

Conference - Rare diseases and European Reference Networks: how to ensure European solidarity for patients?

11.10.2023

Bilbao

Panel session 1: Rare diseases: organisational models and good practices in health and social care.

Opinion of the European Economic and Social Committee on Ensuring strong European solidarity for rare disease patients

Alain Coheur

Director European and international affairs, Solidaris- Belgian mutual health insurance fund

Member of the European Economic and Social Committee

Rapporteur, own initiative opinion (2023/C 75/10)



Ensuring strong European solidarity for rare disease patients: EMR-realities inspiring EU possibilities.

	Huntington	Duchenne	CML	PV/ Vaquez	Rett	Silver- Russell	PKU	Galactosemia
1. Primary HCP	X	X	X	X	X	X	X	X
Baby-sitter or live-in nurse		X			X			X
GP	X	X	X	X	X	X	X	X
Medical secretary						X		
Nurse	X	X		X	X		X	X
Paediatric nurse		X			X	X	X	
Paediatrician		X			X	X	X	X
Pharmacist								X
Social assistant	X	X			X		X	
2. Paramedical HCP	X	X	X	X	X	X	X	X
Acupuncturist					X			
Dietician	X	X			X	X	X	X
Occupational therapist	X	X	X		X			
Ortho-pedagogist					X			
Osteopath					X			
Physiotherapist	X	X	X		X	X		
Prosthetist		X			X			
Psychologist	X	X	X	X	X	X	X	X
Psychomotor therapist					X			
Speech therapist	X	X			X	X		X
3. Specialists	X	X	X	X	X	X	X	X
Cardiologist	X	X	X	X	X			
Endocrinologist			X			X		
Eye doctor		X			X	X		X
Gastrointestinal paediatrician		X			X	X		
Geneticist	X	X			X	X	X	
Haematologist			X	X				
Lung specialist		X						
Metabolic specialist							X	X
Neurologist	X	X			X			
Neuro-psychiatrist	X							
Orthopaedic surgeon		X			X			
Psychiatrist	X	X			X			
Revalidation specialist	X	X			X			
Specialist (not specified)	X	X	X	X	X	X	X	X
N HCP mentioned	15	22	8	6	25	13	10	11

Figure 1: HCPs mentioned in the patient pathway, comparison between diseases, EMRaDi, Report of work package 2 Field analysis of existing RD patient pathways in the EMR p57.

28.2.2023	EN	Official Journal of the European Union	C 75/67
Opinion of the European Economic and Social Committee on Ensuring strong European solidarity for rare disease patients (own initiative opinion) (2023/C 75/10) Rapporteur: Alain COHEUR			
Plenary Assembly decision	24.2.2022		
Legal basis	Rule 52(2) of the Rules of Procedure Own-initiative opinion		
Section responsible	Employment, Social Affairs and Citizenship		
Adopted in section	29.9.2022		
Adopted at plenary	26.10.2022		
Plenary session No	573		
Outcome of vote (for/against/abstentions)	171/1/1		

Figure 2: Official Journal of the European Union, C75, Volume 66 28 February 2023

Ensuring strong European solidarity for rare disease patients: conclusions and recommendations

1.1... The EESC deeply regrets that, **more than ten years** after the adoption of its opinion, it has to reiterate its call for a comprehensive European approach that takes into account all the needs of people with rare diseases, and calls for **European solutions to mitigate the impact of rare diseases on daily, family and professional life.**

1.2 ...Supporting fundamental research and a **European Health Data Space (EHDS)** based on the FAIR principle (findability, accessibility, interoperability and reusability) ...

1.3 ...**the similarities in the rare disease patients' pathways and social protection challenges** despite the **heterogeneity** or multitude of diseases and the dispersion of patients and expertise.

1.4 ensuring that the **situation for rare disease patients is not aggravated by health inequality.**

1.5 ...establishing a comprehensive **European action plan** on rare diseases with SMART goals attainable by 2030...

1.6 create **a new European authority** for non-communicable diseases that would foster coordination on and solidarity for rare diseases in order to coordinate the **implementation of a European action plan** on rare diseases and to ensure a European approach to non-communicable rare diseases

1.7 The EESC recommends that **the next trio-presidency 2023–2024 Spain, Belgium, Hungary** keep the rare disease policy on the agenda.

