LUXEMBOURG

EUROPLAN NATIONAL CONFERENCE

in the framework of the EU Joint Action RD-ACTION

Mamer 8 November 2017

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FOREWORD

The EUROPLAN national conferences or workshops 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:

1. **Patient-led**: National Alliances are in the best position to address patients' needs;
2. **Multi-stakeholders**: National Alliances ensure to invite all stakeholders involved for a broad debate;
3. **Integrating both the national and European approach to rare disease policy;**
4. **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;
5. **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	Luxembourg
National Alliance (Organiser)	ALAN - Maladies Rares Luxembourg
Date & place of the national workshop/conference	8 November 2017 Mamerschlass – Mamer Luxembourg
Website	www.alan.lu
Members of the Steering Committee	Gwennaëlle Crohin, Social Worker - ALAN Catherine Gérardin, Conference Coordinator Dr. Jos Even, Vice President ALAN Shirley Feider, President ALAN Dr. Francesca Poloni, Coordinator Rare Disease National Plan–Ministry of Health Marc Ries, Director ALAN
List of Themes addressed	National Plan for Rare Diseases: progress report Testimony: rare disease and social inclusion INNOVcare social care European Reference Networks The European context, national plans, projects
Annexes :	I. Programme II. List of Participants (by stakeholders' categories)

I. INTRODUCTION

The second EUROPLAN Conference in Luxembourg was organised by ALAN, under the auspices of the Ministry of Health, the Ministry of Social Security and the Ministry of Family and Integration. There were 81 participants including representatives of all ministries and national institutions working in the field of rare diseases, medical professionals and organisations, laboratories, social organisations and patients' associations.

Shirley Feider, President of ALAN, welcomed and thanked all participants and briefly reviewed the history of rare diseases recognition in Luxembourg, starting in 2005 with the creation of the working Group MARA and the first EUROPLAN conference in 2013.

The President of ALAN underlined that Luxembourg was lagging behind in the development of its National Plan for Rare Diseases but was, however, pleased to report that things were now moving forward and progressing well since the beginning of 2017. She recalled that the National Plan targets the five main objectives developed during the first Rare Disease National Conference in 2013, i.e. Health care system supporting people with RD - Creation of a rare disease platform - Codification/Creation of a registry/Research - Social services for people with rare diseases - and, as transversal theme: Methodology and Governance.

The President expressed her satisfaction at the progress made in the course of the last months, pointing out that any interested stakeholder could still join this process. She took the opportunity to state her wish that the National Plan and the corresponding financial framework should be adopted during the present legislative period.

Shirley Feider then presented the program and the main themes to be discussed during the conference

1. National Plan for Rare Diseases: progress report and perspectives
2. INNOVcare-Social care: achievements and challenges in Luxembourg
3. European framework and European Reference Networks- the Luxemburgish experience of ERNs

The Minister of Social Security acknowledged the work accomplished towards the definition of a rare disease strategy and plan for Luxembourg. The Minister explained that since 2013, the government had had to establish priorities and, as a result, the National Plan project had been delayed. He recalled that one of the mottos of the government was "people's well-being" and that this motto applied to all persons facing health problems, namely rare diseases. He reaffirmed the involvement of the Ministry of Social Security and also expressed the will for the National Plan and financial framework to be adopted during this legislative period.

The patients had a voice in the introduction session. Denis Garzaro, 46 years old, President of the Marfan Patients Association and affected by Marfan syndrome, shared his experience. Despite his health condition, he could return to work. However, he highlighted the prejudices, administrative obstacles (he had to appeal to the administrative court), delays and refusals he had to overcome in order to be heard. He underlined that Luxembourg had a generous social framework and declared that it was within this favourable context that the National Plan for Rare Diseases had to focus on helping the patients and their families.

II. THEMES

THEME I National Plan for Rare Diseases: progress report and perspectives

Dr Francesca Poloni, Coordinator National Plan for Rare Diseases within the Ministry of Health

During the last ten months, many national stakeholders were involved in setting up the National Plan for Rare Diseases which aims to cover the period 2018 - 2022. It is designed around the patients' needs and as one of the main challenges, it will have to address diagnostic wavering.

Four workshops were set up dealing with four main themes which were then subdivided into more specific objectives:

<u>Health care system supporting people with RD</u>: financial care/ reimbursement- orphan drugs - continuity of care – genetic council - care pathway - precautionary principle

<u>Creation of a rare disease platform</u>: collaboration with the European Reference Networks - network of resources – training on Rare Diseases – Web information platform - RD Hotline

<u>Codification – Creation of a registry - Research</u>: Rare diseases data gathering in Luxembourg – Rare diseases traceability improvement - data harmonisation on a European level – monitoring system – research

<u>Psycho-Social services</u>: socio-administrative rights – support services – psycho-social care – school integration – professional integration – social inclusion

The plan will address other complementary issues such as National Plan for Rare Diseases- Governance - Awareness raising policy – Uniting national associations – Multidisciplinary dynamics and dialogue.

