European Economic and Social Committee

OPINION

European Economic and Social Committee

Ensuring strong European solidarity for rare disease patients

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Full title Ensuring strong European solidarity for rare disease patients
[Own initiative opinion]

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1. Conclusions and recommendations

1.1 In 2009, the European Economic and Social Committee (EESC) adopted opinion SOC/330 on the *Proposal for a Council Recommendation on a European action in the field of rare diseases*, expressing its support, concerns and suggestions with a view to raising awareness of all the needs of people living with rare diseases[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 The EESC strongly reaffirms its support for and solidarity with rare disease patients, their families and the rare disease community at large. The European Union (EU) could be the champion of the right to healthcare for all throughout the European Union and demonstrate that rare doesn't mean alone. Supporting fundamental research and a European Health Data Space (EHDS) based on the FAIR principle (findability, accessibility, interoperability and reusability) is necessary for faster diagnosis and treatment of rare diseases. The EESC advises recognising and fully promoting Orphanet's expertise in order to enhance the European health data ecosystem to benefit rare disease patients. Making Orphanet's - website available in all EU languages would provide great added value for rare disease patients and health care professionals.

1.3 The EESC reaffirms the findings on the prevalence of rare diseases in the EU, 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 The EESC welcomes the principle of the right to access healthcare proposed by the European Pillar of Social Rights, the United Nations resolution and the attention given by the Conference on the Future of the European Union (COFEU) and the 2022 French Presidency of the Council of the EU to rare diseases, with a view to ensuring that the situation for rare disease patients is not aggravated by health inequality. The Committee highlights the importance of an ambitious European care strategy for informal caretakers of rare disease patients.

1.5 The EESC recommends seizing the political momentum and building on the recommendations by institutions and civil society, with the aim of establishing a comprehensive European action plan on rare diseases with SMART goals attainable by 2030, to ensure that all rare disease patients in the EU enjoy equal opportunities to diagnosis, treatment and a holistic perspective on integrated care. The aim must be for patients to receive a diagnosis of their rare disease within a year.

1.6 The EESC suggests extending the Health Emergency Preparedness and Response Authority (HERA) mandate or using it as a model to 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. Working in synergy with Orphanet,

1 OJ C 218, 11.9.2009, p. 91

which would benefit from structural EU support so that it can publish its work in all the official languages of the EU, would guarantee that both patients and professionals have access to the information they need.

1.7 The EESC gives Member States' civil society a voice in order to enhance political dialogue with the public and supports the European institutions through structural and permanent cooperation in order to develop policy that is fully supported. The EESC recommends that the next trio-presidency 2023–2024 Spain, Belgium, Hungary keep the rare disease policy on the agenda, bearing in mind the 2022 evaluation of the European Reference Networks (ERNs) and the Commission's pledge to revise its rare disease strategy by early 2023, by incorporating rare diseases into public health policy for future Commission terms of office. Stakeholder and social partner involvement is crucial in developing an ambitious strategy.

1.8 The EESC requests initiatives such as a resolution to empower rare disease patients and stimulate their participation in rare disease policy and recommendations in line with Article 4 of the UN Convention on the Rights of Persons with Disabilities (UNCRPD). Patient associations, a source of experience, can play a vital role as representatives by speaking out on behalf of patients; their media appearances and participation in policy recommendations are to be guaranteed and supported[2].

1.9 The EESC calls for recognition of the importance of accessing a rare disease diagnosis during perinatal or neonatal screening or as soon as possible after the onset of health or developmental problems, the benefits of multidisciplinary healthcare and a holistic perspective in relation to patients' needs and pathways, which might benefit from integrated medical and social care and centralised care coordination, and above all, optimisation of the financial accessibility of care.

1.10 The EESC states that quality health services can never be the privilege of those who can, for any reason, secure better access to the relevant national health service, afford the highest insurance premiums, out-of-pocket payments or organise the most profitable collection campaigns. The importance of solidarity-based health insurance systems that protect rare disease patients cannot be underestimated. The EESC would welcome a debate on the benefits and challenges for European solidarity-based mutual health insurance funds to cover innovative treatment for rare disease patients.

