THE GOVERNMENT OF THE SLOVAK REPUBLIC

RESOLUTION OF THE GOVERNMENT OF THE SLOVAK REPUBLIC

No. 671

of 9 December 2015

On the National programme for the development of care for patients with Rare Diseases in the Slovak Republic for the period 2016-2020

Material number:	40897/2015
Submitter:	The Minister of Health

The Government

A. approves

A.1. The National programme for the development of care for patients with Rare Diseases in the Slovak Republic for the period 2016-2020;

B. actions

The Minister of Health

B.1. to ensure the implementation of the activities under the action plan, set out in Annex 1

according to the deadlines set out in the action plan

B.2. to submit to the Government an assessment report on the implementation of activities under the action plan for the previous calendar year

by 31 May 2017 and annually thereafter until 31 May 2021

B.3. to update the action plan in relation to the current volume of released funds for the practical implementation of the National programme in the respective two-year period

up to 31 December 2017

B.4. to submit the updated action plan to the Government for approval

up to 31 March 2018.

Task for: The Minister of Health

THE GOVERNMENT OF THE SLOVAK REPUBLIC

Proposal

The National programme for the development of care for patients with Rare Diseases in the Slovak Republic for the period 2016-2020

The National programme for the development of care for patients with Rare Diseases in the Slovak Republic for the period 2016-2020, (hereinafter as the "National programme") was drawn up by the Ministry of Health of the Slovak Republic Commission for Rare Diseases (Rare Diseases – RD) (hereinafter referred to as "the Commission") on the basis of task no. C.1. of the resolution of the Government of the Slovak Republic No. 578 of 24 October 2012, whereby the Government of the Slovak Republic approved the National strategy for the development of health care for patients with Rare Diseases for the years 2012 and 2013.

The National programme's content focus builds on the National strategy for the development of health care for patients with Rare Diseases for the years 2012 and 2013. Practical implementation of the National programme is submitted for the period 2016-2020. Part of the National programme is also the action plan, elaborated to the policies of the Health Ministry/ Department of Health by 9 key priorities (see Annex 1). The Departmental action plan will be updated on a biennial basis, in particular in relation to the current volume of released funds for the practical implementation of the National programme in the two-year period. It will be submitted to the Government of the Slovak Republic for approval.

Introduction

The existing European initiatives identified several key problems in the field of Rare Diseases (hereinafter "RD"):

⇒ lack of identification of the scope of the issues in very heterogeneous diseases,
⇒ lack of identification of RD within the International Classification of Diseases, version 10 (hereinafter as "ICD-10")
⇒ serious shortcomings in diagnosis and treatment due to extreme rarity, and incorrect or delayed diagnoses often lead to irreversible damage to the patient's health or even their death,
⇒ unevenness and the lack of quality of the services provided (specific needs for support and nursing care, as well as to specialized social services),
⇒ a very limited selection of drugs for the treatment of RD,
⇒ complexity of RD research, especially in the RD treatment research field,
⇒ deficiencies in data collection at National and regional levels,
⇒ lack of awareness about RD, among the professional as well as they lay public,
⇒ frequent shortcomings in the field of social care and legal aid because of the chronic and often progressive course of these diseases.

The elaboration of a National programme to contribute to a substantial improvement in the overall care for patients with RD.

Task priority no. 1

Develop public awareness about the topic of RD:

Awareness of the RD issue, among the professional as well as the lay public is generally inadequate. The dissemination of knowledge and information about RD is a prerequisite for developing the better care of patients with RD.

A major contributor to the development of care for patients with Rare Diseases are the initiatives of the Slovak Alliance of Rare Diseases (hereinafter "SAZCH") and cooperation with the specialized societies within the Slovak Medical Society. Practical supportive steps span the whole range of mass media communications: conferences, seminars, workshops, media campaigns, print materials, information materials, websites, international web resources (e.g.: Orphanet, OMIM, EUCERD, CEGRD, EURORDIS, EUROPLAN), significant commemorative events (e.g.: Rare Disease Day, National Conference on Rare Diseases).

