CZECH REPUBLIC

EUROPLAN NATIONAL WORSKHOP

in the framework of the EU Joint Action RD-ACTION

Prague, 20 September 2017

FINAL REPORT

Co-funded by
the Health Programme
of the European Union

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 Union 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	Czech Republic
National Alliance (Organiser)	ČAVO – Czech Association for Rare Diseases
Date & place of the national workshop/conference	Ministry of Health, Prague, 20 September 2017
Website	www.vzacna-onemocneni.cz
Members of the Steering Committee	Prof. Milan Macek, MD Mrs Anna Arellanesová Mr René Břečťan Mr Vojtěch Kučera
List of Themes addressed	- Centres of Expertise for Rare Diseases and European Reference Networks for Rare Diseases - State of the Art of the Czech Republic's Second National Plan for Rare Diseases 2015-2017
Annexes :	I. Programme in English II. List of Participants (by stakeholders' categories)

FINAL REPORT

I. Introduction

The Czech Republic's EUROPLAN national workshop, organised within the framework of the 2nd EU Joint Action for rare diseases – RD-ACTION (2015-2018), took place in the context of the meeting of the national Rare Diseases Steering Committee at the Ministry of Health of the Czech Republic in Prague, on 20 September 2017.

The audience included representatives of the Ministry of Health, health insurances and renowned healthcare specialists for rare diseases in the country. The patients were represented by ČAVO and EURORDIS.

Nearly a year after the endorsement of 24 European Reference Networks (ERNs) for Rare Diseases composed of the best medical specialists and centres of expertise across EU member States, the meeting assessed the situation in the Czech Republic as regards the involvement of Czech medical specialists for rare diseases in these ERNs and their recognised status of expert at the national level.

The meeting also discussed in the second session, the state of the art of the National Plan for Rare Diseases, presenting achieved actions and those which remain to be implemented.

II. Theme: Centres of Expertise and European Reference Networks

The national context regarding designation criteria for Centres of Expertise in the Czech Republic. The Czech Republic has adopted a National Strategy for Rare Diseases for 2010-2020. Within this strategy, the second National Action Plan for Rare Diseases was adopted for the period 2015-2017.

The designation of Centres of Expertise for Rare Diseases in the Czech Republic is an important action of the Strategy and the Plan. However, **the criteria for designating Centres of Expertise at the national level are still under discussion despite a relatively high number of national renowned healthcare specialists for various rare diseases.**

As a side note, the EU Committee of Experts on Rare Diseases (EUCERD), including all 28 EU member states' representatives and other experts, adopted a Recommendation on *'Quality Criteria for Centres of Expertise for Rare Diseases in Member States'*, on 24 October 2011.

The European context for the establishment of European Reference Networks for Rare Diseases
In March 2016, the European Commission (EC) launched a Call addressed to all healthcare providers in the EU member states to form European Reference Networks (ERNs) for complex diseases requiring a high concentration of medical expertise as defined in the article 12 of the *EU Directive 2011/24/EU on the application of patients' rights in cross-border healthcare*, 9 March 2011.
Each network corresponds to large clinical groupings encompassing altogether most of rare diseases and aiming to cover all of them in the future as proposed in the *Addendum of the Recommendation on European Reference Networks* (10 June 2015) adopted by the European Commission Expert Group for Rare Diseases (replacing the EUCERD).

As of January 2017, the EU Board of Member States on European Reference Networks endorsed all the applications for ERNs – 24 in total - given the excellence of these applications.

These ERNs create a clear governance structure for knowledge sharing and care coordination across the EU to improve access to timely diagnosis and treatment as well as to the provision of high-quality healthcare for patients with rare and complex conditions. ERNs are networks of Centres of Expertise and Healthcare Providers that are organised across borders.

Around 900 healthcare providers from the EU have joined the 24 ERNs. In early 2018, the European Commission will launch another call for additional centres and healthcare providers to join the existing networks.

The Czech Republic can be proud to belong to the most active EU Member States within this unique European endeavour. Indeed, **29 centres located in all major Czech university hospitals have successfully joined 17 ERNs, which demonstrates the recognised high-level medical expertise of healthcare professionals and centres of expertise for rare diseases in the Czech Republic**. In this context, it needs to be noted that these centres have fulfilled complex and stringent international criteria and had successfully passed an independent international audit, both from the professional and healthcare provider perspectives. This attests their extraordinary quality which surely complies, or even exceeds, minimum national criteria for centres of highly specialised care stipulated in Article 112 of Czech health care Act 372/2011 Coll.

This success should encourage the Health Ministry to accelerate the process for designating these centres at national level.

