An empty promise: accessing cross-border healthcare for people living with a rare disease

EURORDIS – Rare Diseases Europe responds to the evaluation of patients' rights in cross-border healthcare – and provides recommendations to improve the system

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EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of 970 rare disease patient organisations from 74 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe.

EURORDIS-Rare Diseases Europe welcomes the ongoing evaluation of Directive 2011/24/EU on patient rights in cross-border healthcare ("Cross-border Healthcare Directive"). Ten years after its entry into force and eight years after its transposition deadline, the evaluation of the Directive is timely.

It is the ambition of the Directive on cross-border healthcare to ensure the right of every person living in Europe, including people living with a rare disease, to access the best possible diagnosis, care and treatment without unnecessary delay or physical, social or financial burden, when this cannot be met within the country of residence.

Rare diseases are disabling and degenerative conditions, most of them with no cure, affecting 30 million people in Europe. They scarcely have access to the right expertise and diagnostic infrastructure where they live, as healthcare systems face specific challenges in organising and delivering care due to their complexity and rarity. The needs of these extremely rare conditions are of such magnitude that they require greater cross-border healthcare collaboration to meet them.

However, despite the Directive in place to facilitate such collaboration, obstacles remain that not only make it difficult to access healthcare in another EU country, but leave the patient carrying the administrative, financial, and emotional burden to fight for and organise it. This means that the patients' best interests are not always prioritised and results in a system entrenched in inequity.

WHY IS THE CASE FOR ACCESSING HEALTHCARE IN ANOTHER COUNTRY SO IMPORTANT FOR PEOPLE LIVING WITH A RARE DISEASE?

A patient's odyssey to access a rare treatment for their rare disease

Rare disease expertise, knowledge and centres administering specialist therapies and treatments are scarce and scattered across the European territory. This often means that satisfactory expertise and/or care exists only in countries different from their country of residence.

Unsurprisingly, this means that **86% of people living with a rare disease who responded to our survey Rare 2030 are willing to travel to another country to receive medical treatment**, versus 49% in the general EU population in 2014[1]. Even at the time of a global pandemic, only 9% of people living with rare diseases would ***not*** be willing to travel to another country to receive medical treatment.

The reasons are:

- To improve the standard of care and treatment received in the home country, due to the non-availability or less effective treatment or care.
- Satisfactory expertise and/or care exist only in countries different from the country of residence. This is the case for many Advanced Therapeutic Medicinal Products that are only administered in one specialist centre in one country due to the complexity of the procedure.

[1] EURORDIS-Rare Diseases Europe, May 2021. The future of rare diseases: leaving no one behind!

A) Summary of the responses to the public consultation on the evaluation of the Cross-border Healthcare Directive

EURORDIS-Rare Disease Europe carried out an extensive consultation with people living with a rare disease and patient advocates, including surveys through EURORDIS' survey programme "RareBarometer", webinars, online consultations with specific member groups (for example, Rare Disease National Alliances and European Federations representing a disease area across Europe) and patient advocates in European Reference Networks ('ePAGs').

From this broad membership consultation, it unequivocally emerged that, despite its ambition, the c**ross-border healthcare** is **currently failing people living with a rare disease in Europe**.

More precisely, EURORDIS has identified the following barriers in the Cross-Border Healthcare Directive meeting patients' needs in accessing healthcare in another EU country.

1. The burden of accessing cross-border healthcare weighs heavily on the patient

To access cross-border healthcare, the patient is responsible for overcoming many of the bureaucratic, financial and information obstacles, with limited support from Member States or clinicians.

a) Lack of awareness and information about how to access care in another country

Awareness of the Directive 2011/24 is low amongst patients and healthcare professionals, even amongst those specialised in rare diseases. This hinders the patient from accessing healthcare in another EU country on many levels:

- ***Patients are not aware that accessing healthcare abroad is even an option***

  You cannot look for what you do not know exists. Findings of the recent Rare2030 survey show that people with rare diseases only have partial knowledge of their rights regarding cross-border healthcare and are less aware of these rights compared to the general EU population.

