

WE HAVE
#30 MILLION REASONS
FOR EUROPEAN

ACTION

ON RARE DISEASES



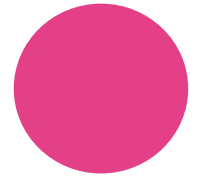
POLITICAL SUPPORT

European Parliament	<ul style="list-style-type: none">• Plenary Debate Nov 21• Letter to Commissioner
European Council	<ul style="list-style-type: none">• Conferences under both French and Czech Presidencies• Czech “Call to Action” for MS endorsement
European Commission	<ul style="list-style-type: none">• Meeting with European Commission for Health 3 Nov• Meeting Deputy Director General 17 Oct
Internationally	<ul style="list-style-type: none">• UN Resolution adopted; WHO Resolution in the works

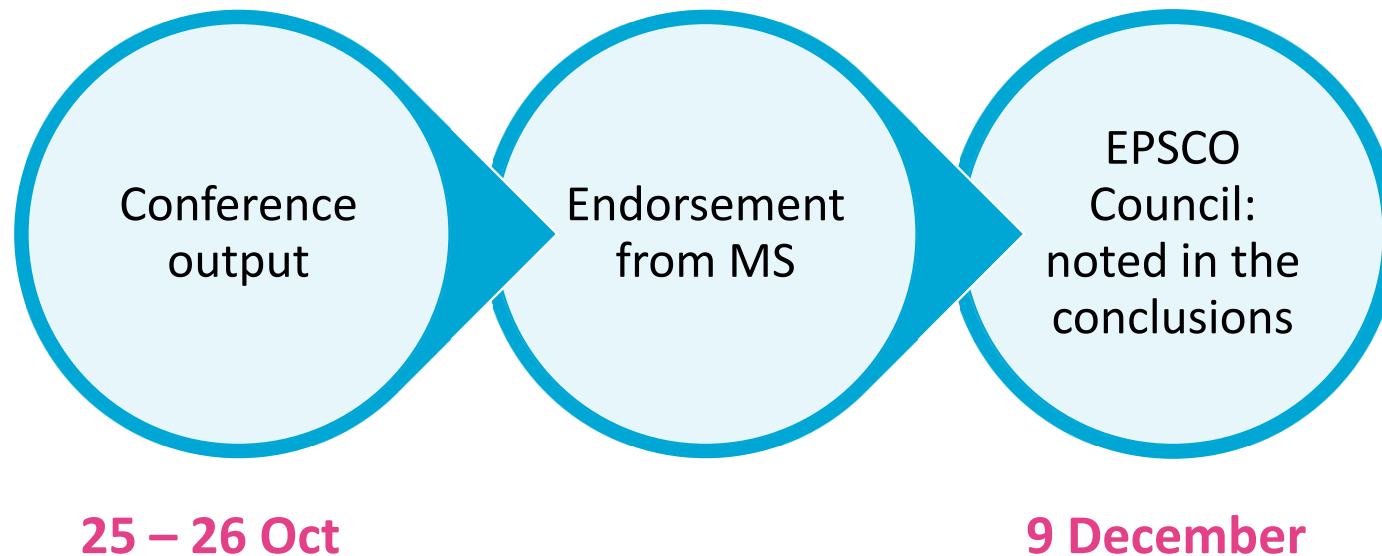


EXPERT CONFERENCE ON RARE DISEASES, PRAGUE 25-26 OCT

- Czech Presidency event
- 90 delegates, including 3 invitees from each MS
- Speakers incl. EC representatives, ERN representatives, Patient Advocates, industry
- Agenda split into five blocks:
 - A new goal-based and coordinated strategy for rare diseases
 - Early Diagnosis for Rare Diseases
 - Revision of the Orphan Drug and Paediatric Drug Regulations
 - Instruments for improving access to treatments for rare diseases
 - Holistic healthcare pathways: Integrating European Reference Networks into European health care and social systems



CZECH CALL TO ACTION



CZECH CALL TO ACTION

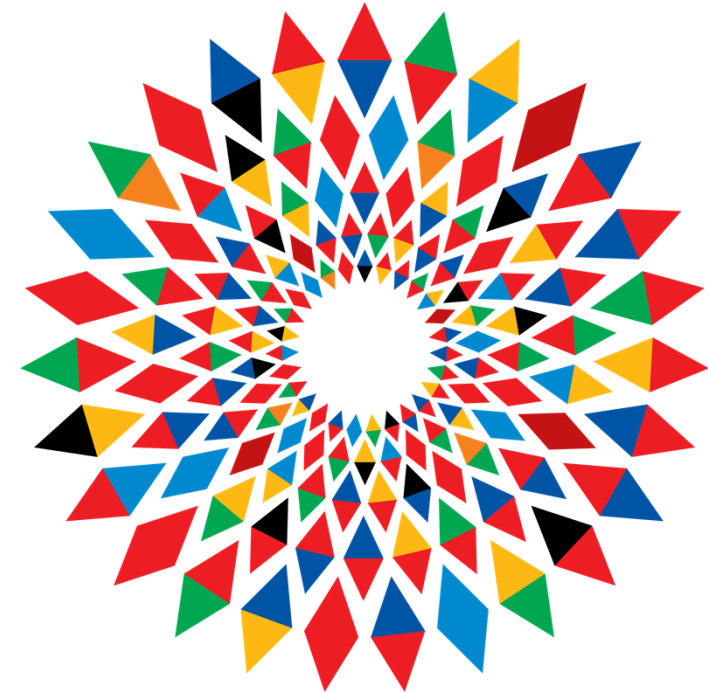
A call for a European Action Plan on rare diseases