1.11 The EESC recognises how important it is for European rare disease patients to be able to access cross-border treatment for diagnosis and care. The ability to both travel for treatment and avoid excessive travel via telemedicine can improve access to care for rare disease patients, especially those with very rare diseases. The EESC requests that the functioning of the ERNs be optimised and calls for their integration into the entire EU and healthcare systems of the Member States. The EESC suggests that the option of drawing up a convention for care in ERNs be explored.

1.12 In view of the unequal economic situations of the Member States, the EESC recommends and expects a reflection on the possibility of creating a Special EU Financial Fund which Member States contribute to and benefit from according to their financial capacity in order to ensure

2 The UN Human Rights Office (2006), Convention on the Rights of Persons with Disabilities

access to treatment for all European rare disease patients, especially those with unmet medical needs, ensure genuine solidarity in the EU. The EESC supports models of joint purchasing and contributions, such as the European fair price calculator for medicines, to increase the accessibility of pharmaceutical treatment for Member States and rare disease patients and requests that this be taken into account in the Revision of the EU's legislation on orphan and paediatric medicines (medicines for people with rare diseases and for children).

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 in order 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.

2. General observations on rare diseases and the European rare disease policy

2.1 General observations on rare diseases

2.2 Rare diseases are rare, but rare disease patients are numerous – a disease is classified as rare based on prevalence. In the EU a rare disease is defined as a condition which is often chronic, sometimes disabling or life-threatening, and which does not affect more than 1 in 2000 persons[3]. In 2019, Orphanet – the portal for rare diseases and orphan drugs – counted 6172 unique rare diseases[4]. 71.9% of these rare diseases have a genetic origin and 69.9% manifest in early childhood. 3.5% – 5.9% of the population is estimated to have a rare disease resulting in approximately 36 million patients in the EU.

2.3 The complexity and chronic nature of many rare diseases often reach beyond the life of the patient alone and impact many others such as the family, but also health and social care systems. Families can become at risk of isolation and aggravated vulnerability and, given the

3 European Commission (2019) Rare Diseases/

4 Orphanet (2021) Orphanet in numbers: 6172 diseases https://www.orpha.net/consor/cgi-bin/index.php; Nguengang Wakap S, Lambert DM, Olry A, Rodwell C, Gueydan C, Lanneau V, Murphy D, Le Cam Y, Rath A. Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database. Eur J Hum Genet. 2020 Feb;28(2):165-173. doi: 10.1038/s41431-019-0508-0. Epub 2019 Sep 16. PMID: 31527858; PMCID: PMC6974615.

gender dimension of informal care[5], a rare disease can significantly affect the lives of mothers and women in particular. Access to social protection for informal carers should therefore be a particular focus.

2.4 Although more than 6172 diseases have already been identified and can lead to a patient being diagnosed, definitions, characterisations or diagnostic tests may still be lacking for some conditions and these are so-called syndromes without a name (SWAN). The health gap is even more pronounced for patients without a diagnosis. Their unmet needs are even higher and the inequality even greater since a diagnosis is necessary to obtain appropriate medical care or additional social and health insurance benefits.

2.5 Persisting inequalities in access to healthcare require proactive and targeted community-based models so that the most vulnerable groups for instance people with physical, psychosocial and sensory disabilities can receive diagnoses and care. Previous EESC opinions have addressed healthcare for migrants and for EU citizens with a migrant background; this expertise and these recommendations need to be built on as part of a common approach to rare diseases[6].

2.6 Diagnosing a rare disease – if not identified by perinatal screening - takes an average of around 4.5 years after health or developmental challenges arise. Research has found that periods of diagnostic uncertainty – often including misdiagnosis and/or incorrect treatment that causes deleterious consequences – fluctuate between up to 5 and 7 years[7]. The path to a conclusive and correct diagnosis often amounts to an odyssey that involves being seen by several healthcare professionals: research has demonstrated how 22% of diagnosed rare disease patients consulted more than five healthcare professionals and 7% even more than ten[8].

2.7 Raising awareness among healthcare professionals so that they can better detect potential rare diseases and empowering them to refer patients and speed up the diagnostic process require information sharing, adequate and continuous high-quality training of the workforce and timely workforce planning with the involvement of social partners.

