tr>
</table>

13.45 – 15.15	***Workshop 4: Methodology, governance and monitoring of the national plan*** *Chair: Ingrid Jageneau, Radiorg* *Rapporteurs: Lode De Bot, Flemish Patient Platform & Jonathan Ventura, Radiorg* **1. Indicators for the monitoring of the plan** *Lene Jensen, Rare Disorders Denmark* **2. Patient participation** *Ilse Weeghmans, Flemish Patient Forum*	***Workshop 5: Orphan medicinal products*** *Chair: André Lhoir, FAGG* *Rapporteur: Marc Dooms, Fund for Rare Diseases and Orphan Drugs* **1. Unmet medical needs** *Greet Musch, FAGG* **2. Evidence generation plan** *Daniel Brasseur, FAGG* **3. Network** *Dominique Bron, Institut Jules Bordet* **4. Support to SMEs** *Wilfried Dalemans, Tigenix* *Erik Halioua, Promethera*	***Workshop 6: Social services for rare diseases*** *Chair: Claude Sterckx, Mucoviscidosis Association* *Rapporteur: Chris Van Hul, MLOZ* **1. How to promote the foundation of centres of expertise within the global network? What about the role of care coordinators?** *Magali Colinet, PRIOR, France* *Dominique Le Berre, Alliance for Rare Diseases France* **2. How to provide the patients with adjusted information? How to integrate new tools so the information is better adjusted to the individual needs?** *André Gubbels, Federal Public Service for Social Security* ***3.*** **To which extent are respite centres 'specific' for rare diseases and what do they have to comply with?** *Danielle Huse, Villa Rozerood* *Thijs Verbruggen, Zeepreventorium*
15.15 – 15.30	***Distribution of the Edelweiss Award***		
15.30 – 17.00	***Plenary session: closing*** ***Presentation of the recommendations that were formulated in the different working groups and final discussion*** *Moderator: Peter Raeymaekers*		
17.00 – 18.00	***Goodbye drinks***		

Annexe II – Final List of Participants

Name	Surname	Organisation, company...	Stakeholder groups
Kouadria	Abderaouf	Etablissement publique de santé et de proximité	Healthcare professional
Marc	Abramowicz	ULB – Genetics	Healthcare professional
Chris	Aubry	Radiorg	Patient representative
Jean-Pierre	Baeyens	Observatorium Chronische Ziekten RIZIV	Insurer
Jacques	Barbieaux	APTES-Belgique (Vice-président)	Patient representative
Vinciane	Berckmans	H.A.E. Belgium	Patient representative
Georges	Binamé	Parlement Fédéral Belge	Politician
Bettina	Blaumeiser	UZA/UA, Medische Genetica	Healthcare professional
Daniel	Blockmans	UZ Gasthuisberg, Leuven	Healthcare professional
Carole	Bodart	Bristol-Myers Squibb	Industry
Gerard	Boehm	Actelion Pharmaceuticals	Industry
Jacques	Boly	ANMC	Insurer
Valentina	Bottarelli	Eurordis	Patient representative
Léon	Brandt	Association Belge du syndrome de Marfan	Patient representative
Daniel	Brasseur	AFMPS	Public administration
Dominque	Bron	Institut Jules Bordet	Healthcare professional
Marc	Buchet	Orphan EU	Industry
Jean-Jacques	Cassiman	Fund Rare Diseases KBS	Academic
Magali	Colinet	PRIOR	Healthcare professional
Wim	Colle	Vzw pulmonale hypertensive	Patient representative
Jean-Marc	Compère	asbl X fragile – Europe	Patient representative
Martine	Cools	DSD Clinic, UZ Gent	Healthcare professional
Nadia	Costantino	BOKS vzw	Patient representative
Albert	Counet	Ligue Huntington Francophone Belge asbl	Patient representative
Karin	Dahan	Institut de Pathologie et de Génétique - Centre de Génétique Humaine	Healthcare professional
Eline	Darquennes	MSD Belgium	Industry
Julie	De Backer	University Hospital Ghent	Healthcare professional
Lut	De Baere	BOKS vzw	Patient representative
Koen	De baets	Orphan EU	Industry
Lode	De Bot	Vlaams Patiëntenplatform	Patient representative
Rudy	De Cock	PFIZER n.v.	Industry
Thibault	de Lary de Latour	Alexion	Industry
Henri	De Ridder	RIZIV – INAMI	Insurer
Marcel	De Wulf	vzw Belgische Vereniging voor Longfibrose (vzw BVL)	Patient representative
Annelies	Debels	Studiedienst Groen	Industry
Michelle	Deberg	Centre de génétique humaine de Liège,	Healthcare professional