Early diagnosis

Revision of OMP
Legislation

Instruments for
improving
access to
treatments for
rare diseases

Integrating
European
Reference
Networks into
European health
care and social
systems



I) A CALL FOR A EUROPEAN ACTION PLAN

- Call upon the European Commission **to adopt a coordinated EU Strategy on rare diseases to support and complement on-going and future efforts** at both the EU and Member State level.
- This should take the shape of a Commission Communication on addressing the challenges of persons living with rare diseases.
 - Bringing together existing legislation towards common goals
 - Integrating and sustaining European and national plans and strategies for rare diseases on a long-term basis;
 - Measurable and time-bound goals
 - Space to innovate



II) EARLY DIAGNOSIS

- Share best practice and lessons from national newborn screening (NBS) programmes;
- Collect, collate and develop key performance indicators to improve the quality of NBS programmes;
- Create an EU-level NBS Expert Advisory Committee, free from bias or national interests, to provide trusted, high-quality information to support decision making at a national level.



III) REVISION OF THE ORPHAN DRUG AND PAEDIATRIC DRUG REGULATIONS

- A model that centred on the unmet needs of people living with a rare disease, and includes patient participation in its establishment and implementation;
- Transform the European Research & Development for the rare disease ecosystem building upon advances of the past 20 years, for the next 20 years;
- Situate Europe as a global leader in research, development and access, through a regulation that is attractive and competitive globally;
- Establish a European pathway, from development to access, to ensure innovation coupled with affordability and to gain that crucial strategic autonomy in research and development;
- Ensure convergence and coherence between different relevant legislation.



V) INSTRUMENTS FOR IMPROVING ACCESS TO TREATMENTS FOR RARE DISEASES

- Strengthening European cooperation in pricing and negotiations: mechanisms of voluntary cooperation between EU Member States need consolidating and unifying while respecting current treaty competences.
- Establish a European pathway, from development to access, to ensure innovation is coupled with affordability and to gain that crucial strategic autonomy in research and development, starting with very low prevalence diseases and complex treatments.
- Explore the feasibility of piloting cross-country mechanisms to improve best practices and information exchanges, value assessments, demand pooling and negotiating and purchasing models, as stated by WHO Europe . Such a platform should be able to explore new approaches to affordable pricing, reimbursement and funding (for example: external reference pricing, price regulation, equity-based tiered pricing, value-informed pricing, and staggered, performance-based or subscription payment models).
- The European Commission should consider the initiation of action towards the creation of a “European Fund” to support the generation of evidence across the whole life cycle of products, focusing on products for very small populations and/or complex treatments, such as Advanced Therapeutic Medicinal Products (ATMPs), for which evidence at time of pricing and reimbursement (P&R) is often immature.
- explore opportunities for joint negotiations with producers of complex treatments and treatments for small populations that have the potential to improve accessibility of treatment across the EU, in a way that could possibly be incorporated into the revision of Orphan Drug and Paediatric Drug Regulations as regulatory incentive.



STUMBLING BLOCKS?

- “Already have a lot for rare diseases, what is the added value?”
- Ursula von der Leyen makes all the decisions
- Capacity in DG Sante – secretariat proposals but forward



WE NEED YOUR HELP!

- Draft letter to your Minister of Health calling on them to endorse the Call to Action
- Ask who will be attending the conference?
- Communications around the conference: reiterating member state support. What does this mean for your country?
- Don't forget the reasons!

TOOLKIT

Template letter & CTA

Reasons from your
country



KEY ARGUMENTS

- There is a need for a **comprehensive policy framework to connect all policies and initiatives** affecting people living with a rare disease at European and national levels
- This would **bring areas such as digital to data to research to diagnosis to treatments to social care to ERNs under one umbrella framework**

Orphacodes... to connect to experts ... to drive research... to build awareness... to better manage care... to bring treatments to market...

KEY ARGUMENTS

- It would **drive innovation** to make the EU a competitive world leader on innovative therapies and technologies, and,
- **Promote a European model of care** for persons living with a rare disease

European added value... in line with European values

KEY ARGUMENTS

- For the first time this would be a strategy that looks beyond health, by addressing the whole spectrum of challenges faced by people living with a rare disease in Europe, in line with the UN Resolution Addressing the Challenges of Persons Living with a Rare Disease
- This would also introduce measurable goals, similar to those in obesity or cancer and aligned with the SDGs for all countries to work towards for longer, better lives thanks to innovation

Improve life expectancy by three years... Reduce psychological, social and economic vulnerability of people living with a rare disease and their families by ⅓... Diagnosis within six months of seeking medical advice... Best practice across European Member States ... Approval of approximately 1000 innovative (symptomatic or transformative) treatments for people living with a rare disease... Increase investment in rare disease innovation by 200%

Q&A