The development of the plan is now in its final stage. The steering committee will need to review and validate the plan by the end of November 2017. It will then be submitted to the Minister of Health for approval, before being ratified by the Government council. Since the Government council will meet, for the last time this year, in mid-December, the plan will be submitted for approval early February.

THEME 2 INNOVcare-Social care

Raquel Castro (EURORDIS), Gwennaëlle Crohin and Stéphanie Magar (ALAN) Dr. Marguerite Leches (Centre Hospitalier de Luxembourg)

Through its survey initiative "Rare Barometer Voices", EURORDIS carried out the first European- wide survey "juggling care and daily life", on the impact of rare diseases in everyday life. The survey carried out in 2016 and 2017 with the participation of patients and helpers, shows that people in Luxembourg face the same challenges as in other countries. The pilot experience conducted in Romania within the framework of INNOVcare project, proves the benefits of concentrating resources in one centre and thus enhancing a better coordination. The role of case managers is essential in the patients' health pathway.

There are many similarities with what ALAN does in Luxembourg: social workers and psychologists act as case managers "référentes parcours santé", providing support to patients, all along their health pathway and helping them to live with their conditions and create their personal life plan.

The European recommendations aiming at integrating rare diseases within national policies and social services are partly implemented by ALAN and other patient associations, through the work developed in the field, as case managers, trainers, promoters and innovators.

ALAN professionals notice there has been an improvement since the last EUROPLAN conference, in the way social services cooperate, even if there have been no changes so far at legislative and political levels.

ALAN social worker Gwennaëlle Crohin and psychologist, Stephanie Magar, both in charge of ALAN's Socio-Therapeutic Consultation Service, explained their achievements and the positive impacts created, not only due to the support provided to the patients and their families along their health pathway, but also due to the fact that their approach has helped patients to regain self-confidence, thereby allowing them to play a more active role in their daily life.

Contacts and communication with the school system are considered satisfying.

The recurrent problem for patients and their families lies in the search for diagnosis and treatment. ALAN aims at intervening as early as possible, in order to define the best pathway and prevent educational, professional and social disruption.

* « Rare Barometer Voices » is a community of people living with a rare disease who are willing to participate in EURORDIS surveys and studies

However, there are still many obstacles to be dealt with

The multidisciplinary cooperation needs to be enhanced. It is difficult to identify the contact persons and experts on a national and international level. Patients and helpers are faced with a large variety and a great number of professionals (health and care providers) and there is little or no coordination between these.

Patients' associations are not represented in the government services where the decisions regarding the patient's future (professional, educational orientation…) are taken. These services do not have enough knowledge of the particular situation of RD patients.

The administrative and financial impact on patients and their families is very heavy. Some specific treatments are not covered by the social security system.

Healthcare professionals (e.g. general practitioners), social workers and psychologists do not have an in-depth knowledge of Rare Diseases and they very seldom contact the professionals who have it. There is a need to provide information to those involved and to train them (continuous training program).

Not all patients benefit from support, some giving up after having tried without success, to obtain support. Social equity must be promoted.

In the last few years, ALAN has registered a considerable increase in requests. Today, a new patient or family requiring ALAN support will have to wait for up to one year before receiving it. Furthermore, increased life expectancy and emerging medical advances generates new expectancies and needs in terms of social care. Considering that the support needed implies time, ALAN has already exhausted its capacity; therefore its resources are not sufficient to cope with the growing demand. Additional staff and appropriate premises are necessary.

Dr. Leches explained that even though social support in Luxembourg can be generous, there are several gaps in the health system when it comes to Rare Diseases. There is neither a national registry nor a Rare Diseases inventory. Most Rare Diseases do not possess a code in the national health system that uses ICD 10 *. Coordination is poor. There is a lack of knowledge and training in the area of rare diseases. When it comes to making a diagnosis, doctors have to battle in order to refer the patients to reference centers, send them abroad, have a genetic test performed within a reasonable delay and obtain reimbursement of healthcare treatment costs. The follow up is a challenge.

Doctors are faced with a dilemma: taking charge of patients with a Rare Disease and their families requires more time compared to other types of patients. The psychological load associated with the complexity of the tasks, the time constraints and the families' distress are significant. Furthermore, the communication with patients, sometimes speaking a different language, is an arduous task; it is worth noting that nearly 48% of the Luxemburgish population have a foreign nationality. For a general practitioner or specialist in private practice, these issues need to be considered.

