BELGIUM

EUROPLAN ROUND TABLE

in the framework of the EU Joint Action RD-ACTION

6 October 2017, Brussels

FINAL REPORT

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FOREWORD

The EUROPLAN national workshops / round tables are organised in many European countries as part of a coordinated and joint European effort to foster the development of comprehensive National Plans or Strategies 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/ workshops are jointly organised in each country by a National Alliance of rare disease patients' organisations and EURORDIS–Rare Diseases Europe. **Rare Disease National Alliances and Patient Organisations have a crucial role to shape the national policies for rare diseases.**

The strength of EUROPLAN national conference/ workshop lies in its shared philosophy and format:

- **Patient-led**: National Alliances are in the best position to address patients' needs;
- **Multi-stakeholders**: National Alliances ensure to invite all stakeholders involved for a broad debate;
- **Integrating both the national and European approach to rare disease policy;**
- **Being part of an overarching European action** (project or Joint Action) that provides the legitimacy and the framework for the organisation of EUROPLAN national conferences/workshops;
- **Helping national authorities adhere to the obligations stemming from the Council Recommendation of 8 June 2009 on an action in the field of rare diseases.**

Since 2008, National Alliances and EURORDIS have been involved in promoting the adoption and implementation of National Plans and Strategies for rare diseases. Altogether, 41 EUROPLAN national conferences took place in the framework of the first EUROPLAN project (2008-2011) and the EU Joint Action of the European Committee of Experts on Rare Diseases – EUCERD - (2012-2015).

Within RD-ACTION (2015-2018), the second EU Joint Action for rare diseases, National Alliances and EURORDIS continue to get involved in a coordinated European effort to advocate for and promote integrated national policy measures that have an impact on the lives of people living with rare diseases.

The EUROPLAN national conferences or workshops taking place within RD-ACTION focus on specific themes identified by the National Alliances as the most pressing priorities to tackle with national authorities. These thematic priorities are addressed in sessions where all the stakeholders discuss relevant measures to be taken or ways to sustain the full implementation of already approved measures.

Each National Alliance prepares a final report on the national workshop, based on a common format such as the one that follows.

GENERAL INFORMATION

Country	Belgium
National Alliance (Organiser)	RaDiOrg.be
Date & place of the national workshop/conference	6 October 2017, Brussels
Website	www.radiorg.be
Members of the Steering Committee	RaDiOrg and the Fund for Rare Diseases and Orphan Drugs (Fondation Roi Baudouin)
Theme addressed	**Governance of the Belgium National Plan for Rare Diseases**
Annexes :	I. Programme in English II. List of Participants (by stakeholders' categories)

FINAL REPORT

EUROPLAN Round Table on the Governance of the Belgium National Plan for Rare Diseases

Note: Opinions expressed by participants are theirs alone and do not constitute Radiorg.be's position within the context of this round table.

DIAGNOSTICS

Representatives of the Scientific Institute of Public Health (ISP, *Institut de Santé Publique*) briefly discuss the status of the Plan with regards to diagnostics: the central registry for rare diseases has been created, a budget has been set aside for the DNA tests sent abroad.

Despite this progress, genetics centres that provide **DNA tests** are having issues with the new types of tests (exome or genome analysis, for example) and their reimbursement. This makes it extremely difficult to develop modern tests with the present nomenclature.

A second element relates to **tests that are sent abroad**, which are very important for rare diseases. In fact, for some of these tests, there are only a few laboratories in the world that can perform these tests. The budget available under the convention for "genetic counselling" is limited, and therefore,

tests are only sent abroad if having a final diagnosis has immediate therapeutic benefits or if the family needs genetic counselling.

In general, genetic testing is under-funded in Belgium. At present, EUR 40 million are allocated to genetics testing in Belgium, of which only 2 million is allocated to genetic counselling; this per capita budget is far lower than that of our neighbouring countries, such as the Netherlands, Germany...