Task priority no. 2

Expand the education of the professional public about RD:

⇒ **Further education**

Further education of healthcare staff, in particular in specialized medical fields: medical genetics, paediatrics, clinical oncology, neurology, ophthalmology, haematology and transfusiology, endocrinology, clinical immunology and allergology as well as rheumatology, aiming to draw attention to the issue of RD, bringing sufficient information and motivating activities for the benefit of patients with RD.

⇒ **RD themed Conferences**

Conferences, seminars, workshops, and international educational materials are an essential tool for updating knowledge, activities and awareness of RD issues. It is advisable to benefit from extensive international initiatives. Support for organizing a Slovak nationwide RD conference.

Task priority no. 3

Expand and enhance the diagnosis, treatment and care of patients with RD:

⇒ **Expansion of laboratory diagnostics**

Support for the application of the latest diagnostic methods, including the latest laboratory techniques in the diagnosis of RD, extending the options for information sources to include the availability of RD laboratory diagnostics in the Slovak Republic. In medical genetics the hot topic is extending the scope of molecular genetic diagnostics capabilities for genetic and hereditary diseases.

⇒ **RD in the DRG and e-Health systems**

Support to including RD as part of the development and use of DRG and the e-Health system, so as to also take account of the objectives of the National programme.

⇒ **Centralization, hierarchical organization of care, and support for the development of specialized RD centres**

For the development of health care for patients with RD an important role from the viewpoint of effectiveness is that of centralization, hierarchical organization, and support for the National specialized expert workplaces for RD in the Slovak Republic (in the EUCERD called 'RD centres of expertise'). In establishing the specialized departments for the National

RD centres of expertise in the Slovak Republic quality criteria will largely apply, based on European quality criteria. The network of National RD centres of expertise in the Slovak Republic should be linked to the European reference network in accordance with Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare (hereinafter the "EU directive").

⇒ **Professional guidance on the quality of healthcare**

For the subject area of RD, the criteria of quality in the provision of healthcare are paramount. It is imperative to develop further professional guidelines for the RD subject area and to encourage the systematic use of European standard diagnostic and therapeutic procedures.

⇒ **Funding of health care**

In cooperation with the health insurance companies is necessary to provide for funding health services essential for the diagnosis and treatment of patients with RD, as well as medical devices, while also taking into account the actual prevalence of RD.

⇒ **The EU directive and its impact on the area of RD, diagnosis and treatment of patients with RD**

The application of the EU directive aims to remove the barriers to availability of diagnostics and treatment for patients with RD. The implementation of the EU directive into the Slovak legislation and its practical use will allow the diagnosis and treatment of a wide range of RDs, which is provided for in specialized centres in the EU.

The availability of 'Orphan drug' RD medicines in the Slovak Republic is assured under applicable legislation (e.g. categorization of drugs, particularly imports). As of 2010 we see a significant decrease in the categorization of medicinal products with the Orphan status (2002-2010:26 as against 2011-2014:2). The direct availability of drugs is only one of the European indicators of availability of Orphan drugs for RD. The National programme should create scope for streamlining the process of access to Orphan drugs. We need to consider using the alternative options of pharmaco-economic analyses, as is the case in other European countries (e.g.: budget impact analysis or managed entry agreements or market access agreements, i.e. potential agreements with the payer for cost-sharing and/or the risks when introducing new pharmacological interventions), or even a separate budget head for the health insurance of these drugs.

⇒ **Additional technical fitting out of workplaces, funding of support for developing the workplaces**

The issue is primarily one of building up National specialized departments RD centres of expertise in the Slovak Republic and their linkage to the European reference network of RD specialized centres. Within the funding limits there is support for the introduction of new technologies and the necessary instrumentation.

Also important is the promotion and development of a selective screening of RD in the Slovak Republic, the promotion of science and research in the field of RD, development of international cooperation etc.

