



Slovak republic

EUROPLAN NATIONAL CONFERENCE

in the framework of the EU Joint Action RD-ACTION

Bratislava, 25 May 2018

FINAL REPORT



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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:

- **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	Slovak Republic
National Alliance (Organiser)	Slovak Alliance for rare diseases
Date & place of the national workshop/conference	25.5.2018 Hotel Crowne Plaza, Bratislava
Website	http://www.zriedkave-choroby.sk/page/58 http://sazch.sk/crowne-plaza-bratislava-25-5-2018/
Members of the Steering Committee	MUDr. František Cisarík, CSc., PharmDr. Tatiana Foltánová, PhD., Ing. Beáta Ramljaková, MUDr. Katarína Štepánková
List of Themes addressed	National list of the centers of expertise European reference networks Registries Cooperation with patients' organisations and National Alliance
Annexes :	I. List of Participants (by stakeholders' categories) II. Programme in English

FINAL REPORT

I. Introduction/ Plenary session

- Welcome and short introduction of the speakers as well as the main idea of the conference thus to introduce the National list of centers of expertise, to support their further cooperation and motivate them for possible involvement in the European Reference Networks. PharmDr. Tatiana Foltanova, PhD, introduced the work of the Slovak National Alliance, its big achievements as well as challenges and further cross connections in the field of rare diseases in Europe as well as in Slovakia. She called for stronger cooperation between health care providers and the National Alliance and introduced also current projects to support cooperation. Precisely, the initiator was the National Alliance representing currently 20 patient's organizations – the Slovak voice of patients with rare diseases. In line with this, further issues were addressed as there is no support from the State for patients' organizations. Out of the discussion came also the point of the importance of creation and sustainability of independent patients' organizations. One of the suggestions was to introduce public programs, which would support patients' organizations on clearly defined criteria as integration in national and European associations from the field, at least 5 years of experience, experience with European grants and funding options etc. The audience were all stakeholders, mainly representatives of the centers of expertise, as well as payers and some patients.

II. Themes

THEME 1 National list of centers of expertise

- **Identification of specific gaps, challenges and needs:** At the end of the year 2017 the committee for rare diseases at the Ministry of Health (MoH) finished the evaluation of Centres of Expertise (CoE). Together 86 applied, out of them 59 were accepted. Amongst these 59 CoEs, 17 are at higher level and have the potential to be involved in the European Reference Networks. Representatives of all Centres were invited for this conference, to support further dialogue about their needs, expectations as well as to provide them with more information about the European concept of Centres of Expertise for rare diseases. Nevertheless, it is important to motivate and support them for involvement at the European level in European medical societies as for example the European Hematology Association (EHA). It is needed to look for further utilization of the European or international cooperation in the field of rare diseases. High standards and guidelines at the European level will be translated also into rare diseases field and future European Reference Networks. The healthcare professionals appreciated deeper involvement at European level and further cooperation with their European societies.
- **Further issues were recognised in the discussion as the need of more involvement of CoE in clinical trials.** It is in interest of MoH as well of the CoE and patients to support high quality centers and benefit from clinical research. Health care professionals together with patients need improvement of the current conditions to support staff, material, technical and financial conditions for further development of CoE to be competitive in the field of European clinical research.
- Health care professionals expressed personnel limitations, the lack of physicians and other healthcare professionals (nurses etc...) are most limiting obstacles for them to be more involved in the rare disease field. They expressed interest to formally declare the quality and quantity of care for rare disease patients. They would welcome the possibilities to have fixed hours per month allocated for rare disease patients. The experience of cystic fibrosis in Slovakia is in favour of strict requirement defined in law which clearly defines the amount of hours/month for healthcare professionals, which they dedicate to rare disease patients in the CoE. A recommendation alone isn't sufficient. Further issues were the working conditions and technical equipment of the CoE.
- The healthcare professionals are expecting the MoH to develop further programs to increase the interest in rare diseases among the medical students and young graduates and improve the conditions for further work at the national as well as international level.
- Some health care providers also expressed interest to be involved in the National list of Centers of Expertise. MoH together with the working group for rare diseases is planning a next call for the extension of this List.