2.8 Prioritisation and structured investment in fundamental medical research on causes of rare diseases, including genetic causes, must lead to more efficient treatment and possibly even access to cures for rare disease patients. European financing instruments such as the 2021-2027 EU4Health programme – a vision for a healthier EU – and regulations such as the proposal on the EHDS should support such research.

2.9 The knowledge and expertise required to diagnose and manage the specialised therapeutic care needs required for some rare diseases can be unavailable in some Member States and geographically dispersed throughout the EU. Treatments need to be more available, accessible

5 Eurocarers (December 2021), The gender dimension of informal care.

6 OJ C 286, 16.7.2021, p. 134; OJ C 286, 16.7.2021, p. 141 notably 6.8 Inherited diseases and 6.12 Barriers for migrant's access to and effective use of health care systems.

7 Eurordis; Rare disease impact report: insights from patients and the medical community 2013 detailing diagnostic uncertainty of low prevalence diseases in the United States and the United Kingdom.

8 Koning Boudewijnstichting (2014) , Zoom: nieuwe perspectieven op gelijke kansen- Zeldzame ziekten.

and affordable, with patients reporting unavailability where they live (22%), waiting lists hampering access to treatment (14%), financially unaffordable treatment (12%) and financial support being unavailable to facilitate travel to receive treatment in another country (12%)[9].

2.10 Quality of care requires health services to be timely, equitable, integrated and efficient[10]. Perinatal and neonatal screening are vital processes in early diagnosis. The *Recommendation on Cross Border Genetic Testing Of Rare Diseases in the European Union* by the Commission Expert Group on Rare Diseases and Eurordis' work on screening throughout the EU lay the groundwork for a Europe-wide recommendation.

2.11 The path to diagnosis, receiving a diagnosis and life with a rare a disease can be mentally challenging for the patient and/or their family. Psychological and social vulnerability can be caused by the invisibility of a disease, its physical burden and a lack of knowledge or understanding of the conditions from others. Daily life can be aggravated by poor care coordination but also by challenges of a practical, administrative, educational, professional or financial nature[11]. A holistic approach to care covers the 360° spectrum of health (prevention and continuous, curative, rehabilitative and palliative healthcare), social and everyday needs and requires high-quality integrated multidisciplinary medical and social care.

2.12 Rare Disease Day raises awareness and recognition among society at large and increases understanding and social inclusion of patients and families. Informing rare disease patients and ensuring the wellbeing of patients and their family requires an ecosystem of healthcare professionals, mutual health insurance funds, (digital) contact groups and patient associations.

3. General observations on the European rare disease policy

3.1 The EU identified rare diseases as a priority in the field of public health more than twenty years ago and took action that resulted in: increased research and development, the Member States' adopting national action plans on rare diseases, coordination of cross-border cooperation in the ERNs and patients' rights to access cross-border care[12]. The Commission accepted recommendation 3 "Improve support to facilitate rare disease patients' access to healthcare" and announced that it would revise its rare disease strategy where appropriate by early 2023[13]. The European Parliament adopted its resolution on the EU public health strategy in the post COVID-19 era, which calls for an EU action plan for rare diseases[14]. The EU also enshrined "timely

9 Kole, A., Hedley V., et al. (2021) Recommendations from the Rare 2030 Foresight Study: The future of rare diseases starts today: Available, accessible and affordable treatments – what do people living with a rare disease think? P.119

10 World Health Organization (2022), Quality of care.

11 Loridan J., Noirhomme C. (2020) Field analysis of existing rd patient pathways in the EMR.

12 Official Journal of the European Union: (22. 1. 2000 L 18/1) Regulation (EC) no 141/2000 of the European Parliament and of the Council of 16 December 1999 on Orphan medicinal products; (2009/C 151/02) Council Recommendation of 8 June 2009 on an action in the field of rare diseases; (4.4.2011 L 88/45) Directive 2011/24/EU of the European parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare.

13 European Court of Auditors (2019), EU actions for cross-border healthcare: significant ambitions but improved management required.

14 European Parliament (10 July 2020), The EU's public health strategy post-COVID-19 European Parliament resolution of 10 July 2020 on the EU's public health strategy post COVID-19 (2020/2691(RSP)).

access to affordable, preventive and curative health care of good quality" in the European Pillar of Social Rights[15].