Involvement of EURORDIS and the rare disease patient community in ERNs

The EURORDIS' representative, Ms Ariane Weinman, provided an overview of ERNs. She reminded the audience that the rare disease patient community has advocated for over ten years for the establishment of ERNs, seen as the best way to map out medical expertise across Europe, share knowledge on specific conditions (via virtual tools) and reduce long delays to the right diagnosis and treatment for many rare diseases. The patient community together with all other stakeholders got involved in a long-term dialogue with a view to transform the concept of ERN into concrete networks of clinical excellence. Several legal steps were necessary such as the adoption of the EU Directive on on the application of patients' rights in cross-border healthcare in 2011, the Delegated and Implemented Acts of the European Commission to establish ERNs as defined in the Article 12 of the EU Directive, and several policy recommendations adopted by the EUCERD and Commission Expert Group on Rare Diseases.

The rare disease patient community has also advocated for the promotion and integration of Centres of Expertise for rare diseases and ERNs to be included in National Plan/Strategy.

The establishment of ERNs for rare diseases is a huge success. ERNs represent a unique and innovative European endeavour in terms of fostering access to clinical excellence in a timely fashion. These networks shall optimise clinical outcomes, hence generate efficiencies in healthcare systems.

ERNs represent a great hope for the rare disease patient community.

In parallel to the establishment of ERNs, EURORDIS launched in 2016 **the European Patient Advocacy Groups (ePAGs).** There is one ePAG for each of the 24 ERNs. These ePAGs ensure that patients are integrated in the development, governance and operations of the ERNs and guarantee the founding principle of ERNs comes into practice (i.e. patient-centered care, patient empowerment, patient engagement). The ePAGs' member organisations have elected their **ePAGs' representatives** who have an official permanent mandate to represent ePAG member organisations. They liaise with the ERN coordinating team and ensure true and equitable representation of the patient voice by participating in the Board and sub-clinical committees of their respective ERN; they are voting members of ERNs Board.

EURORDIS and the entire rare disease patient community strongly encourage EU member states to a) further map out national medical expertise for rare diseases, b) officially designate national centres of expertise as per the *EU Council Recommendation on Rare Diseases,* 8 June 2009, and c) provide support to national centres to join existing ERNs.

In early 2018, the European Commission will launch a second Call for Expression of Interest to Health Care Providers across the EU to join existing ERNs. This is another very important opportunity for member states and the patients.

Recommendation:

In order to successfully run and develop the ERNs and foster long-term sustainable Czech participation, it is crucial that individual centres and healthcare providers are acting as legally anchored centres of expertise for rare diseases (i.e. “centres of highly specialised care”) in compliance with the Art 112 of Czech health care Act 372/2011 Coll.

The Ministry of Health is invited to undertake the necessary administrative steps required for the establishment of the national centres of expertise for rare diseases in the Czech Republic. This would contribute to further recognise the clinical excellence of the Czech Republic and reinforce its role in ERNs as well as in research on rare disease.

III. Theme: State of the Art of the Czech Republic’s Second National Action Plan for Rare Diseases 2015-2017

Identification of achievements, specific gaps, challenges and needs: As the second NAP is about to be concluded it was necessary to perform evaluation from the patient perspective. In 2015, the inclusion of the terms “rare diseases” and “medicinal product for rare diseases” entered our legislation. The awareness about rare diseases among professionals and lay public increased (survey performed in 2015). The neonatal screening increased by 5 metabolic rare diseases. Orphanet was translated into Czech and finally, the Czech Republic successfully joined 17 ERNs. However, there are many challenges and gaps that need to be addressed and achieved in the next NAP. Specifically the finalization of giving legal background to the health care providers that joined the ERNs on national level. It is absolutely crucial to improve pre and post gradual education and work on improvement of system of access to treatment, as the Czech reimbursement approval process is one of the longest in Europe.

Assessment of the integration of European guidelines and policy recommendations into the national system: It is absolutely crucial to comply with the given guidelines and recommendations arising from the European level. Specifically to share the knowledge and information which was performed on national level in other countries, so as not to duplicate our efforts. International cooperation is the key to successful implementation of standards of care accepted within expert societies for RD.

Expected outcomes in the near future, medium and long term: The patient community for RD in the Czech Republic relies on the work done together with the Ministry of Health to establish the centers of expertise which will comply with the recommendations stated by the EUCERD. Access to drugs for RD should be faster and easier. Higher rate of participation in clinical trials. Long term, the necessity to dramatically improve collaboration between the ministry of health and the ministry of social affairs and labor. Social support for families with RD is not present in the current legislation.

