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EVENT REPORT: The future of rare diseases in Europe and Czech Republic - what are the solutions at EU and national level?

20 June 2022, 16:00 – 17:30

The roundtable **'The future of rare diseases in Europe and Czech Republic - what are the solutions at EU and national level?'** was hosted by EUCOPE on Monday 20 June 2022. In the following document, you may find the summary of the key points discussed.

Panellists:

- Jakub Dvořáček, Deputy Minister of Health, Czech Republic
- Anna Arellanesova, Chairperson, Rare Diseases Czech Republic
- Prof. Pavla Doležalová, Doctor, General University Hospital, Prague
- Leona Perinova, CEE Lead, Swedish Orphan Biovitrum

Keynote speech:

- Jakub Dvořáček, Deputy Minister of Health, Czech Republic

Welcome address:

- Oliver Sude, Deputy Secretary General, EUCOPE

Final remarks

- Vittoria Carraro, Associate Director, Government Affairs, EUCOPE

Moderator:

- Antoine Mialhe, Senior Managing Director, FTI Consulting

The Commission's proposal for the review of the Orphan Medicinal Product (OMP) Regulation is expected by the end of the year. It aims to address the main challenges for orphan drugs and to ensure EU can maintain a regulatory framework that can foster, and drive innovation and investments address unmet medical needs.

The future of rare diseases at EU and national level

- In a little over twenty years, since 2000, the European Regulation on orphan medicinal products EC 141/2000 (the OMP Regulation) greatly contributed to the development of orphan medicines with the approval of 200 drugs in at least one orphan indication, ultimately improving the lives of 6.3. million people living with rare diseases in Europe, their families, and carers. The Regulation is a positive example of how a regulatory framework can foster and drive innovation and investments to address unmet medical needs.

- However, there are still important steps to be taken to address the 95% of rare diseases without an authorised treatment. To address these challenges, a review of the OMP Regulation is expected by the end of the year.
- The national dimension is also crucial to ensure orphan therapies reach patients. In the Czech Republic a new orphan drug procedure to expedite access has been established, that builds on the current EU framework for orphan drugs.
- The EU OMP Regulation alone cannot address the unmet medical needs of people living with rare diseases, and Member States play a crucial role and coordination of national access policies is an essential step.
- The fragmented access environment for orphan and rare disease therapies across the 27 Member States increases uncertainty and leads to unnecessary delays. There is a need for a more coordinated approach at EU level (e.g., EU HTA).

The Czech Presidency to the Council of the EU and rare diseases (July 2022 – December 2022)

- Rare diseases are a key priority for the Czech Presidency, with as key objective to improve access and availability of treatment to people living with rare diseases. The goal is to deliver a progress report at the EPSCO meeting of 9 December 2022. There will be a rare disease conference on 25-26 October in Prague to host further discussions with various stakeholders. The conference will build on the French Presidency work towards a common EU approach to rare diseases.
- The Czech Republic recognises that there is momentum for change in Europe. The last two years demonstrated that healthcare policies have a space for discussion at EU level.
- The Czech Presidency will aim to build as well on the French work done on the Beating Cancer Plan. Oncology will remain a key priority.
  The Czech Presidency will drive the Commission to build a similar plan for rare diseases as it worked well for cancer.
- The Czech Presidency will also focus on the learnings from the success story of vaccinations. This does not only concern procurement and joint negotiations but also the importance of communications and closer regulations.

The main challenges for people living with rare diseases

- People living with rare diseases face similar issues and challenges across Member States, more particularly in Eastern Europe.
- The main challenges for people living with rare diseases are availability of and access to expert care. These challenges cause delayed diagnosis and in access to treatment and therapies.
- In addition to expert care, there is a challenge in attracting junior physicians to healthcare systems which traditionally have extremely high workloads.
- Cross-border healthcare still has big obstacles and is generally only available for well-spoken patients who know how to navigate healthcare systems.

New Czech procedure for access to orphan drugs

- The Czech Republic has recently introduced a new orphan drug procedure to expedite access, building on the current EU framework for orphan drugs. The new legislation

recognises the social burden of rare diseases and includes patients and experts as part of the decision-making process.
- Thanks to the new pathway, authorisation holders can now apply for access in three different ways. Traditional reimbursement is thus still possible.
- There are currently five requests being considered via the new pathway, with more filings to be expected.

The patient organisation landscape

- The Czech Republic has 120 patient organisations of which 30% represent rare disease patients.
- In 2017 the Patient Council was created as part of the Ministry of Health alongside the Department for Patient Support. The Patient Council serves as an advisor to the ministry to bring information from the impacted patients.
- This involvement required a professionalisation of the patient organisations. The establishment of patient academies was very helpful in this education.

The role of European Reference Networks (ERNs) & improving rare disease care

- As mentioned, rare disease patients face similar challenges across countries, and the collaboration is crucial for smaller countries where expertise and infrastructures might have not developed at the same pace as in more advanced ones.
- Key challenges in the Czech Republic are to develop and maintain this needed expertise and to grant patients access to expert care. To face these hurdles the role of the European Reference Networks (ERNs) has been key.
- The European Reference Networks (ERNs) are virtual networks involving healthcare providers across Europe. To further improve and expand their role, the availability of data and support to experts in ERNs are two of the main issues to be tackled.
- It is important to build a robust system of care also in smaller Member States. There is no need for a specialised treatment centre for every rare disease in every Member State if a network of centres that share capacities is built.

Considerations for investment in rare disease R&D

- Companies need to consider prioritisation of treatment delivery and financial stability. Rare disease drug development has high risk of failure and uncertainty.
- It is often difficult to get therapies reimbursed – payers' willingness to pay is a key aspect in decisions to invest.
- Obtaining high-quality data is crucial to obtain stakeholder approval. Links between clinical programmes can be helpful here.
- Without tailored incentives to incentivise R&D, developer interest would be much lower.
- There is a recognised need for specialised value assessment framework, for improved knowledge expertise and expectation on data on rare diseases that can be realistically shared at the time of value assessment. This is coupled with the need to change the public debate on rare diseases to ensure alignment of all stakeholders and change the landscape of rare diseases.

Final considerations

- Back in 2000, European Member States adopted the 'EU Orphan Regulation'. The ambition of the European Commission was to stimulate the development of new medicines for treatment of rare diseases, better known as 'orphan medicines'.
- Looking at the EU Orphan Regulation in 2022, more than twenty years after it was adopted, the European Commission managed to get more investments and more treatments approved for patients with rare diseases. Almost 200 orphan medicines had been authorised in the EU for several unique conditions, including some very rare ones.
- The revision of the OMP regulation should build on the success of the current system. Considering the upcoming revision, there difference between tailoring incentives and simply reducing them should be taken into consideration, to give the right instruments to continue developing rare disease treatments for European patients and ensure the right level of predictability to developers to continue delivering these treatments.
- While we are gearing up for this crucial review expected at the end of the year, it is important to look at the national level for a holistic incentive environment, that goes beyond the OMP Regulation: from the importance of dedicated expertise and infrastructure to the need of specific orphan access pathways.
- This meeting on the future of rare diseases in Europe and the Czech Republic allowed us to explore this dual perspective.

Rewatch the roundtable

The recording of the roundtable is available on the EUCOPE YouTube channel here.

Resources

Some further background readings and proposals for the EU Orphan Medicinal Products legislation revision and the improvement of rare disease policies in Europe are available below:

- EUCOPE's proposals on the OMP regulation revision
- Rare 2030 recommendations
- European Expert Group of Orphan Drug Incentives Recommendations to improve incentives for the lifecycle of orphan drugs