3.2 The announced Communication on a European care strategy is expected to include support for implementation of the European Pillar of Social Rights and proper recognition of informal carers. Families of rare disease patients would benefit from a strategy that better recognises carers and their rights across the EU, that offers more flexibility in the exercise of carers' rights in cross-border situations and that includes mental health (that of formal and informal carers) as a main concern[16].

3.3 EU Member States co-sponsored the 2021 United Nations Resolution *Addressing the challenges of persons living with a rare disease and their families*[17], which includes a call to "strengthen health systems…to empower persons living with a rare disease in addressing their physical and mental health needs to realize their human rights, including their right to the highest attainable standard of physical and mental health, to enhance health equity and equality, end discrimination and stigma, eliminate gaps in coverage and create a more inclusive society".

3.4 The Employment, Social Policy, Health and Consumer Affairs Council (Health) addressed Europe's response to rare diseases and discussed whether it would be useful to strengthen cooperation and coordination on rare diseases between Member States and at EU level. The presidency of the Council considered that boosting EU action in this field would bring the tangible benefits of the public health union to European citizens. The European Health Data Space is one tool that should contribute to more effective EU action, by playing a role in combating rare diseases and ensuring access to high-quality health data within a safe framework. It must make new, safer, personalised treatment accessible sooner[18].

3.5 The report on the outcome of the Conference on the Future of the EU includes a proposal on equal access to healthcare for all, with the objective of establishing a "right to health" – guaranteeing all Europeans equal and universal access to affordable, preventive, curative and quality health care. The Conference plenary specifically recognized and referenced the rare disease community, supporting: faster and stronger decision-making on key subjects and to improve the effectiveness of European governance as it moves towards the development of the European Health Union; the need to make sure anyone can access existing treatments, wherever first available in the EU; to that end, facilitating cross-border cooperation, especially in the case of rare diseases; reinforcing the healthcare system to reinforce the resilience and quality of our healthcare systems, in particular, through the further development, coordination and funding of

15 COM(2021) 102 Final communication from the Commission to the European Parliament, the Council, the European Economic and Social committee and the Committee of the Regions The European Pillar of Social Rights Action Plan {SWD(2021) 46 final}.

16 International Association of Mutual Benefit Societies (AIM) (2022), AIM's Views on the EU Care Strategy.

17 United Nations (5 January 2022), A/RES/76/132: Resolution adopted by the General Assembly on 16 December 2021 *Addressing the challenges of persons living with a rare disease and their families*.

18 Employment, Social Policy, Health and Consumer Affairs Council (Health), (29 March 2022), Main results – Europe's response to rare diseases.

the European Reference Networks as they constitute the basis of the development of networks of medical care for highly specialised and complex treatments[19].

3.6 The 2021 "Europe's Beating Cancer plan: A new EU approach to prevention, treatment and care", the list of actions to be achieved by 2030 and the involvement of stakeholders are an approach to European health policy aimed at tackling health inequalities within the EU[20]. The plan also builds on the European Reference Networks, which are pioneers in exchanging expertise on diagnosing and treating rare diseases.

4. Specific observations on rare diseases and rare disease policy

4.1 Evaluating the follow-up to the EESC opinion on the *Proposal for a Council Recommendation on a European action in the field of rare diseases*[21], the EESC sees that, while the recommendations are currently still being addressed with varying degrees of success for instance, through the development of ERNs from 2017 onwards, the introduction of a communication and reporting system, handbooks or guidelines to facilitate dialogue between different professional cultures within the EU and the EHDS to include a requirement for patients to be able to access their data the EU rare disease policy requires urgent action, with a lot of catching-up to be done.

4.2 ERNs are a flagship of concrete European cooperation between healthcare systems facilitating clinical trials and expertise in diagnosis and treatment for European patients with rare diseases. The potential of these ERNs has currently not yet been fully examined and is not yet in operation. An evaluation is scheduled to commence in 2022[22]. The 24 ERNs, founded in 2017, reached 1.466 ERN-members in all European Member States, including more than 900 healthcare units located in over 313 hospitals. 1.7 million patients are being treated by ERN-members, however, only 2.100 complex and very rare disease patients cases have been dealt with through the Clinical Patient Management System (CPMS).