- ***The quality of information on cross-border healthcare is inadequate***

  For patients who are seeking information, it is difficult to find, bureaucratic, repetitive or incomplete. National Contact Points, mandated to hold this information, vary significantly, with some Member States taking the political decision to bury this resource. Where it exists, it is unclear, and contact details are often generic or inaccessible. This means the patient is required to gather information through their personal contacts or by calling healthcare institutions directly or through patient organisations. This also means that information is incomplete: they are not entirely sure of their rights, for example, the level of reimbursement to which they are entitled.

CASE STUDY – THE EXPERIENCE OF RARE CANCER PATIENTS IN LITHUANIA

In Lithuania, EURORDIS' members reported that most healthcare professionals, including physicians at university hospitals, where rare disease patients are treated, are unaware of Reg. 883/2004 or Dir. 2011/24. Reg. 883/2004 is applied only a few dozens of times per year in Lithuania, mostly for paediatric cancers and genetic disorders, where no treatment options are available in Lithuania. Most adult rare cancer patients

are not able to get prior authorisation under Regulation 883/2004 because they have access to treatment, even when it is not effective (standard chemotherapy or radical and invasive surgeries), and are not able to use Directive 2011/24 because they are not aware of the costs of the specific outpatient treatment not just abroad, but also in Lithuania (as finding such information requires specific knowledge – it is not "googleable").

- ***National Authorities are unable to make informed decisions***

Despite being entrusted to give prior authorisations, making informed decisions for people with rare diseases seeking care abroad, it can be challenging due to lack of access to necessary information or knowledge about the specific conditions, or lack of mechanisms to access knowledge to support them (e.g. Centres of Expertise for rare diseases, European Reference Networks or their national members, for example, or other organisations able to provide that information, such as accredited help lines, national alliances of rare disease organisations, etc.). Such mechanisms are particularly important for rare diseases, on which information is scarce by definition.

b) The bureaucratic burden is often carried by the patient

- ***Patients are in the driving seat to transfer the information***

Patients are responsible for filling in the right forms and carrying the information between their home country and country of treatment, often asked to transfer highly complex details between specialists in their non-native language. This includes their health records and data that cannot be transferred between hospitals and often need to be translated. They are also responsible for integrating the care that has been received abroad into their regular care pathway, which may require changes to the course of treatment.

- ***Lack of data exchange systems***

The transfer of data from hospitals is a challenge. Not only do patients often take care of the transfer of medical records: requesting records, making the transfer, translation, but they also suffer the consequences of lack of interoperability of healthcare systems and the lack of portability of their health data. Patients often are unable to bring, for example, MRI and CT scans from one country to another: even if they request a copy of the scan in USB format, it unlocks with specific software, which is often non-compatible with the one used in the hospital abroad. For such reasons, tests are repeated in the country of treatment and when the patient returns to the country of origin.

- ***Two parallel systems add bureaucratic complexity without bringing benefits to patients***

The current legal system, offering two routes for accessing care abroad, has increased the bureaucratic complexity and has decreased the uptake of rights to cross-border care. In fact, the large majority of people living with a rare disease continue to travel under the so-called "Social Security" Regulation 883/2004, rather than the Directive 2011/24. The Directive and its implementation at the national level have regrettably not overcome the shortcomings of the previous legislation, particularly the need for prior authorisation, which can be an extensive and unclear process. As a result, planned care is provided more favourably under the Regulation, also because patients' costs are covered in full, rather than up to the cost level in the home country as in the Directive.

- ***Lack of a clear pathway for accessing transformative treatments***

For certain rare diseases, transformative treatments (notably, gene and cell therapies) have been approved but are administered only in one or very few centres in one country. This is due to the complexity of the treatment administration, which requires highly specialised physicians and hospitals working in networks of reference centres. Eligible patients, often very young hence travelling with their families, have no choice but to travel to the only European centre to have access to life-saving treatment. The RareImpact analysis (www.rareimpact.eu) identified

the administrative and logistical challenges healthcare systems face in catering for advanced therapeutic medicinal products (ATMPs).

Unfortunately, the Directive has failed the objective of enabling their rights to access such specialised treatments and novel therapies when these are not available in their home country. In all the above-mentioned cases, care and reimbursement have been made possible via the Social Security Regulation or bilateral agreements.

Similar patterns are observed for many complex surgeries for paediatric patients. In such cases, the only option is to receive such care via a specialised centre of expertise in a different country. Patients would always prefer to receive treatment as close to home as possible. However, the rarer the condition or, the more aggressive the type of cancer is, the more the patients are left with only option to attempt to receive such therapy abroad.