This leads to various problems. On the one hand, patients are not treated equally, since some do not benefit from a final diagnosis if their tests are not sent abroad. This means that patients cannot put a name to their disease, which is a problem in itself for the patient, especially psychologically. On the other hand, as a result, if a treatment is discovered for the rare disease concerned, this patient will not have access to it since they will not be listed with a final diagnosis in the databases or records.

The Rare Disease Function is in charge of arranging patient's access to a diagnosis through **genetic counselling consultations** or through multidisciplinary consultations. The Rare Disease Function is a hospital function which aims to take care of patients with rare diseases. This care includes getting a diagnosis as quickly as possible, follow-up care, and transferring patients to care units that are best suited to treating their pathology. Various conditions have to be met. Seven Rare Disease Functions have been recognized in Belgium. However, patients are faced with various challenges in this respect as well. The fact that these functions (which were only recognized in June 2016) are still not financed to date, prevents support structures from developing. Today, these rare disease functions remain an abstract concept for patients and have no positive impact on their daily lives. The Federal Public Service's representative confirms that there is a budget, however, this budget will only become available in July 2018, at the latest. Furthermore, Belgium has few geneticists even though the demand for tests has been rapidly increasing in recent years, and not only for rare diseases (paediatrics, oncology, etc.). The lack of geneticists could have something to do with the fact that this speciality was only recently recognized in Belgium.

There is also a lack of funding for these genetics tests, despite NIHDI establishing the genetic counselling convention a few years ago.

All these factors imply long waiting lists, which means that patients have to wait a long time to get a diagnosis. Misdiagnosis remains an issue for patients with rare diseases, as illustrated by the representative of the HTAP association. In the case of pulmonary hypertension (a progressive rare disease) the time needed to get a diagnosis and finalize the process means the patient's disease could already be very advanced with severe and irreversible consequences. One of the issues identified by patients is primary care physicians' (General Practitioners and specialist consultants) lack of awareness with regards to rare diseases. Meanwhile, for HTAP, the challenge is getting the diseases diagnosed by primary care physicians so that they can then be directed to medical care centres (not officially recognized) in Belgium.

RECOMMENDATIONS

(1) The rare disease function needs to get funding quickly so that they can start to establish support structures and care for patients who are waiting for a diagnosis. These structures will be multidisciplinary and will enable them to organize a multidisciplinary review of files and direct patients to the best medical care centres.

(2) Authorities should increase the budget allocated to genetic counselling and sending tests abroad. Earmarking a bigger budget for genetics is a question of democracy and patients' equal rights to a diagnosis.

(3) Raising awareness among primary care physicians and including rare diseases in the medical curriculum is crucial. Stories of patients' misdiagnosis should be used when raising students' awareness. Medical schools should be reached this way.

(4) In order to reduce the waiting lists for genetic counselling, consider:
    - Making it an option for genetic counselling to be carried out by "genetic counsellors" as well as doctors.
    - Better screening the reasons for seeking genetic counselling in advance.

(5) Identifying the rare diseases concerned via neonatal tests (e.g. cystic fibrosis) and starting a dialogue with the Communities, which are competent in the field.

(6) The importance of integrating European codification standards within the central registry of rare diseases (ICD code, Orpha code).

CARE FOR PATIENTS LIVING WITH RARE DISEASES

This part of the Plan for Rare Diseases includes recognizing the functions and establishing networks for rare diseases. These two measures are prerequisites for the development of new reference centres and centres of expertise.

Nevertheless, no federal funding is expected for these measures until mid-2018.

When the Plan was adopted in December 2013, the relevant areas of competence for Healthcare were the Federal government's responsibility. In the meantime, the Sixth State Reform has devolved some of these powers, which means that the Plan for Rare Diseases is now implemented differently in the North compared to the South of Belgium.

This State Reform gave federated entities the power to define standards for recognising centres of expertise while the federal level still has the power to designate such centres.

