



Slovak republic

EUROPLAN NATIONAL CONFERENCE

in the framework of the EU Joint Action RD-ACTION

Bratislava, 25/5/2018

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 Cisarik, 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. Programme in English II. List of Participants (by stakeholders' categories)

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 appealed on stronger cooperation between health care providers and 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 no support of 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. Out of them 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 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 European Hematologic Society. 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 profit from clinical research. Health care professionals together with patients need improvement of the current conditions to support personal, material – technical and financial conditions for further development of CoE to be concurrence capable in the field of European clinical research.
- Health care professionals expressed personal limitations, lack of physicians and other healthcare professionals are most limiting obstacles for them to be more involved in 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. The way of recommendation isn't successful. 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.
- Further health care provides expressed interest to be involved in the National list of Centers of expertise. MoH together with the working group for rare diseases is planning 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 personal 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 statuses.

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 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 and that's registry of basic data of rare diseases patients, who are in charge of the centre. This could help with the most complicated work and thus starting the register of rare disease patients at the Centre of expertise

THEME 3 Reimbursement and access to treatment

Identification of specific gaps, challenges and needs:

Beginning 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 was 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 pharmaco-economic 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 payer presented data about its expenditures for orphan medicinal products in previous years as well as prediction models for the upcoming years. They presented big 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 SR 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 network.*
- *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 personal support, and declaration/definition of their work for rare disease patients in their contracts. Now it is mainly nowhere described, 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 in the committee of ERNs Monika Hurna*
- *The Slovak alliance for rare diseases will work on the increase of the awareness about National list of Centers of Expertise, not only among the patients, professionals but also general public*
- *Patients organizations in Slovakia need state driven support for creation and sustainability of their activities, according 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 gather high quality data about the healthcare in rare diseases.*
- *Initial personal/financial support (projects/grants) of the Centers of expertise, in 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 beginning 2018. Although 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 of all stakeholders and needs real life data of the expenditures. There is big willingness of the payers supported by the National alliance and working group of the Ministry of health to work on it and help to produce this data.*