THEME 2 Registries

Identification of specific gaps, challenges and needs:

High quality epidemiologic data about rare disease patients was set as further priority of the healthcare system. In Slovakia the information about rare disease patients is very scarce. Although, thanks to the National Action Plan, the National Registry of Rare Diseases was established in 2014. However, it still doesn't have sufficient data which could be further used to predict quality and quantity of the high quality care of rare disease patients. The presentation of Geza Marshal together with the presentation of Frantisek Cisarik stressed the importance of simplicity and cooperation even at the European level. Healthcare professionals expressed mainly personnel needs for help on data gathering. The National Health Information Centre is ready to help. However, they can't be involved in every registry, they welcome basic data, which could help them to get information about prevalence and incidence of certain rare disease. They are opened to medical societies to consult their individual status.

Clinical genetics are supposed to provide data about all rare diseases and syndromes into the National Registry of Rare Diseases. Rare diseases are being reported legally by genetic specialists since 2014 to National Registry of Congenital Disorders at National Health Information Centre. The report on a rare disease is a part of this registry. Following the approval of List of Centres of Expertise dealing with diagnostics and treatment of corresponding RDs (in 2017), it becomes mandatory to report as many as possible patients with RDs (new encounters together with those identified in the past). The National Health Information Centre is aware that gaining retrospective personified information on persons with RD in the Slovak Republic (SR) is a challenge from the aspect of global prevalence, however SR has the ambition to outreach diagnosed cases in a most possible time span in frame of the period prevalence. For the issue of treatment, it would be of high importance to find approach how to link this data with the data of the payers. This would allow to follow patients with certain diagnosis on their medical treatment or dispatching medical devices. The cooperation with payers with this issue is in place. Out of the discussion came a suggestion, of clear financial support of the Centres of Expertise with the aim to provide exact numbers of patients with certain rare diseases in the Centres of Expertise. This could be performed as a grant of relatively small amount, for example 5000 euro for one year, which will be fixed on the outcome. The registry should include basic data about rare disease patients, who are treated in the centre. This could help with the most complicated work and thus starting the registration of rare disease patients at the Centre of Expertise. Orpha codes were mentioned in the discussion, however only clinical geneticists are familiar with them.

THEME 3 Reimbursement and access to treatment

Identification of specific gaps, challenges and needs:

At the beginning of the year 2018, a new law handling the reimbursement process came into force. After 7 years, the situation in the reimbursement process is changing. One of its main aims was to support innovative treatment and access to therapy. The willingness to pay has been increased from 31 000 euro/QALY to 37 000/QALY, based on the multicriterial approach. For the purpose of reimbursement, Slovakia still doesn't accept the European definition of orphan drugs. However, there are special reimbursement conditions for drugs which are intended for diagnosis and treatment of diseases which affect not more than 1/50 000 (subgroup of orphan drugs). This means that rare diseases which affect maximum 108 patients in Slovakia, aren't supposed to provide the pharmacoeconomic analysis. Finally, several tools are applicable, which allow to split the financial risk between the payer and marketing authorization holder. However, this amendment has also its limitations and the biggest is its imprecise budget impact analysis. The budget which was considered at the creation of this amendment, didn't really take into account the robustness of development in innovative treatments, nor the real prices of emerging innovative treatments. To solve this problem a detailed information about the tangible use of innovative treatments in Slovakia is needed. Thus precise analysis of the current use and its impact on healthcare expenditures are needed. This is in line with the previous topic of registries.

The representative of the biggest payers presented data about its expenditures for orphan medicinal products in previous years as well as prediction models for the upcoming years. The payers demonstrated their willingness to provide high quality healthcare for rare diseases patients. On the other hand, there are two other payers in Slovakia, private ones, who were not present. Their view of the situation could be different.

The healthcare providers from the Centres of Expertise expect better communication with the payers, thanks to the status of Centre of Expertise. They would appreciate simplification of the whole reimbursement of the treatment, mainly in cases when the treatment is given on exception, on patients named basis.