In some cases, especially for advanced therapies, the refusal of or the unwillingness to grant authorisation has been made on the ground of the excessive costs of the treatment that the patient would have received abroad.

CASE STUDY: A BUMPY ROAD TO ACCESSING GENE THERAPY

In 2020, EURORDIS provided assistance to resolve a referral through the Social Security Regulation for a gene therapy treatment from Sweden to Italy. The treatment is life-changing for an extremely rare disease, but its administration is very complex.

Only one hospital in Europe (in Milan, Italy) can administer the treatment. The patient was diagnosed through Newburn Screening and was eligible for the therapy. However, a series of misunderstandings regarding the implementation of the Regulation and differences in the understanding of the cost effectiveness of the therapy, coupled with contrasting advice on how to approach the request for the S2 form in the host country, threatened access for the patient.

Ultimately, the patient received the therapy, but the unnecessary delay in the process put the patient's life at risk.

- ***Financial burden and hidden costs***

Under the Directive, the need for upfront payment is a clear setback. Not only do many families have difficulty in affording this upfront, but there is also a fear that the government will not reimburse costs of such care, especially as it is difficult to estimate costs, and the information is not always straightforward nor provided by the National Contact Point or authorities. In addition, there have been cases of incomplete reimbursements, and the cost of travel and accommodation ensued are not covered in most cases. Foreign prescriptions for paramedical or care support are often not recognised; patients have to either pay out-of-pocket or revisit a doctor in their home country to have those re-prescribed.

Moreover, it is not always easy to estimate costs in advance when unexpected investigations are made, which is not an infrequent occurrence for rare disease patients. This is in addition to the hidden costs of days off work, translation and interpretation costs and the cost of preparing the paperwork.

Altogether, **the unpredictability of reimbursement decisions and the complexity of reimbursement procedure, coupled with upfront payments and hidden costs, are strong deterrents for rare disease patients seeking care abroad.**

2. The best interests of people with rare diseases are not put first

a) Lack of drive and trust from clinicians

One of the most cited barriers to accessing healthcare in another country was imposed by clinicians or competent national bodies in the home country, who refused to accept that a better treatment or expertise may be available elsewhere, even when peer-reviewed literature proved the opposite. Only the most empowered patients dare to fight the clinicians and the public insurer to receive prior authorisation and often can only do so by hiring lawyers to submit the necessary documentation, landing an extra administrative and financial burden.

The personal relationship between the doctor and the family is therefore extremely significant. No legal pathway is fast enough for urgent treatment, and even without time pressure, it depends on the good will of a doctor to fight with the patient for their rights.

For patients who were able to access cross-border healthcare, patients have also faced mistrust between clinicians both in their home country and abroad regarding the tests and advice given, leading to additional consultations and investigations, or even refusing to conduct follow-up blood tests when the patient has returned home.

As a result of the above, the **enforcement of patients' rights to care abroad is extremely unpredictable and varies significantly from one Member State to another, from one condition to another, with people living with a rare disease experiencing an exacerbated vulnerability in the healthcare systems in addition to those caused by their conditions**.

b) Arbitrary discrimination from National Authorities

The 2019 European Parliament's Resolution on the implementation of the cross-border healthcare Directive observes that "…in a considerable number of Member States, the obstacles that patients encounter when dealing with health systems remains significant… certain prior authorisation systems appear to be unduly burdensome and/or restrictive…" and that insurers "in Member States have discriminated arbitrarily or created unjustified obstacles to the free movement of patients and services…". This is the experience of many people with rare diseases, who reported facing resistance and unjustified obstacles or obstruction from national authorities when seeking cross-border healthcare. They often end up resorting to personal financial means when possible or simply give up.

CASE STUDY: THE JOURNEY OF LYSIANE, OR HOW TO GUARANTEE THE RIGHT TO ACCESS TO CROSS-BORDER CARE TO THE MOST VULNERABLE

The story of Lysiane, a baby born in France with a life-threatening rare disease, exemplifies the difficult road to the enforcement of the right to healthcare abroad. Lysiane's parents identified a highly specialised, medically proven, safe, non-surgical, and cost-effective treatment, offered in Germany, not available in France or anywhere else in the EU.