Task priority no. 4
Screening and Prevention of RD:

⇒ **Neonatal screening centre in the Slovak Republic**

The Neonatal screening centre in the Slovak Republic has lately attained such a high level of quality and efficiency as to be on a par with most developed countries.

⇒ **Selective screening for inherited metabolic disorders**

The extension and improvement of selective screening for inherited metabolic disorders is one of the priority activities of the National programme. Neonatal screening has been expanded to cover 9 metabolic diseases, but supplementary diagnostics and the subsequent medical treatment is not catered for in terms of resources, nor is instrumented relevantly provided. Additional requirements for high-quality selective screening are upcoming in the areas of: paediatrics, neurology, clinical genetics and other medical disciplines. Meanwhile this subject area is closely associated with advisory genetic testing. From a medical point of view, the complex of diagnostics is developing extremely quickly and the appropriate resource capacity needs to be catered for.

⇒ **Other errors and diseases screened in the new-born**

The Slovak Republic has a well-developed neonatal screening programme for hearing loss, the hip joints, the CNS, kidney and heart issues. Development of this area of screening and subsequent healthcare needs to take it further.

⇒ **Pre-conception and pre-natal diagnostics (new genetic methods, new diagnostic methods)**

The rapid developments in genetic testing methods with high resolution (e.g.: arrayCGH, karyotyping massive parallel sequencing, non-invasive prenatal genetic diagnostics, next generation sequencing) also find application in Slovak practice. We need to support the implementation of these methods, as well as other new diagnostic methods across the full range of RD issues. We also have to find and allocate financial resources to make use of these techniques in clinical practice. In this regard, ongoing communication between professional associations and the Ministry of Health of the Slovak Republic as well as the health insurance companies is very important.

⇒ **Technology retrofit, personnel requirements, funding**

Needs have been identified in particular for a technological refit of the National specialized RD centres of expertise in the Slovak Republic with commensurate technology and qualified personnel (e.g.: tandem mass spectrometry for selective screening in Slovakia, array karyotyping in cytogenetics, next generation sequencing in the diagnosis of monogenetic diseases, etc.).

Also of importance is the funding of the entire healthcare system organization, following on from neonatal screening in the Slovak Republic (specialized departments of RD clinical diagnostics + paediatrics + medical genetics + centres for diagnosing inherited metabolic disorders + clinical genetics laboratories).

Task priority no. 5
Support of science and research in the RD field

⇒ **Departmental research**

In the field of RD there is much interest in support for research, pure and applied, under the grant schemes at the Departmental level.

⇒ **Extra-Departmental research**

In the field of RD there is much interest in linking fundamental research with applied research in the RD subject area.

⇒ **International cooperation in research**

As part of the international cooperation framework in the field of RD research, it is foreseen that the Slovak Republic will engage in international projects, exchanges of experience, information (e.g.: ORPHANET, EURORDIS, ERNDIM, EUROCAT, etc.). The involvement of the Slovak Republic in international projects should allow easier access to the National grant projects and guarantee a systematic approach to the subject.

Task priority no. 6

Collection of data about patients with RD and the creation of an RD register:

⇒ **An RD Register, as a collection point for data from all relevant National Health Registers and their link to the virtual National register for RD and subsequent link to the e-Health project**

In the Slovak Republic there are no objective epidemiological data on the prevalence of RD. This is particularly to do with the complexity of verifying RD diagnoses, the availability of diagnostic procedures to determine RD diagnoses, as well as the low incidence of RD diagnoses within the ICD-10. In addition, the coordination of activities and the cooperation of relevant subject-matter specialists at the National level has been limited, in particular by favouring widespread or other chronic diseases to the detriment of ‘marginalized’ RD.

The National health information centre keeps several National health registers, out of which patients with RD can be identified. In the Slovak Republic there are also a number of specialized National registers only for certain RD types or RD groups, which are kept at specialized centres. To establish the prevalence of RD in the Slovak Republic we need to make use of current and new reported cases in the respective National registers, which are primarily a function of the level of reporting discipline. A key source of data is the National registry of patients with congenital developmental defects, which includes the reporting of inherited diseases, genetic defects, and RD.