Patients and families need to find an answer to these problems. During the question and answer session, they pointed out that it will be necessary to define a legislative and administrative framework. They concluded stating that nothing could be achieved without means and financial resources.

* International Statistical Classification of Diseases and Related Health Problems (medical classification list by the World Health Organization)

THEME 3 EUROPEAN REFERENCE NETWORKS (ERNs)

Lenja Wiehe (EURORDIS), Dr. Michael Witsch (CHL), Dr. Françoise Berthet (Ministère de la Santé), Dr. Jos Even (ALAN)

ERNs represent a unique opportunity for Luxembourg since there is neither a sufficient critical mass of patients nor scientific expertise in the country to handle the rare diseases issue. ERNs provide a powerful answer to diagnostic wavering and inadequate care. Access to a network of expertise and structured healthcare pathways will help to reduce delays, prevent misdiagnosis and inadequate care. Patients will not have to travel abroad, as it is the case today, to look for help. They will also reduce inequalities for the people concerned (diagnosis, treatment and follow-up) and contribute to fulfil the national objective of "quality of life and quality of care". Furthermore, within the development and implementation of the National Plan for Rare Diseases, ERNs encourage concertation and cooperation.

Luxembourg through the Diabetes Endocrinology Care Clinique Pédiatrique, is a full member of ENDOcare ERN. This clinic has been endorsed by the Ministry of Health in May 2016. It is also an active member of "Sweet": an International network for paediatric diabetes centres created in 2008. "Sweet" provides its members with a data base on rare forms of diabetes. The role of the clinic as an ERN member and the care provided by the clinic to its patients are enriched by this double membership.

Dr Witsch, Luxembourg representative in ENDOcare ERN, pointed out that belonging to a ERN offers many advantages, namely access to experts in almost all pathologies, exchange of information, work with other teams, access to genetic testing, participation in research projects, possibility to develop contacts between patients themselves.

The cooperation with experts in other countries, some using totally different approaches and others with more limited resources, can give rise to changes in the practice of health professionals and enrich the quality of care.

However, belonging to a ERN is demanding for the members who have to provide information, take part in meetings, write reports, visit other healthcare providers, participate in assessments etc. It means a lot of work for specialists who already have many responsibilities and time constraints. This is something which will have to be addressed sooner or later.

ERNs raise the questions of reimbursement within the framework of cross border healthcare. A minority of patients is well informed. As it was already mentioned, there are gaps in the health system when it comes to problems raised by rare diseases' treatments.

At national level, Luxembourg has a representative in ERN board of member States: Dr. Françoise Berthet, in charge of curative medicine for the Ministry of Health. Dr. Berthet explained that the integration of ERNs in the national health system will be based on a structured organisation under the principles of solidarity and equity. Some tools are already in place: referring doctor, shared health record, health costs reimbursement procedures, existing structures (hospitals, national health institutions, competence networks), quality approach and certification. As in all small European countries, it will be difficult to have full members and associated partners of the 24 different ERNs. Therefore the Ministry of Health proposes to set up a National Hub complying with the requirements for ERN affiliated partners. The patients and health care providers could have access to the ERNs through this hub. This model could evolve and develop into National Associated Centres, meeting the criteria applicable to the ERN they would be part of. The "Rare Disease House" integrated in the National Plan could host this hub.

Over the last decades, as Antoni Montserrat, Senior Expert on Cancer and Rare Diseases from DG Santé, explained in detail, the European Commission has developed an extremely structured and powerful framework to support its Member States and provide them with various tools aiming at integrating rare diseases in the national policies and improving patient care and welfare. ERNs are one of these tools. Luxembourg, as a small European country, has also a lot to gain by having access to this framework.

III. DIRECTOR OF HEALTH SPEECH

Dr Jean Claude Schmit, Director of Health, acknowledged the excellent work carried out by the National Plan for Rare Diseases teams with the coordination of Francesca Poloni. The Director of Health explained that the budget estimates for the national plan are significant and cover the next five years. They will be used to finance the implementation of the National Plan for Rare Diseases and its coordination. If the government council gives it a strong support, additional resources could be allocated in the following years. He also pointed out that the social security system needs to be improved, for example the "fee for service" principle has to be revised. The issue of orphan drugs is also a serious preoccupation. There is no legal basis for the use of orphan drugs (off label, compassionate use, medical need…). A European regulation mentions that the countries can have special legislation in this area but this regulation was never put in place in Luxembourg. Today, the ”procedure” is based on a kind of “gentleman’s agreement”, meaning that the reimbursement of costs approved by the Health Division of the Ministry of Health is usually accepted. A law aiming at giving some clear rules will be submitted to the government council in December. The government is supportive. The Ministry of Health is highly involved in the development of the National Plan for Rare Diseases and will ensure its implementation within a clear financial framework.