François-Guillaume	Debray	Department of Medical Genetics, CHU Liège	Healthcare professional
Karen	Degroote	centre for medical innovation	Academic
Nathalie	Delbrassine	CHR CItadelle Liège	Healthcare professional
Marion	Delcroix	UZ Leuven	Healthcare professional
Belinda	Delys	Novartis	Industry
Godelieve	Depla	vlaamse vereniging voor erfelijke bindweefdsel aandoeningen	Patient representative
Marc	Dooms	University Hospital Leuven	Academic
Winand	Eerens	AHVH vzw Hemofilievereniging	Patient representative
Gerry	Evers-Kiebooms	Department Human Genetics, KU Leuven	Healthcare professional
Stefaan	Fiers	Bristol-Myers Squibb Belgium SA	Industry
Karel	Fol	Shire	Industry
Charissa	Frank	Vlaamse Vereniging voor Erfelijke Bindweefselaandoeningen	Patient representative
Nicola	Fresu	bxl Europe	Industry
Natalia	Garcia Salcedo	Volcano	Industry
Pol	Gerits	FOD Volksgezondheid	Public administration
Philippe	Ghyssels	AbbVie	Industry
Guy	Gillard	Association belge des paralysés-section polio	Patient representative
Yves	Gillerot	RDB	Patient representative
Caroline	Gillissen	Alpha-1 Advocacy and Action Coalition	Patient representative
Arnaud	Goolaerts	FNRS, Fonds de la Recherche Scientifique	Academic
Paul	Guijt	Gaucher patient	Patient representative
Filomeen	Haerynck	Departement of pediatric Pulmonology and immunology	Patient representative
Samia	Hammadi	Scientific Institute of Public Health	Public administration
Séverine	Henrard	Université catholique de Louvain/Institut de Recherche Santé et Société	Academic
Hilaire	Herrezeel	None	Patient representative
Marleen	Hoebus	Amgen	Industry
Danielle	Huse	Villa Rozerood	Social worker
Christian	Huyghe	CHHC	Healthcare professional
Ingrid	Jageneau	Radiorg & Debra Belgium	Patient representative
Anne	Jambor	Boppi	Patient representative
Anna	Jansen	UZ Brussel	Healthcare professional
Lene	Jensen	Eurordis/Rare Disorders Denmark	Patient representative
Verena	Jirgal	Rohde Public Policy	Industry
Yvonne	Jousten	Association Belge du Syndrome de Marfan ABSM	Patient representative
Marleen	Kaatee	PSC Info Europe (PSCIE)	Academic
Veerle	Kempeneers	Pfizer	Industry