There have also been further reforms since 2013, such as the reform of hospital organisations, which also had an impact on the implementation of the Plan for Rare Diseases, and particularly on measures regarding patient follow-up.

With regard to patient follow-up and monitoring, Measure 20 of the Plan for Rare Diseases aims to introduce a specific team within SPF Public Health. This same measure also provides for a Federal Steering Committee whose mission is to oversee the Plan's implementation. This Steering Committee would meet regularly to monitor the implementation of the plan and to adapt it, if necessary.

In terms of implementation, SPF Public Health was made responsible for the regulatory aspects related to the creation of rare disease functions and networks by publishing the necessary Royal Decrees in 2014. Thereafter, in June 2016, functions were recognized by federal entities. Rare disease networks should now be established.

In this regard, it seems that the Flemish Region is moving ahead. Since the Royal Decree regarding functions was published, Flemish authorities supported Flemish University Hospitals so that they could get this recognition. From the outset, the four university hospitals considered setting up rare disease networks. The first five networks (around five rare diseases/groups of rare disease) were launched on 9 October 2017. The first 5 rare disease groups covered were metabolic diseases, neuromuscular diseases, those suffering from multi-systemic and cardio-vascular diseases, musculoskeletal and connective tissue diseases, bone diseases. In order to coordinate their work, an organization was created (VNZZ- *Vlaamse Netwerk voor Zeldzame Ziekten*) uniting all four university hospitals (Leuven, Ghent, Antwerp, UZ Brussels) Zorgnet-Icuro (an umbrella organisation of healthcare institutions, primarily of general hospitals) and Domus Medica (an umbrella organisation of general practitioners), VPP (*Vlaams Patiëntenplatform,* the Flemish patient advocacy platform) and Radiorg as "advisory" partners. Although these are all Flemish networks, they will establish collaborations with other networks that will be developed in the other regions of the country.

Unlike Flanders, the francophone hospitals were not supported by their authorities. All the hospitals that have a genetics centre tried to get a rare disease function recognised. The three university

hospitals with a genetics centre obtained recognition. Currently, St Luc (UCL), Erasme (ULB) and CHU Sart-Tilman (ULG) are looking to create a network among themselves including IPG, the Institute of Pathology and Genetics, which is a genetics centre that does not have a rare disease function, primary carers and patient advocacy organizations. The aim is also to develop strong relationships with Flemish networks.

For example, the notion of a network is not clearly defined within the Royal Decree, allowing for interpretations to vary between the regions.

There are at least three levels of networks to consider: (1) a network of university hospitals, (2) a network of university hospitals and other surrounding hospitals and finally, (3) a network at European level (ERN)! All these levels of networking are important. The Royal Decree does not provide all of the details, which leads to some uncertainty, however it also means that the implementation can be more made-to-measure.

Regarding patient follow-up, it is important to establish links with the central registry of rare diseases. The data in the registry will not be immediately available to the patient, but they will be for care providers. These care providers should also enter the data in the registry. For this to be a success, the registry needs to be simple, quick to implement and capable of gathering interesting and useful data.

For the patients and their representatives, the progress made so far has not made any tangible difference. Patients require multidisciplinary care, which above all, should factor in their transition into adulthood. Getting the functions recognised and establishing networks are both positive steps in the right direction, however, this needs to translate into real care for patients via centres of expertise.

For patients, centres need to be centred around real rare disease expertise and supplies. The mapping of this expertise at a Belgian level was initiated by the Institute of Public Health a few years ago but this work was abandoned last summer.

RECOMMENDATIONS

(7) To reinforce recommendation No. (1), regarding the urgent need for financing the rare disease function coordinators, we will use some examples of the important counselling role they play during the whole duration of the disease.

(8) The rare disease network coordinators should also receive funding as quickly as possible, since they need to organise network activities and get involved in the international networks.