The French body competent to grant prior authorisation refused it, claiming that the treatment offered in France is "the same or equally effective" as the treatment developed by multidisciplinary German medical teams specifically for this rare disease whose effectiveness and safety have been definitively proven in numerous international peer-reviewed medical studies. Babies from other EU Member States have been transferred to Germany to receive the treatment.

Despite the refusal of authorisation, Lysiane was treated in Germany at her family's expense. The treatment dramatically improved the baby's quality of life, addressing her breathing difficulties, liberating her from a life attached to a breathing machine in an intensive care unit. Lysiane's family has appealed the

decision pointing at the violation of EU law that guarantees citizens, especially rare disease patients, the right to access cross-border healthcare.

c) Delays in prior authorisation reduce the effectiveness of treatment

Prior authorisation is de facto always required for people living with a rare disease seeking healthcare in another EU country. However, this is a long process that can delay access to treatment, causing medical complications for the patient, reducing the effectiveness of the treatment or the ability for the treatment to be given. This is especially significant, for example, for children with a congenital malformation who need surgery within a few days.

CASE STUDIES: PRIOR AUTHORISATION LEAVES THE PATIENT WITHOUT HOPE

Cases were reported of patients with an advanced rare cancer diagnosis who were asked for extra information once prior authorisation – already a cumbersome process – had been sought. This led to additional consultations, considerations, and collection of information. By the time the patients had received a decision, their disease had progressed to the point where only palliative care could be administered.

d) Prescriptions are not accepted in other countries

Despite the possibility of having your prescription recognised by a pharmacist in an EU country, in practice, this is not working. Prescription names vary between countries, and doctors prefer the language they know or assume that a patient is being treated elsewhere and therefore do not look at other medications taken in conjunction that could be detrimental to their health.

To work around this, clinicians, therefore, have to communicate between countries to issue a prescription in the country where the patient is physically located, requiring the patients to pay a larger team of physicians.

e) Variation between Member States

The legal certainty of accessing healthcare in another EU country is very weak, leaving the granting of patients' rights extremely unpredictable and varies significantly from one Member State to another. Prior authorisation varies significantly, meaning some patients will face unjustified obstacles from national authorities or health insurers responsible for prior authorisation. The differences in governance and financing of the National Contact Points result in a considerable variation of services to citizens.

3. European Reference Networks: one of the greatest achievements of the community, but working to an adjusted ambition

European Reference Networks (ERNs) are a crucial output of the Cross-border Healthcare Directive, with its adoption creating their legal basis. Indeed, ERNs are one of the greatest achievements that the rare disease community as a whole has ever accomplished. With more than 1,500 expert teams expected to participate in the 24 Networks by 2021, ERNs bring together a significant capacity of human resources, clinical expertise and knowledge, that offers all stakeholders a 'once-in-a-lifetime' opportunity to leverage this scale of commitment and drive forward improvements in healthcare systems capacities and capabilities in the field of rare diseases.

Under Article 12 of the Cross-border Healthcare Directive, ERNs are required to work towards a number of objectives to ultimately improve the prevention, diagnosis, treatment, care and research within the field of rare diseases in Europe. Progress is being made incrementally, as the Networks are now formed and experimenting with new ways of cooperating cross-borders.

However, in these four years, the Networks have had to adjust their ambition to the level of investment that they have received. Likewise, the lack of an action plan by each Member State to formally connect the Networks with their own health systems as well as the lack of a full EU service model definition for the ERNs virtual expert advice has substantially limited their impact. As a result, ERNs have been unable to fulfil a critical part of the mandate as set out in Article 12 of the Cross-Border Healthcare Directive. In particular:

- **ERNs have been unable to deliver high-quality, accessible and cost-effective healthcare for all rare disease patients**
- **ERNs have been unable to help Member States with insufficient numbers of patients or expertise to provide highly specialised services of high quality**

EURORDIS has set out its detailed analysis, vision and recommendations for a mature ERN ecosystem, where ERNs can deliver on their promise to "Share, care, cure".

B) Recommendations

Cross-border healthcare is a system that needs to work for people living with a rare disease. As only 6% of rare diseases have a treatment, they already face inequity in access to medicines. Where an option exists in another country, they should not have to overcome obstacles to receive it. This is only going to become increasingly important as more Advanced Therapeutic Medicinal Products (ATMPs), administered in one highly specialised centre in one country, are approved. Patients will have no choice but to travel to access a life-saving treatment.