III. Conclusions

- *The national list of Centers of Expertise in the Slovak Republic includes 59 units, which provide care for rare disease patients. Out of them, 17 are at higher level and have the capacity to be involved in European Reference Networks.*
- *The MoH together with its working group for rare diseases is planning a new call for Centers of Expertise to extend the list.*
- *All Centers of Expertise call mainly for staff support, and declaration/definition of their work for rare disease patients in their contracts. Currently, it is mainly nowhere described and it seems to be a voluntary work.*
- *The involvement in European Reference Networks will be organized by the Ministry of Health. The deadline is expected in autumn 2018. The attendees had the opportunity to listen to the presentation about European Reference Networks from the Slovak representative on the Board of EU Member States on ERNs, Dr Monika Hurna.*
- *The Slovak Alliance for rare diseases will work on raising awareness of the National list of Centers of Expertise, not only among the patients and professionals but also the general public.*
- *Patients' organizations in Slovakia need state-driven support for the creation and sustainability of their activities, according to strictly defined criteria.*
- *The project of registries at the National Health Information Centre will be further supported, mainly in the part of interoperability and further cooperation with other registries of rare diseases to collect high quality data about the healthcare in rare diseases.*
- *Financial support (projects/grants) for the Centers of Expertise with regard to the issue of registries and their cooperation with the National Health Information Centre within the system is needed.*
- *New reimbursement policy came into force, just at the beginning of 2018. Despite its limitations, it is supportive for innovative treatments and orphan medicines. The issue of real cost of this innovativeness could be solved only in cooperation with all stakeholders and needs real life data of the expenditures. There is a strong will on the part of the payers supported by the National Alliance and working group of the Ministry of health to work on it and help produce this data.*

ANNEXE I : LIST OF PARTICIPANTS BY STAKEHOLDERS' CATEGORIES

Slovak National Alliance for Rare Diseases	4
Patient Organisations	5
EURORDIS – Rare Diseases Europe	1
Health care providers	27
Representatives of Public institutions	2
Payers	5
Industry	0
Total	44



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Aliancia
Zriedkavých
Chorôb



CENTERS OF EXPERTISE FOR RARE DISEASES: WHAT DO THEY BRING AND WHERE DO THEY GO?

9,00 - 10,00

Registration

10,00 – 10,15

Opening

MUDr. František Cisarík, CSc., Ing. Beáta Ramljaková, PharmDr. Tatiana Foltánová, PhD., MUDr. Katarína Štepánková

10,15 – 11,05

Slovak alliance for rare diseases – who we are, what we do and why is it important

PharmDr. Tatiana Foltánová, PhD., Ing. Beáta Ramljaková
UK in Bratislava, Faculty of Pharmacy, Department of Pharmacology and Toxicology, Slovak alliance for rare diseases

11,05 – 12,00

**Center of expertise for rare disease
How does it work, what does it add to the healthcare professionals, patients, payers?**

Dr Gabor Pogany, PhD
EURORDIS – Rare Diseases EUROPE
President of HUFERDIS, National Alliance for Rare Diseases of Hungary
Member of the former EU Committee of Experts on Rare Diseases – EUCERD

12,00 – 12,45

Centered care for patients with cystic fibrosis in Czech republic

Doc. MUDr. Pavel Dřevínek, CSc.
Charles University, Second Medical Faculty, Faculty Hospital Motol, Prague

12,45 - 13,45

Lunch



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13,45 - 14,35

Healthcare Insurance view on management of rare Disease

PhDr. Ľubica Hlinková, MPH

Všeobecná zdravotná poisťovňa, a.s., Bratislava

14,35 – 15,25

Working together - Examples of the cooperation among Healthcare professionals and patients organizations in Hungary

Geza Marsal

The Assotiation of Cystic Fibrosis Patients

15,25 – 15,40

COFFEE BREAK

15,40 – 16,30

Rare diseases in the health care system – the view of the Ministry of health working group for rare diseases

MUDr. František Cisarík, CSc.

Faculty Hospital Žilina, Department of Medical Genetics

16,30 – 16,45

Closing remarks

MUDr. František Cisarík, CSc., Ing. Beáta Ramljaková, PharmDr.Tatiana Foltánová, PhD., MUDr. Katarína Štepánková

Place of event: Crown Plaza Hotel, Hodžovo nám. 2

How to get there:

Bratislava main railway station:

Bus No. 93, get off at Hodžovo námestie (Hodžovo square) stop

Tram No. 1, get off at Poštová stop

Bratislava – Petržalka railway station: Bus No. 93, get off at Hodžovo námestie (Hodžovo square) stop

Bratislava Bus station: Bus No. 50 (to OD Slimak), get off at Svätoplukova stop, change here for trolleybus No. 208 or 212, get off at Hodžovo námestie (Hodžovo square) stop