4.3 Factors to be addressed in order to optimize the potential of ERNs are: the lack of reimbursement for healthcare providers participating in the ERNs, no specific reimbursement for virtual consultations via CPMS, problems of administrative or technical interoperability. Another working point is the integration of ERNs into national healthcare systems via the affiliated reference centres for rare diseases, guaranteeing the promotion of their existence and accessibility.

4.4 Centralising care for rare disease patients while maintaining a sufficient number of centres of expertise would benefit the quality of care. Criteria that define a centre of expertise need to be established. Centres of expertise require specific and adequate funding. Given that civil society

19 Conference on the Future of Europe, Report on the final outcome May 2022.

20 Europe's Beating Cancer Plan Communication from the commission to the European Parliament and the Council (2021).

21 OJ C 218, 11.9.2009, p. 91

22 SWD(2022) 200 final, Commission staff working document Accompanying the document Report from the Commission to the European Parliament and the Council on the operation of Directive 2011/24/EU on the application of patients' rights in crossborder healthcare {COM(2022) 210 final}, European Reference Networks p. 29-36.

and the social partners generate the resources used to fund public health expenditure, they should be given a strategic role in distributing such resources. Local, regional and national care networks need to be made aware of the existence of centres of expertise and encouraged to participate in ERNs to facilitate access to care and improve the quality of care.

4.5 Cross-border multi-stakeholder partnerships and consortia supported with EU funding, bringing together civil society and experts on rare diseases and health or social policy, academia, medical partners, knowledge centres, patient associations, not for profit mutual health insurance funds and expert patients have been shown to be enriching ecosystems for European research and cooperation. They have contributed to formulating patient-centred policy recommendations, pilot projects and studies to improve the access of European rare disease patients to high quality holistic and integrated health and social care[23]. The time has come to consolidate these recommendations and best practices into coherent policy that integrates national, cross-border and European initiatives, leaving no rare disease patient behind.

4.6 The participatory Rare 2030 Study on *Foresight in Rare Disease Policy* formulated eight crucial recommendations on treatment, care, research, data and European and national infrastructure, with a roadmap and SMART goals setting the tone for the next decade of rare disease policy: 1) long-term, integrated European and national plans and strategies; 2) earlier, faster, more accurate diagnosis; 3) access to high quality healthcare; 4) integrated and person-centred care; 5) partnerships with patients; 6) innovative and needs-led research and development; 7) optimising data for patient and societal benefit; 8) available, accessible and affordable treatments[24].

4.7 Acknowledging the expertise of rare disease patients, relatives and health care professionals formed the crux of the EMRaDi-project, which amongst others, examined supply and demand in the field of rare diseases in the Euregio Meuse-Rhine. The project also analysed the day-to-day reality and patient pathways based on 104 in-depth interviews on eight rare diseases[25]. The latter confirmed assumptions about diagnostic difficulties, an increased burden in care coordination (with patients encountering between six and 25 healthcare professionals in their patient pathway), the need and preference for multidisciplinary care in specialised centres and by extension the need for a broader holistic perspective on the entire 360° spectrum of informational needs, psychological support, social inclusion and development chances, practical and administrative but also cross-border healthcare needs. The project formulated recommendations on holistic care; telemedicine and European solidarity[26].

4.8 The COVID-19 pandemic accelerated the digitalisation of healthcare, the use of new technologies and the deployment of telemedicine. Regulation, capacity building and reimbursement of telemedicine, comprising teleconsultation, tele-expertise, telemonitoring and

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23 INNOVCARE (2018) Bridging the gaps between health, social and local services to improve care of people living with rare and complex conditions; EMRaDi (2020) Rare diseases do not stop at borders; RARE 2030 (2021) Foresight in Rare Disease Policy.

24 Kole, A., Hedley V., et al. (2021) Recommendations from the *Rare 2030 Foresight Study: The future of rare diseases starts today*.

25 EMRaDi (2020) Final report of the EMRaDi project.

26 EMRaDi project (2019) Factsheet EMRaDi – How to get EU actions on rare diseases (RD) closer to RD patients and their relatives? From local and cross-border developments to European solutions.

mobile health, need to be the result of consultation with social partners and stakeholders from the medical sector and must above all ensure patient safety and the quality and continuity of care and treatment. Optimal use of telemedicine prevents patients, including those with rare diseases, from having to travel excessively either within their own country or across Europe.