We trust that the evaluation of the Directive on Patient's Rights in Cross-border Healthcare will be the opportunity to reconsider, and redesign where necessary, the policy and legal framework for access to cross-border access to healthcare, which is essential to truly leave no one behind.

EURORDIS-Rare Diseases Europe proposes the following recommendations to ensure that the Cross-border Healthcare Directive is delivering for all people living with a rare disease in Europe:

1. Improve the process of accessing healthcare in another country

1.1. ***Improve information on accessing care in another country***. Clear and easily accessible information should be made available on government/National Contact Point (NCP) websites and be publicised. Legal pathways and options should be explained. NCPs should support patients needing to travel abroad in providing all necessary information (treatment options, centres where they are administered, costs, reimbursement rates, etc.) to help them define a care pathway and make an informed choice.

1.2. ***Establish and acknowledge clear mechanisms for National Authorities entrusted to give prior authorisation to access reliable sources of information for rare diseases***, to enable them to make informed decisions on granting prior authorisation (e.g. Centres of Expertise for rare diseases European Reference Networks or their national members.). Such mechanisms are particularly important for rare diseases, on which information is scarce by definition.

1.3. ***Review rules on prior authorisation*** following a thorough mapping of the way they are implemented in Member States. Ensure that the prior authorisation process reflects the spirit of the legislation, is not an impediment to the granting of patient's rights and is proportional, i.e. not containing unrealistic requests for information.

1.4. ***Define EU-wide guidelines for competent national authorities*** on how to navigate the dual system, with clear roles and responsibilities for national authorities (insurers, payers, etc.). The guidelines will provide details and guidance on how to apply the existing rules in the most favourable way for the patient seeking care abroad, keeping in mind that "all patients are treated equitably on the basis of their healthcare needs rather than on the basis of their Member State of affiliation" (Dir. 2011/24/EU, recital 20).

1.5. ***Specific solutions for advanced therapies*****.** Given the scientific advances in the area of ATMPs for rare diseases, in order to ensure there are no barriers to the mobility of patients to specialised treatment centres for ATMPs, clear guidance must be issued by the European Commission on what legislative instruments apply in the case of ATMPs after centralised EMA approval and being administered in single or few treatment centres across Europe.

1.6. ***Remove the upfront payment of treatments by patients,*** considering soft loans for cross-border treatments, direct payment for low-income patients, guidelines for banks on the reimbursement procedure. The possibility of creating an 'EU guarantee' for upfront payment for patients travelling abroad should also be explored.

1.7. ***Demands for cross-border care should be considered valid by default when signed by a specialised doctor in a Centre of Expertise*** (e.g. prescription of treatment, diagnosis, consultation hospitalisation, surgery…).

1.8. ***Give ERNs a legal mandate to perform an advisory role in the decision-making process on requests for face to face cross-border healthcare,*** creating a coordination mechanism to embed the ERNs and/or their centres into the National Contact Points.

1.9. ***Ensure timely access to relevant patients' health data for the purpose of cross-border healthcare***: electronic health records electronically should be made accessible, and transfer of patients' health data must be facilitated across healthcare systems, supported by interoperable solutions and platforms. The above should be central elements of the upcoming European Health Data Space proposal.

1.10. ***Digitalise prescriptions in an agreed international format*** that is accepted across Europe and is compatible with the rules of all countries (EU+EEA).

1.11. ***Addressing accessibility of treatments through a single European pathway.*** The cost of therapies, especially advanced therapies for rare diseases, has been pointed as a deterring factor for granting authorisation for care abroad. For many years, EURORDIS has suggested addressing accessibility and affordability of rare disease treatments, both within and beyond national borders, via a single European pathway with a table of negotiation that allows for payers to discuss the economic value of treatments, rules for access and common agreements on outcome-based payments or other types of payment mechanisms; and access to European funding for post-authorisation data gathering. Such solutions are recommended for advanced and transformative therapies for the rarest diseases by the European Commission co-funded Rare 2030 Foresight Study and are in line with the 2018 EURORDIS 'Access Paper'.