Dr. Jos Even concluded the conference. He stressed the importance of EUROPLAN conferences for the recognition of rare diseases and their integration in national policies. As Vice President of ALAN and also President of the steering committee for the National Plan for Rare diseases, he thanked all the participants for their devoted commitment.

IV. CONCLUSIONS

In Luxembourg, people living with a rare disease and their families can benefit from a range of services. However, not all the patients have access to support or receive adequate information. This may be due to a lack of means and sustainable human resources on the part of healthcare providers and psycho-social services. Furthermore, taking care of patients as soon as possible in the context of a coordinated, multidisciplinary and holistic approach remains a challenge.

Coordination between all the professionals and institutions needs to be improved as well as the knowledge and skills in the RD domain. Awareness campaigns, focused information, training and communication tools should therefore be developed. Patients’ empowerment needs also to be improved through information on treatments, drugs, support, and structures.

The European Reference Networks provide answers to some of the problems raised by the professionals, namely diagnostic wavering, inadequate care and unnecessary constraints and costs. The benefits are numerous, for the patients who have access to the best of expertise without having to travel abroad to specialized centres, for the health care providers who gain invaluable support (medical, research) and who can enrich their professional practice, for the social workers and for the social security system, thanks to the avoidance of unnecessary costs and procedures. In order to fully benefit from these networks, the Ministry of Health will first set up a specific structure (Hub) complying with the requirements for ERN affiliated partners and mobilize the professionals. Luxembourg being a small European country can benefit from this structure by having access not only to ERNs but to the Rare Diseases European framework.

The involvement of medical professionals is essential for the success of the plan. How to help these professionals maintain their commitment to the Plan and its implementation, knowing that it will imply extra workload and a change of habits, is a cause for concern.

The National Plan for Rare Diseases includes the European recommendations. The main objectives presented in p. 5 will be subdivided into concrete actions during the next phase, transforming the implementation of the recommendations into a national reality. It is the closest Luxembourg has ever been to an effective recognition of rare diseases for all stakeholders. However, in order to give this National Plan the best chance of success, some fundamental issues still need to be addressed. They concern resources, legal arrangements and administrative framework.

ENCLOSURES

ALAN
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PROGRAMME

CONFERENCE EUROPLAN – 8 NOVEMBER 2017 – MAMERSCHLASS - MAMER - LUXEMBOURG

13h30	**Welcome**
14h00	**Introduction speech** ALAN President, *Shirley Feider*
14h10	**Opening speech** Minister of Social Security, *Romain Schneider*
14h20	**Testimony, President of Den-I (Marfan Syndrom)** -*Denis Garzaro*
	«Back to work despite the "rarity" of my condition! How perseverance and combined efforts can lead to better social integration »
14h30	**Rare Diseases National Plan in Luxembourg – status report**
	Francesca Poloni – Coordinator National Plan for Rare Diseases, Ministry of Health
14h50	**Round Table 1** **INNOVcare – Social Care** –
	Raquel Castro (EURORDIS), Gwennaëlle Crohin and Stéphanie Magar (ALAN), Dr. Marguerite Leches (Centre Hospitalier de Luxembourg)
	Introduction - Presentation of results; strengths and areas for improvements - future challenges. Q&A
16h00	*Break*
16h15	**European framework –** National plans and European joint actions, projects and other initiatives *Antoni Montserrat (DG Santé, EC)*
16h30	**Round table 2** **European Reference Networks**
	Lenja Wiehe (EURORDIS), Dr. Michael Witsch (Centre Hospitalier de Luxembourg), Dr. Françoise Berthet (Ministry of Health), Dr. Jos Even (ALAN)
	Introduction - Presentation of a concrete example; strengths and points for improvements - future challenges. Q&A
17h30	**Wrap-up speech,** Director of Health, *Dr. Jean-Claude Schmit (Ministry of Health)*
17h45	**Closing speech,** *Dr. Jos Even – ALAN Vice President*
18h00	**Cocktail – networking – display of books**

LIST OF PARTICIPANTS BY STAKEHOLDERS' CATEGORIES

ALAN - Maladies Rares Luxembourg **Patient Associations**	**28** **12**
Ministry of Health, Ministry of Social Security and public institutions	**16**
Health care providers	**10**
Laboratories and pharma	**6**
Research Centres	**3**
EURORDIS	**2**
European Commission, DG Santé	**1**
Others	**3**
Total	**81**