(9) All nine of the Belgian Ministers responsible for public health (Federal, Regional and Community) need to set up a task force dedicated to rare diseases, which can raise topics at the Inter-Ministerial Conference on Social Security. Furthermore, it is important that the rare disease functions get support from their regional authorities.

(10) Patients suffering from rare diseases, their representative and all stakeholders should regularly receive information from the competent authorities regarding the progress made in the implementation of the Belgian National Plan for Rare Diseases. This information should come from the Federal Steering Committee for Rare Diseases.

(11) The mapping of rare disease expertise should be started up again.

(12) Regarding the transition into adulthood, this issue should be tackled by implementing centres of expertise on rare diseases.

(13) The concept of “networks” should be clearly defined, in order to ensure that the implementation of rare disease networks in both regions is harmonised.

(14) In 1/10/2017, reference pharmacists were introduced in Belgium. It is important to take this into account when building up networks with primary care.

(15) Ensuring that very rare diseases will be well taken care of when creating a network of rare diseases.

ORPHAN MEDICINAL PRODUCTS AND MEDICAL NEEDS THAT ARE NOT COVERED

Orphan drug development makes it possible to cover medical needs that are not covered.

For 17 years, we have seen the number of orphan medicinal products increase significantly. European regulations played an important role in the drugs' development, however, they would have been pointless without innovations from the pharmaceutical industry.

The real issue is... HOW DO WE GIVE PATIENTS ACCESS TO THESE INNOVATIONS? This means prioritizing the innovation that meets patients' needs. To ensure this, a specific task force was established by NIHDI by applying measure 15 of the Plan "Inventory of needs that are not being covered". Unfortunately, patient representatives are disappointed as its initial ambitions have not been met.

There are various ways to speed up the process to access innovative treatments: taking part in clinical trials, benefiting from the "compassionate use" programme, seeking solidarity fund aid or the ETR (Early Temporary Reimbursement) programme. This ETR programme was implemented by measure 14 of the Plan for Rare Diseases.

Measure 14 of the Plan for Rare Diseases concerns Unmet Medical Needs (UMN). The aim is to improve access to innovative medicines by speeding up treatments through *faster reimbursement by health insurance specialised in pharmaceuticals establishing innovative treatments for serious or fatal pathologies for which there are no therapeutic alternatives, and this is prior to being registered on a European level for specialisations that are not yet authorized, or before accepting new information for which a medical need is not covered for specialisations which are already authorised.*"

This ETR programme was launched in 2014, in collaboration with representative of the pharmaceutical industry.

After two years, it could only be considered a failure as no requests had come in. One of the main reasons was fear, in the industry, that the price set by the UMN programme would become a point of reference during the regular reimbursements procedure for the same treatment. To overcome this, the flat rate principle was introduced, which triggered two requests. These are now being reviewed by a colleague of NIHDI's senior doctors.

Access to medication in general and to orphan medicinal products in particular is determined by the reimbursement procedure at the level of NIHDI (*Commission de Remboursement de Médicaments* - Committee for Drug Reimbursements CRM) including the procedure of article 81. This article requires a contract to be signed by the company and the public authorities. The number of such contracts being signed is increasing rapidly in Belgium. According to the industry, in recent years, hardly any new innovative drugs can be sold in Belgium market without such contracts. However, also according to the industry, these contracts have meant that Belgian patients have access to new medication. Nevertheless, the drawback of this method is that the terms of said contracts are not disclosed.

The issue surrounding patient's access to innovative treatment is tightly linked to the States ability to pay the reimbursements, which could amount to hundreds of thousands of euros per patient.

Given what is at stake, this problem cannot be solved at a national level. Taking a supranational approach to the matter is utterly essential. This issue should be handled at a European level, if not at a global level.

At this scale, we need innovation so that we can take on the costs related to drugs and innovative treatments for patients with rare diseases.