Régine	Kiasuwa	WIV/ISP	Public administration
Daniel	Knockaert	university hospital Leuven, general internal medicine	Academic
Claude	Krygier	MSD	Industry
Dominique	Le Berre	Alliance Maladies Rares, France	Patient representative
Erik	Legius	UZ Leuven, centrum menselijke erfelijkheid	Healthcare professional
Kelly	Lentini	Bindweefselaandoeningen	Healthcare professional
André	Lhoir	AFMPS/FAGG	Public administration
Fransiska	Malfait	Center for Medical Genetics, Ghent University Hospital	Academic
Annelies	Mallezie	WIV-ISP	Public administration
Peter	Mathijs	SOBI	Industry
Stefan	Mattheeuws	Contactpuntncl	Patient representative
Luc	Matthysen	HTAP BELGIQUE ASBL	Patient representative
Isabelle	Maystadt	Centre de Génétique Humaine, IPG	Healthcare professional
Véronique	Mège-Sarek	Radiorg	Patient representative
Ingrid	Mertens	FOD Volksgezondheid, Datamanagement	Public administration
Tom	Meuleman	BioMarin Europe	Industry
Muriel	Mignolet	Genzyme	Industry
Hamed	Mobasser	Rohde Public Policy	Industry
Yannh	Moray	asbl X fragile – Europe	Patient representative
Geert	Mortier	Department Medical Genetics Antwerp	Academic
Gustaaf	Nelis	NVSM	Patient representative
Violeta	Nikolova	Weber Shandwick	Industry
Irene	Norstedt	EC, DG Research & Innovation. Head of Innovative and Personalized Medecine	Public administration
Sandra	Paci	Shire	Industry
Louis	Paquay	Wit-Gele Kruis van Vlaanderen	Other
Julien	Patris	Celgene	Industry
Huong	Phan Thi	PUBLIC HOSPITAL	Healthcare professional
Alessia	Pintus	bxl Europe	Industry
Alice	Pintus	faber	Patient representative
Gregory	Piron	Dyskinesia	Patient representative
Vanessa	Pirottin	Ligue Huntington Francophone Belge	Patient representative
Bruce	Poppe	Ghent University Hospital	Healthcare professional
Evy	Reviers	ALS Liga België vzw	Patient representative
Jessica	Robinson	CHRU Lille, Centre de Référence Maladies Rares - STB et Cervelet	Healthcare professional
Bruno	Santoni	PPTA	Industry
Michèle	Scaillon	HUDERF	Healthcare professional
Elisabeth	Schraepen	AbbVie	Industry
Karin	Segers	human genetics CHU Sart tilman Liège	Healthcare professional
Julie	Serre	Ligue Huntington Francophone Belge	Patient representative

Guillaume	Smits	HUDERF - IB2 – ULB	Healthcare professional
Ovidio	Soler Leonarte	Rohde Public Policy	Industry
Delphine	Stokard	GESED	Patient representative
Jan	Swiderski	Rohde Public Policy	Industry
Elfriede	Swinnen	WIV-ISP	Public administration
Erik	Tambuyzer	CMI vzw	Academic
Alphonse	Thijs	INAMI	Insurer
Viviane	Tordeurs	Retina Pigmentosa ASBL	Patient representative
Annemie	T'Seyen	King Baudouin Foundation	Academic
Montse	Urbina	WIV-ISP	Public administration
Veronique	Van Assche	RDB	Patient representative
Johan	Van Calster	CLIVAN bvba, Policy and Government Office for Medicinal Products	Industry
Viviane	Van Casteren	Scientific Institute of Public Health	Public administration
Jean-Luc	Van Cauwenbergh	Alkaptonuria	Patient representative
Elisabeth	Van Damme	GlaxoSmithKline	Industry
Saskia	Van den Bogaert	FOD Volksgezondheid	Public administration
Herman	Van Eeckhout	pharma.be	Industry
Katrien	Van Elk	Shire	Industry
Katrien	Van Geyt	Bayer	Industry
Freia	Van Hee	FNRS, Fonds de la Recherche Scientifique	Academic
Ine	Van Hoyweghen	KU Leuven	Academic
Joanna	Van Reyn	CMP Vlaanderen vzw	Patient representative
Sonja	Van Weely	ZonMw	Patient representative
Stéphane	Vandendael	Genzyme - a sanofi company	Industry
Tinne	Vandensande	Koning Boudewijnstichting	Academic
Omer	Vanhaute	SPF santé publique	Public administration
Jonathan	Ventura	Radiorg	Patient representative
Thijs	Verbruggen	Zeepreventorium	Social worker
Kristof	Verschaetse	PSC Info Europe (PSCIE)	Academic
Alice	Vicaire	Celgene sprl	Industry
Miikka	Vikkula	UCL	Healthcare professional
Marie-Françoise	Vincent	Cliniques universitaires Saint-Luc	Healthcare professional
Wouter	Vyvey	Ugent - faculteit Gezondheidswetenschappen	Academic
Françoise	Wauthy	GESED	Patient representative
Ilse	Weeghmans	Vlaams Patiëntenplatform	Patient representative
René	Westhovens	KU Leuven, president orphan drug colleges RIZIV/INAMI	Insurer
Arabelle	Willems	ABMM	Industry
Wim	Wuyts	unit for iterstitial lung diseases, pneumology UZ Leuven	Healthcare professional