Treatment without borders: the EU case for equitable patient access to advanced therapies
Welcome and introduction
• Bernard Grimm, EuropaBio

Opening keynote: Is EU cross-border access to advanced treatments a reality or a goal for the future - value of treatments for rare disease patients?
• Jana Popova, Executive Committee Member of the European Alliance of Neuromuscular Disorders Associations & EUPATI Patient Engagement Training Coordinator

Panel
• Martin Dorazil, Deputy Head of Unit Digital Health, DG Health, European Commission;
• Irina Grossenbacher, Region Europe Patient Access Director at Novartis Rare Diseases;
• Brieuc Van Damme, Director General, RIZIV/INAMI, the National Institute for Health and Disability Insurance;
• Tomislav Sokol, MEP, EPP, Croatia;
• Dirk Vander Mijnsbrugge, Vice President Medical Affairs Lead Rare Diseases International Developed Markets, Pfizer

Reaction to the Panel discussion by Jana Popova, Executive Committee Member of the European Alliance of Neuromuscular Disorders Associations & EUPATI Patient Engagement Training Coordinator

Closing remarks
• Bernard Grimm, EuropaBio
Your role today

• Thank you for your attendance and active participation!
• Ask your questions in the Q&A box
• Share a comment with all participants in the chat box
• Event recording and slides will be available after the event
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What are the key barriers in the implementation of CBHC legislation?

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Is EU cross-border access to advanced treatments a reality or a goal for the future - value of treatments for rare disease patients?
Jana Popova, Executive Committee Member of the European Alliance of Neuromuscular Disorders Associations & EUPATI Patient Engagement Training Coordinator
Rare and equal –

EU legislation on cross-border healthcare

Jana Popova,
EUPATI
EuropaBio’s Advanced Therapies Series,
07.09.2021
Who am I?

- Patient advocate since 2006.
- Member of the Bulgarian Association for Neuromuscular Diseases since 2006.
- Executive Committee Member of the European Alliance of Neuromuscular Disorders Associations (EAMDA) since 2017.
- Former member of the EPF Youth Group and EPF Board.
- Recipient of the EURORDIS Black Pearl Award 2020 for Young Patient Advocate.
- EUPATI Fellow since 2021.
- PhD in media and digital communications at Sofia University.
What is EUPATI?

- 2012 IMI Project
- 2017 EPF
- 2020 EUPATI Foundation

EUPATI: Patient Engagement Through Education
Providing patients with accessible and reliable information and training on medicines research and development and other therapeutic innovations. A patient-led partnership working across stakeholders to enhance patient engagement.
What is CBHC?

Article 168 of the TFEU: MS and organisations of health services

1. A high level of human health protection shall be ensured in the definition and implementation of all Union policies and activities. **Union action, which shall complement national policies**, shall be directed towards improving public health, preventing physical and mental illness and diseases, and obviating sources of danger to physical and mental health. **Such action shall cover the fight against the major health scourges**, by promoting research into their causes, their transmission and their prevention, as well as health information and education, and monitoring, early warning of and combating serious cross-border threats to health. The Union shall complement the Member States' action in reducing drugs-related health damage, including information and prevention.
What is CBHC?

(Continued)

5. The European Parliament and the Council, acting in accordance with the ordinary legislative procedure and after consulting the Economic and Social Committee and the Committee of the Regions, may also adopt incentive measures designed to protect and improve human health and in particular to combat the major cross-border health scourges, measures concerning monitoring, early warning of and combating serious cross-border threats to health, and measures which have as their direct objective the protection of public health regarding tobacco and the abuse of alcohol, excluding any harmonisation of the laws and regulations of the Member States.

6. The Council, on a proposal from the Commission, may also adopt recommendations for the purposes set out in this Article.

7. Union action shall respect the responsibilities of the Member States for the definition of their health policy and for the organisation and delivery of health services and medical care. The responsibilities of the Member States shall include the management of health services and medical care and the allocation of the resources assigned to them. The measures referred to in paragraph 4(a) shall not affect national provisions on the donation or medical use of organs and blood.
Patient perspective on CBHC journey

Historical Background:

▪ European initiatives in the field of CBHC:
  
  
  - Regulation 883/2004 on the coordination of social security systems and its S2 pathway
  
  - Directive 2011/24/EU on patients’ rights in cross-border healthcare – promote cooperation between Member States on healthcare matters (Article 15)
  
▪ **Ultimate goal** – provide better and quicker CBHC for patients, no matter of their disease, country of residence and social status.