4.9 Advancing academic research, health economics and quality of care for rare diseases require patient registries based on the FAIR principle (findability, accessibility, interoperability and reusability). Initiatives such as the European Registry Data Warehouse, the European Rare Disease Registry Infrastructure metadata repository (ERDRI.mdr) and the European Health Data Space need to trigger a debate on concise, standard registration and the purpose of registers.

4.10 Mapping of the supply and demand in the field of rare diseases requires quantitative analyses of prevalence, care consumption and care costs of patients with rare diseases with utmost respect for the patients privacy. An innovative methodology by the Belgian health insurance funds permitted a first analysis of prevalence, care costs and care consumption of rare disease patients compared to the average care cost and care consumption of its affiliated members[27].

4.11 The analysis confirmed a higher care consumption than the average member (more frequent hospital visits and admission, more frequent GP and specialist care), which can be explained by the more complex care needs. The costs in the compulsory health insurance system were confirmed as ten times the average member with a yearly out-of-pocket contribution three times the average member. Medicines comprised the biggest part, averaging half of spending. Real costs are to be expected much higher since the study did not take into account the socio-economic circumstances of the family or other non-reimbursed costs such as psychological care or paramedical care, complementary insurance or mere out-of-pocket costs. The analysis does prove the importance of strong solidarity-based health insurance systems that intervene to protect rare disease patients. When rare diseases patients refuse or do not take up care for financial reasons, this will have an impact on their health, quality of life later on and presents a financial risk of heightened costs in the long run.

4.12 The Revision of the EU's legislation on orphan and paediatric medicines (medicines for people with rare diseases and for children) requires an ambitious approach to ensure that orphan medicines and treatment are affordable for Member State healthcare systems and patients. Currently affordability is a barrier for many rare disease patients. Different forms of European cooperation and models of joint purchasing of medicines between countries – such as Beneluxa[28] or as seen during the COVID-19 pandemic regarding vaccines – have improved access to treatments thanks to a common, transparent, sustainable and supportive EU approach. The debate on fair pricing and the transparency of R&D costs for medicines is enhanced by the proposal for a fair price calculator for medicines and AIM's fair pricing model for calculating a

27 Noirhomme C., (december 2020), MC informations 282, Analyse de la consommation et des dépenses de soins des personnes atteintes de maladies rares, p. 20-29.

28 Beneluxa Initiative on Pharmaceutical Policy.

FAIR price for new or existing medicines (without generic competition) and comparing it to the price paid or being negotiated[29].

4.13 When there is no regular reimbursement possible, different provisions for accessing orphan products by rare disease patients exist in different Member States, including compassionate use programmes, provisions for off-label drugs use and, for instance, also Special Solidarity Fund interventions[30]. Solidarity funds can be a useful addition when the compulsory health insurance does not cover costs for complex or rare disease treatment or cross-border care. This is most certainly the case when no recognised reference centres exist in the EU. Despite the budgetary impact of rare disease treatment, reflections on European conventions for care in ERNs or for rare disease patients accessing care in a reference centre in another Member State, have not materialised.

4.14 The Health Emergency Preparedness and Response Authority (HERA) originated as a response to the COVID-19 pandemic, and as a key pillar of the European Health Union. It aims to prevent, detect, and rapidly respond to health emergencies and to anticipate threats and potential health crises, through intelligence gathering and building the necessary response capacities. Its mission can be extended beyond communicable diseases and its mandate means it can tackle other health threats. The current governance structure of the European Health Union doesn't yet include institutionalised support on preparedness and responses to rare disease challenges the Member States might be faced with. HERA can serve as a model for a new authority for non-communicable diseases that would foster coordination and solidarity for rare diseases.

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Christa Schweng The president of the European Economic and Social Committee

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29 AIM – European fair price calculator for medicines; AIM offers a tool to calculate fair and transparent European prices for accessible pharmaceutical innovations.

30 Maastricht University (2020), Report on the analysis of legal, financial and reimbursement mechanisms of rare diseases for treatment costs of EMR rare diseases patients. 3.2 Orphan medical products p 43-45.