At the same time, we need to reconcile and incorporate the requirements for creating the European register of RD. In this context, the RD register plays a vital role, in particular in the planning of development of healthcare in the RD area, related research and development of RD “Orphan drugs”, as well as in other activities aimed at improving the quality of life of patients with RD (education, social inclusion, etc.). Not least, when planning for RD health insurance costs, to base this on the true number of patients.

Task priority no. 7

Taking care of the quality of life and social inclusion of patients with RD (support and reinforcement of RD patient organizations in the Slovak Republic):

⇒ **International classification of functional capacities, disability and health**

In the healthcare and social care services (in terms of their linkage and sufficiency), there are many areas open to developing coordinated action. The timely question in this context is how the International classification of functional capacities, disability and healthcare is applied. Of benefit is the cooperation with the WHO Office in Slovakia and the Ministry of Labour, Social Affairs and Family of the Slovak Republic, which are active in this field. The international classification of functional capacities, disability and health helps to improve deliberations when classifying the impact of disability, in determining the extent of the impaired function, and scope to assess further career options, which is of particularly great importance for the inclusion of people with disabilities into an active social life.

⇒ **Support for the efforts of patient organizations in the field of RD**

It is important to promote the practical activity of patient organizations and systemically and the potential of the SAZCH, as well the encouraging the National Alliance, well-connected in Europe, to partake in Inter-Departmental and Inter-domain cooperation. The SAZCH representative is a member of the Commission, and via this person cooperation across the full range of RD issues can be coordinated.

It is particularly important to find within the grant projects the financial resources to support SAZCH cooperation, or of patient organizations with professional companies, National specialized RD centres of expertise in the Slovak Republic and RD clinical centres.

Task priority no. 8

Inter-Departmental cooperation in the RD subject area and the Departmental Commission:

⇒ **Tasks of the Departmental Commission**

The main tasks of the Commission are intended to support the implementation of the National strategy for the development of health care for patients with Rare Diseases for the years 2012 and 2013. The Commission shall also prepare the documentary basis for the Departmental action plan update.

⇒ **Inter-Departmental cooperation in the RD subject area**

The subject area of RD is most pertinent to the Department of Health. Its reach is however, much broader, hence the Ministry of Labour, Social Affairs and Family of the Slovak Republic and the Ministry of Education, Science, Research and Sport of the Slovak Republic expect to participate in the practical implementation of the National programme of the Slovak Republic.

The subject area of RD is linked to funding and the manner of its paying for healthcare, social security, research, statistics, social aspects, international cooperation.

Task priority no. 9

International co-operation in in the RD field:

⇒ **Implementation of the EU directive**

Significantly extending and simplifying the practical utilization of international cooperation is provided for by the EU directive, which was transposed into Slovak law. The Commission shall regularly monitor the implementation of the EU directive and its use in practice.

⇒ **Implementing European recommendations**

International cooperation in the field of RD is particularly necessary for small EU Member States. It is implemented by way of sharing experience, and exchanging information and the recommended best practices.

EUCERD – currently CEGRD (Commission Expert Group on Rare Diseases) has drawn up recommendations for three key activities in developing the care for patients with RD, and for all of them has pointed out the importance of international cooperation. To be progressively put into practice are: the qualitative criteria for National specialized RD centres of expertise, the recommendations for the European reference networks and recommendations for the RD registry.

⇒ **International cooperation between patient organizations**

International cooperation with patient organizations, will be arranged most notably through the SAZCH and EURORDIS.

⇒ **Slovak Republic involvement in projects and programmes**

We need to encourage Slovak centres to engage in international programmes and projects in the field of RD within the international cooperation framework (e.g.: EUROPLAN, EUROCAT, ICBDSR, Eurogentest, Orphanet, ESHG, ESHRE, ERNDIM, ICBDSR, the EC IRDiRC project). The involvement of the Slovak Republic in the international programmes should allow easier access to the National grant projects.

1