▪ **Ultimate goal 2** – better observation of patients’ rights and collaboration between Member States.
Patient perspective on CBHC journey

**Historical Background:**

- **Patients’ feedback:** the directive has the potential to improve the access and quality of healthcare, if it is implemented in a patient-center way by Member States.

- **Patients’ feedback:** certain aspects of the directive could be built upon for long-term improvements (stronger European cooperation on safety and quality, better implementation of HTA, stronger European Reference Network, better eHealth etc.).

- **Eurobarometer survey about CBHC:**
  - Provide patients’ evaluation of the directive about CBHC and its implementation;
  - Fewer than 2 out of 10 respondents felt well-informed about their rights in CBHC;
  - Only 1 in 10 respondents is aware of the existence of National Contact Points.

Challenges in the implementation of the Directive

▪ **Patients’ rights** – the original purpose of the directive was to identify legal rights of patients, however based on the implementation to date, this goal hasn’t been achieved.

▪ **Quality of care** – high quality of healthcare should be provided to patients, no matter of the geographical region, social status and other social aspects.

▪ **Information to patients** – equal access to reliable, trustworthy and understandable information is a both right in itself and a fundamental prerequisite to exercise one’s right.

▪ **Dissemination of National Contact Points** – better dissemination in the society of the work of the National Contact Points.

▪ **Opportunities for reimbursement** - better opportunities for cross-border healthcare reimbursement and its better implementation in national policies.

CBHC and rare diseases

- Rare disease patients are defined by many characteristics, such as the need for diagnosis, treatment and information seeking.
- In many cases they have needed to access health services outside their country.
- Directive 2011/24, EU nationals have the right to seek planned healthcare in another EU country.

CBHC and rare diseases

▪ Challenges for patients with rare diseases to seek and receive CBHC:
  - Lack of expertise;
  - Better time planning;
  - Better legislation of the prior authorization requirements;
  - Improvement of the Social Security Regulation.

▪ Requirements for CBHC for patients with rare diseases:
  - Better requirements for clinical evaluation and scientific advice;
  - Increased awareness;
  - Overcoming the isolation.

Dissemination of National Contact Points:

Know before you go

Check your treatment plans with your health professional:
- It is highly recommended that you discuss your planned treatment with your doctor before committing to anything.

Plan your trip thoroughly:
- Research your treatment options.
- Make sure you have a copy of your medical records, information on any medicines you are taking, and any relevant test results.
- Check whether you will need a referral from a general practitioner to access (or be reimbursed for) specialist care.
- Check the details of your healthcare provider.

Check the financial implications with your National Contact Point or insurer:
- Make sure you know how much your treatment will cost and whether your authorities will pay the costs directly, or will reimburse you some or all of the costs.
- Check any requirements for pre-treatment authorisation.
- Remember that some costs (travel, accommodation, repatriation, etc.) may not be covered.

Make sure you get the medical follow-up you need:
- Arrange to get a copy of your record from your healthcare provider.
- If you get a prescription make sure it is suitable for cross-border use (EU law prescribes certain minimum content to make sure that prescriptions can be recognised in every country).
- Arrange appropriate medical follow-up with your home system (in advance if necessary).

You can find more information on this subject and also on emergency or unplanned healthcare at www.europa.eu/youreurope.

Seeking healthcare in another EU Member State: your rights

Did you know?

You have the right to receive medical treatment in another EU Member State and the right to have your home country cover some or all of the costs.

You have the right to be informed about the treatment options open to you, how other EU countries ensure quality and safety in healthcare, and whether a particular provider is legally entitled to offer services.

Look inside to find out more...
Conclusions on patient perspective on CBHC journey

- Guidelines for information about CBHC, NCPs and patient involvement;
- Guidelines about quality and safety of healthcare services;
- Establishment of a platform, which can provide updated and reliable information about CBHC;
- Involvement of patients and healthcare professionals in the development and implementation of standards about safety and quality of healthcare;
- Legislation between the healthcare institutions in different Member States that provide CBHC;
- Health inequalities between different Member States should be overcome;
- Harmonization of methods about NCPs;
- Establishment of a position of patients’ ombudsman.

Questions?
Thank you for your attention!