Recommendation: Which actions/ measures need to be taken:

The Czech Republic recently had its parliamentary elections and the ministries will have new leaders. It is necessary to continue fulfilling the Strategy for RD through the new NAP for 2018 – 2020 which needs to be formed in cooperation with the patient representatives to ensure the effectiveness of this plan.

ANNEXES

ANNEXE I: PROGRAMME

CZECH REPUBLIC

EUROPLAN NATIONAL WORSKHOP

in the framework of the EU Joint Action RD-ACTION

in the context of the meeting of the national Rare Diseases Steering Committee at the Ministry of Health of the Czech Republic

Prague, 20 September 2017

1. Access to specialised care for European citizens living with a rare disease – Ms. Ariane Weinman Public Affairs Senior Manager in Eurordis- Rare diseases Europe

2. Creation of highly specialized centres of care for RD according to § 112 Act no. 372/2011 Coll., on healthcare services and conditions of its usage – prof. MUDr. Milan Macek, DrSc. Czech National Coordination Center for RD

3. Evaluation of National Action Plan for Rare Diseases for 2015 – 2017 and preparation of new – Bc. Anna Arellanesová ČAVO, Ing. René Břečťan ČAVO, prof. MUDr. Milan Macek, DrSc., Czech National Coordination Center for RD

4. Up to date information on involvement of Czech Republic in ERA-NET Co-fund (E-RARE-3) – Ing. Renata Hrubá, Czech Ministry of Education

5. Discussion on the situation od health care providers involved in the ERNs

6. Other

ANNEXE II: LIST OF PARTICIPANTS

Names	Institutions
prof. MUDr. Jan Janoušek, DrSc.	Motol University Hospital Prague
prof. MUDr. Josef Veselka, DrSc.	Motol University Hospital Prague
prof. MUDr. Jan Lebl, CSc.	Motol University Hospital Prague
prof. MUDr. Pavel Dřevínek, Ph.D.	Motol University Hospital Prague
MUDr. Ondřej Souček	Motol University Hospital Prague
prof. MUDr. Tomáš Seeman, CSc.	Motol University Hospital Prague
prof. MUDr. Jana Prausová, CSc.	Motol University Hospital Prague
prof. MUDr. Pavel Kršek, Ph.D.	Motol University Hospital Prague
MUDr. Jana Haberlová, Ph.D.	Motol University Hospital Prague
prof. MUDr. Petr Marusič, Ph.D.	Motol University Hospital Prague
prof. MUDr. Tatiana Dostálová, DrSc.	Motol University Hospital Prague
MUDr. Petra Hliňáková, Ph.D.	Motol University Hospital Prague
prof. MUDr. Jana Hercogova, DrSc.	Bulovka Hospital
prof. MUDr. Jiří Zeman, DrSc.	General Faculty Hospital Prague
prof. MUDr. Evžen Růžička, DrSc.	General Faculty Hospital Prague
prof. MUDr. Jan Roth, Ph.D.	General Faculty Hospital Prague
doc. MUDr. Petra Lišková, Ph.D.	General Faculty Hospital Prague
MUDr. Bohdan Kousal	General Faculty Hospital Prague
MUDr. David Ambrož	General Faculty Hospital Prague
MUDr. Pavel Jansa	General Faculty Hospital Prague
prof. MUDr. Pavla Doležalová, Ph.D.	General Faculty Hospital Prague
MUDr Zdeňka Hrušková, PhD.	General Faculty Hospital Prague
doc. MUDr. Marek Svoboda, PhD.	Masaryk Institute of Oncology
prof. MUDr. Martina Vašáková, Ph.D.	Thomayer Hospital Prague
prof. MUDr. Petr Arenberger, DrSc.	University Hospital Vinohrady Prague
prof. MUDr. Michal Kršek, CSc.	University Hospital Vinohrady Prague
prof. MUDr. Milan Brázdil, DrSc.	Saint Anne's University Hospital Brno
MUDr. Renata Gaillyová	University Hospital Brno
prof. MUDr. Hana Ošlejšková, Ph.D.	University Hospital Brno
prof. MUDr. Jiří Mayer, DrSc.	University Hospital Brno
prof. MUDr. Jaroslav Štěrba, Ph.D.	University Hospital Brno
MUDr. Stanislav Voháňka. CSc., MBA	University Hospital Brno
doc. MUDr. Eliška Dastychová, CSc.	Saint Anne's University Hospital Brno
Prof. MUDr. Milan Macek, MD	Motol University Hospital Prague
Mrs Anna Arellanesová	ČAVO – Czech Association for Rare Diseases
Mr René Břečťan	ČAVO – Czech Association for Rare Diseases
Mr Vojtěch Kučera	ČAVO – Czech Association for Rare Diseases
Ms Ariane Weinman	EURORDIS