1.9 ...benefit from **integrated medical and social care and centralised care coordination**, and above all, **optimisation of the financial accessibility of care.**

Ensuring strong European solidarity for rare disease patients: political message

1.13 The EESC advises researching a **solidarity fund for rare diseases**, specifically those not included in the ERNs. Such a fund can be a useful addition when the compulsory health insurance does not cover costs for complex or rare disease treatment or cross-border care and the EESC believes **that a mutualisation at the European level is a necessity**. A European solidarity fund for rare disease patients should:

- **aim to prevent** rare disease patients from **incurring unbearable costs for medically necessary and justifiable healthcare** that is available in the EU and from suffering further health inequality due to the rarity of their disease;
- **express European solidarity** to improve access to healthcare available across the EU for all patients with a rare disease, to better enforce patients' rights to cross-border healthcare, and to optimise and facilitate the use of ERNs;
- complement national social security and health insurance provisions by developing a fund to cover associated and unavoidable costs related to cross-border care within the EU and facilitate European cooperation in tackling public health challenges that would benefit from a structural and supportive cross-border approach.

Ensuring strong European solidarity for rare disease patients: flagships from the EESC-opinion to achieve patient-centred EU RD policy.



Ensuring strong European solidarity for rare disease patients: evaluating conclusions and recommendations.

Proposal EESC	Reaction Commission
<ul style="list-style-type: none"> (1.7) Political prioritization and ambitious continuation of rare disease policy in public health policy - revision EU strategy RD (ECA- report). 	<ul style="list-style-type: none"> End 2023: results evaluation the European reference networks determines assessment strategic approach RD. Full implementation existing opportunities - Pharmaceutical strategy for Europe.
<ul style="list-style-type: none"> (1.11) Access to care& speed up diagnosis- optimisation functioning ERNs: integration in national HCsystem, idea of convention for care in ERNs. 	<ul style="list-style-type: none"> Start of joint action integration ERN into national health systems: mid 2023. Healthcare provision is a responsibility of the MS. The ERNs link clinical units and support health professionals in reaching specialised knowledge for diagnosis and treatment.
<ul style="list-style-type: none"> (1.12; 1.13; 4.13) Reflection on creating a special EU financial fund (MS contribute to and benefit from according to their financial capacity) in order to ensure access to treatment for all European RD patients especially those with unmet needs. Accessibility of medicines requires new methods – join procurement, a fair price calculator. 	<ul style="list-style-type: none"> The recommendation to create an EU fund to ensure access to treatment for patients is a matter within responsibility of the MS related to organisation and delivery of health services and medical care, including the allocation of the resources. The Commission has no competence. Pharmaceutical strategy for Europe-considers supporting MS in the area of joint procurement and pricing& reimbursement.

Ensuring strong European solidarity for rare disease patients: evaluating conclusions and recommendations.

Proposal EESC	Reaction Commission
(4.1) European financing instruments invest in research on RD.	<ul style="list-style-type: none">• The EU4Health programme – may facilitate uptake, scaling- up and deployment of health innovations & make sure the best use is made of research results.• EHDS will<ul style="list-style-type: none">• create an efficient framework for the use of health data for research, innovation...• allow RD patients to better access and control their health data and make these data available in a secure way to their treating. Stimulate research and development.
(3.1; 4.1; 4.2;) Optimize the potential of the ERNs.	<ul style="list-style-type: none">• Evaluation of ERN expected in late 2023. 2100 consultations took place up to now.
(2.8; 4.9) EHDS- needs to trigger a debate on concise, standard registration and the purpose of registers.	<ul style="list-style-type: none">• Rare disease registers- EHDS standards could apply to RD registers.• The establishment of EU-level standards for RD can support patient data collection and data exchange.
(4.12) Revise the EU legislation on orphan and paediatric medicines to improve accessibility: joint purchasing, fair pricing calculator	<ul style="list-style-type: none">• Pricing and reimbursement are the purview of MS. The Commission will step-up co-operation with and among MS and the affordability and cost-effectiveness of medicines.

Ensuring strong European solidarity for rare disease patients: takeaways for EU rare disease policy.

➤ EU pharmaceutical policy

- Accessibility and affordability of RD medicines
- EESC 1.9 – Solidarity fund, 2.6.1 extending exclusivity, 2.6.4 - 5:10 000

➤ EHDS

- Civil society participation in creation and implementation
- Supporting digital health literacy of citizens (RD patients and HCP)

➤ Evaluation of the ERNs

- An ambitious review of the EU RD Strategy

➤ Policy cycle 2024: public health and rare diseases policy

- European action plan

Thank you

Alain.coheur@solidaris.be