We need to find solutions that change the system. This will consist of fundamental changes in the way research is considered, partnerships between the public/universities/pharmaceutical industry, justifying prices and transparency. It is a big task.

RECOMMENDATIONS

(16) Once the centres of expertise have been established, the College for Orphan Drugs should no longer be required.
(17) Care providers should know all of the options so that they can give their patients access to innovative drugs, especially through solidarity funds.
(18) Belgium should demand that the EU Commission set up an agency which evaluates the reimbursement levels in member countries for treatments granted marketing authorisation by the EMA.
(19) Reimbursements for orphan medicinal products should be based on therapeutic results.

ANNEXE 1

PROGRAMME OF THE BELGIUM EUROPLAN ROUND TABLE in the framework of the EU Joint Action RD-ACTION

6 October 2017, Brussels

DIAGNOSTICS	***Introduction*** - Elfriede Swinnen, Scientific Institute of Public Health - Vincent Bours, University hospital of Liège ***Panel:*** - Elfriede Swinnen, Scientific Institute of Public Health - Vincent Bours, ULG, University hospital of Liège - Saskia Van den Bogaert, Health federal public administration - Luc Matthysen, HTAP Belgium (patients' association)
CARE FOR PATIENTS LIVING WITH RARE DISEASES	***Introduction*** - Saskia Van den Bogaert, Health federal public administration - Marie-Françoise Vincent, University hospital of Louvain-la-neuve - Geert Peuskens, Health flemish (regional) public administration ***Panel:*** - Saskia Van den Bogaert, Health federal public administration - Marie-Françoise Vincent, University hospital of Louvain-la-neuve - Geert Peuskens, Health flemish (regional) public administration - Georges Casimir, University hospital Brussels - Eva Schoeters, be-TSC (patients' association)
ORPHAN MEDICINAL PRODUCTS AND UNMET MEDICAL NEEDS	***Introduction*** - Chris Van Hul, Private health insurance ***Panel:*** - Jean-Jacques Cassiman, Rare diseases and orphan drugs Foundation - Chris Van Hul, Private health insurance - Herman Van Eeckout, Pharma.be - André Lhoir, Federal agency for medicines and health products

ANNEXE 2

LIST OF PARTICIPANTS

First name	Name	Organisation
Vincent	Bours	ULG
Georges	Casimir	HUDERF
Jean-Jacques	Cassiman	FMMO
Marilyn	Coppenrath	Pharma.be
Olivier	Costa	Registre Belge du Diabète
Marc	Dooms	FMMO
Fanny	Duysens	Doctorante Sc. Pol. et Soc.
Yves	Gillerot	Rare disorders belgium
Caroline	Glaude	Projet EMRADI
Sophie	Ingels	LUSS
Ingrid	Jageneau	Debra belgium/Radiorg
Stefan	Joris	Muco association belgium
André	Lhoir	AFMPS
Fabienne	Lohest	UCL
Joyce	Loridan	Projet EMRADI
Geert	Matthijs	KUL
Luc	Matthyssen	HTPA
Dominik	Michiels	UZ Leuven
Anne-Marie	Morrin	KULeuven
Geert	Mortier	UA
Clara	Noirhomme	Projet EMRADI
Geert	Peuskens	Zorg en Gezondheid
Katlijn	Sanctorum	Familiale Adenomateuze Polypose (FAP) & Lynch syndroom (HNPCC)
Eva	Schoeters	be-TSC vsw
Claude	Sterckx	Radiorg
Elfriede	Swinnen	ISP - WIV
Saskia	Van den Bogaert	SPF/FOD
Herman	Van Eeckhout	Pharma.be
Chris	Van Haecht	Mutualité Chrétienne
Chris	Van Hul	MLOZ
Dieter	Vandermeersch	Pharma.be
Nathalie	Vandevelde	ISP - WIV
Jonathan	Ventura	Radiorg
Marie-Françoise	Vincent	UCL
Ilse	Weets	UZ Brussel