Jana Popova,
jana.popova@eupati.eu,
jana.s.popova@gmail.com
PANEL DISCUSSION
Martin Dorazil, Deputy Head of Unit Digital Health, DG Health, European Commission

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Brieuc Van Damme, Director General, RIZIV/INAMI, the National Institute for Health and Disability Insurance

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Tomislav Sokol, Member of the European Parliament, EPP, Croatia
Evaluation of Cross-border healthcare directive: European Reference Networks

Martin Dorazil, DG SANTE - European Commission
EU legal framework for cross-border healthcare

➢ Coordination of social security systems (Regulation No 883/2004)

➢ Directive on the application of patients’ rights in cross-border healthcare (Directive 2011/24/EU)
Patients’ rights in cross-border healthcare Directive

CJEU jurisprudence from 1998
• Healthcare is a service
• Patients can choose healthcare provider abroad
• Level of reimbursement up to cost of treatment at home
• Prior authorisation is acceptable

Harmonized minimum Patients’ Rights

Kohli and Decker (1998); Ferlini (2000); Geraets-Smits and Peerbooms (2001); Vanbraekel (2001); Inizan (2003); Müller Fauré and Van Riet (2003); Leichtle (2004); Watts (2006); Stamatelaki (2007); Elchinov (2010); Petru (2014) etc.

Information to patients

Cooperation between Member States

• Evaluation of the policy and legislation in accordance with “Better Regulation” principles

• 10 years after the adoption of the Directive

• The evaluation will assess:
  • How the Directive’s objective to facilitate access to safe and high quality cross-border healthcare in another Member State has been met?
  • To what extent is the Directive relevant for meeting patient needs in cross-border healthcare?
  • To what extent the Directive has promoted patient rights and cross-border cooperation between Member States for the benefit of EU citizens?
  • Approaches implemented by Member States in practice? How effectively these are working, what barriers patients still face seeking healthcare across borders?
  • How the treatment and diagnosis of patients with rare and complex diseases have benefitted from the support of the ERNs?
European Reference Networks

https://ec.europa.eu/health/ern_en
ERNs: Legal basis

Article 12 of Directive 2011/24
- The Commission shall support Member States in the development of ERNs
- Member States encouraged to facilitate the development of ERNs
- Objectives of the networks
- Commission shall:
  - Adopt list of criteria and conditions that the networks and their members must fulfill
  - Develop and publish criteria for evaluating ERNs
  - Facilitate exchange of information and expertise

Commission Implementing Decision 2014/287/EU
- defining criteria for establishing and evaluating ERNs,

Commission Delegated Decision 2014/286/EU
- defining the criteria and conditions that healthcare providers and the ERNs should fulfil
ERNs: Next Steps

Demonstrate the added value of the ERNs: Evaluation and monitoring

✓ Evaluation of Cross-border Healthcare Directive (2011/24/EU), including legal provisions on ERNs and rare diseases cooperation (2021-2022)

✓ First periodic 5-year evaluation of performance of ERNs and their members (2022-2023)
Some evaluation questions related to ERNs:

- How effective is the directive in supporting the diagnosis and treatment of patients with rare and complex diseases through ERNs?
- How effective is the directive in facilitating knowledge sharing on rare and complex diseases among EU healthcare professionals via ERNs?
- What has been the impact of the directive on the research on rare and low prevalence and complex diseases through the ERNs?
- Are the ERNs as set out in the directive still relevant for meeting the needs of patients with rare and complex diseases?
- In what ways the ERNs established by the Directive provide an added value for patients with rare and complex diseases compared to the national solutions alone?
II. “AMEQUIS” for ERNs

1. “Assessment”
   • Initial assessment of networks and healthcare providers wishing to join the ERN system
   • Each network's and healthcare provider's application should be technically assessed according to the criteria set out in Delegated Decision 2014/286/EU

2. “Monitoring”
   • Continuous monitoring of performance of ERNs and their members throughout the ERN lifecycle
   • Initial basic set of 18 common indicators (structure, process and outcomes) agreed by ERNs (ERN monitoring system)

3. “Evaluation”
   • Periodic evaluation of performance of the networks and their members, five years after their approval or last evaluation (Implementing Decision 2014/287/EU)
I. Evaluation of CBHC Directive

• Started in January 2021 (Roadmap published for feedback)
• Targeted Stakeholder consultations
• Final Evaluation report: 1st half of 2022

II. Periodic 5-year evaluation of ERNs and their members

• AMEQUIS project: methodology, manual and procedure for the evaluation; to be completed in Q1 2022.
• Appointment of new Independent Assessment and Evaluation body: 1st half 2022
• Start of the ERN 5-year evaluation: 2nd half 2022
• Final evaluation reports on each network and each member: 2023
Further information:
What are the key barriers in the implementation of CBHC legislation?
What are the key barriers in the implementation of CBHC legislation?

- HCP's understanding of framework
- Lack of patient engagement
- The need to pay upfront
- Affordability perceptions
- Net price confidentiality
- Member States
- Ongoing monitoring protocols
- Nationalised P&R mechanisms
- Authorization process
- Prices
- Access to network
- The different legislations of
- Lack of awareness among doctors
- HCPs not aware of the ERNs