2. Ensure the sustainability and consolidation of the ERNs

2.1. ***Integrate ERNs into national health systems*****.** The Networks require a mature and enabling environment at a national level to evolve from their early stages to a mature and consolidated structure. All Member States need to take urgent action to implement the integration statement issued by the Board Member States in 2019.[2]

2.2. ***Greater investment*** to enable ERNs to operate more flexibly and effectively and to receive funding from different sources, including 'external' sources such as industry and private donors.

2.3. ***Coordinated public procurement model to cater for ERNs services*** based on a structured collaboration with ERN Coordinators, hospital managers and national health authorities to plan the ERNs procurement strategy, develop tenders' specifications and joint follow-up of the contracts in terms of milestones and deliverables.

2.4. ***A fully defined service model for ERNs expert virtual advice to scale up service provision***. For the Networks to scale up this service, it is urgent to define a reimbursement model; design clear, publicly available referral pathways in each country; formally integrate ERNs' referral in the national referral protocols; agree on clinical governance standards for quality assurance; integrate the service into the hospitals' clinical workflow and IT infrastructure and train the healthcare workforce as well as their National Contact Points on the referral process.

[2] https://ec.europa.eu/health/sites/default/files/ern/docs/integration_healthcaresystems_en.pdf

3. A whole-system response to meet the rare disease patient population's evolving needs to overcome fragmentation and inequalities in access.

We need highways that interconnect ERNs with the national health systems under a European Health Union for rare diseases with clear lines of sight from frontline services to expert centres at both a national and European level, enabling the expertise to travel but also, based on incidence and prevalence levels, the centralisation of care under leading expert centres recognised as 'rare disease lighthouses'. The EU should have greater competencies in health to develop and manage a European highly specialised healthcare system, including workforce planning, where ERNs would be the operational arm for delivery.[3]

3.1. ***Fully defined basket of cross-border healthcare services*****,** including Face to Face cross-border healthcare consultations but also virtual expert advice, and eventually virtual cross-border consultations, with clear referral pathways integrated into the national referral protocols, clinical governance standards for each service and an IT infrastructure that facilitates timely access to health data for cross-border healthcare purposes.

3.2. ***Joint procurement to address the inequities patients face in accessing quality highly specialised healthcare in the EU.*** Only reinforced EU cooperation and joint procurement will allow European countries to organise healthcare services for rarer diseases on optimal population size to ensure safe and sustainable services are accessible for all. This should translate into joint planning, commissioning and contracting from designated European Centres of Expertise, recognise as "rare disease lighthouses" to cater highly specialised interventions where there is either an annual caseload of <500 EU-wide or <250 cases at a national level. A similar logic applies to the procurement of Advanced Therapeutic Medicinal Products, where eventually, only a few centres in Europe will be accredited to deliver these therapies.

4. Europe's Action Plan for Rare Diseases following the review of European Rare Diseases Strategy by 2023, as per the European Court of Auditors' special report no 7/2019

The review of the cross-border healthcare directive cannot be decoupled from the overall review of the rare disease strategy. The revision of cross-border healthcare rules needs to be supported and integrated by improvements across different areas: diagnosis, data, research, integrated care, treatment. An overarching, cohesive framework is, therefore, necessary to pull this all together, as recommended by the European Commission co-funded Rare 2030 Foresight Study. In their report on the Cross-border Healthcare Directive in 2019 (special report n°07/19), the European Court of Auditors also suggested reviewing the entire rare disease strategy.

EURORDIS calls for Europe's Action Plan for Rare Diseases to turn this review into a proposal of concrete actions with uniquely high community added value to address the high unmet needs of people living with a rare disease in Europe. This is needed to:

- Address the remaining unmet needs and inequities all along the patient journey in accessing a diagnosis, treatments and care, leaving people living with a rare disease marginalised in society;
- Keep pace with new technologies, new values and new expectations of Europe's citizens and give a new focus to national rare disease plans and strategies;

[3] https://www.rare2030.eu/

- Sustain the European Commission's strategic approach in addressing a distinctive domain of high European added value and bring together existing and upcoming actions, across countries, across sectors and policy areas, and across the rare disease pathway, where the EU can add the most value under one interconnected framework.

Europe's Action Plan for Rare Diseases would address these gaps while incorporating conclusions of parallel evaluations and revisions of legislation, including the Cross-Border Healthcare Directive, legislation on Orphan Medicinal Products Regulation, Paediatric Use of Medicines and new Commission initiatives such as the European Health Data Space and the European Pillar of Social Rights.