

# Achieving Full Reach: a European Health Union for Highly Specialised Healthcare

## Working Document

### 1. PROBLEM STATEMENT

Healthcare services are best organised as close to the population as possible, where decision makers are best positioned to understand and meet local population needs. This principle also holds true for rare diseases, however given the small patient population size, healthcare services for rare diseases are usually more efficiently organised either at national or even supranational level where there is a sufficient number of cases to understand the associated needs.

In fact, **different infrastructures and organisational arrangements are required to address rare disease patients' healthcare needs**, depending on the interplay of three factors:

1. Prevalence and incidence rate of a given disease or a given intervention;
2. Number of expert teams available to provide the service;
3. Financial implications for countries to arrange services at a national or sub-national level and the innovative capacity of individual health systems.

Recent estimates indicate that almost all (98%) of rare patients are affected by a small group of high prevalent rare diseases (<390), whereas an estimated 2% of the rare disease patient population is affected by a large number of very low prevalent diseases (c. 4.000 diseases), affecting less than 9 persons in 1.000.000<sup>1</sup>.

In most large and medium-size EU countries, Expert Centres will manage a big enough caseload to develop the expertise locally and to meet the needs of the population affected by <390 rare diseases, either at regional or national level. However, for the less frequent and lower prevalent diseases, even the bigger European countries need to arrange cross-border collaboration at a pan-European level or even internationally in order to meet the needs of this patient population.

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<sup>1</sup> Stéphanie Nguengang Wakap et al., "Estimating Cumulative Point Prevalence of Rare Diseases: Analysis of the Orphanet Database," *European Journal of Human Genetics* 28 (2020): 165–73, <https://doi.org/10.1038/s41431-019-0508-0>.

European Reference Networks (ERNs) offer virtual expert advice and knowledge sharing on rare and complex conditions, as well as ongoing professional education and training. In this way, these networks are well placed to support the development of national health systems' competencies to diagnose and treat persons living with highly prevalent rare diseases and to complement national capacities to care for people with the less prevalent conditions via the experts' advice virtual panels.

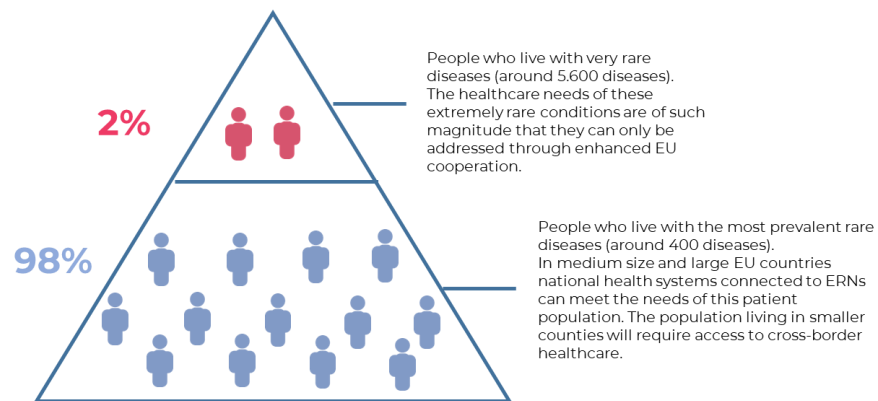


Figure 1. Rare Disease Healthcare population pyramid. Source: Own elaboration. Data from “Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database”<sup>2</sup>.

Secondly, when it comes to highly specialised healthcare services, certain innovative treatments and some complex and rare surgical procedures are provided with adequate safeguards only in a few European centres. In these cases, referring all patients to these centres would be safer, prevent severe postoperative complications, contribute to better health outcomes and quality of life. To achieve these outcomes, the care pathway should be clear, referrals timely, service specifications comprehensive, Expert and the local teams should be well coordinated and the reimbursement tariffs and process should protect people from incurring in out-of-pocket expenses.

Thirdly, the financial implications for hospitals and health systems related to the organisation of highly specialised healthcare services also deserve careful consideration. For example, several challenges may affect the financial feasibility of certain highly specialised surgical services, including lack of economies of scale due to the low-frequency and high complexity of the procedures, stringent quality assurance measures and the imperative of maintaining the competencies of the multidisciplinary specialised surgical team over time. Additionally, national tariffs may lag behind in terms of mapping the real costs of administering the procedures, straining hospital budgets<sup>3</sup>. Obviously, these challenges are further compounded when the number of procedures performed each year in a given country is extremely low.

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<sup>2</sup> Nguengang Wakap et al.

<sup>3</sup> RW Westerman, SB Mostofi, and R Slack, “Complex Surgical Procedures: How Much Are They Costing Your Hospital?,” *The Bulletin of the Royal College of Surgeons of England* 93, no. 3 (March 1, 2011): 98–100, <https://doi.org/10.1308/147363511X558100>.

These financial considerations, alongside other factors such as evidence of a volume-outcome relationship, when available, quality of care and cost savings, explain the decisions that have led to centralise the provision of complex surgical procedures in different countries<sup>4</sup>.

Similarly, administering innovative, gene and cell, therapies poses significant financial challenges for health systems and hospitals, that go beyond the affordability of the therapies<sup>5</sup>. Take, for instance, the AADC gene therapy, where the complex diagnostic and therapeutic procedures demand extensive structural and professional requirements. This includes prehospital, inpatient, and posthospital care managed by a specialised multidisciplinary team, alongside an in-house pharmacy equipped to handle the product safely and ensure timely delivery to the surgical team<sup>6</sup>. Beyond these operational costs, hospitals are exposed to various financial risks such as the upfront investment in infrastructure or the mismatch between national tariffs and the level of clinical intervention required to deliver these therapies<sup>7</sup>. Moreover, if the therapy represents a novel treatment rather than a substitution, it offers limited opportunities to optimise resources and generate savings from existing services<sup>8</sup>.

**All these challenges can only be effectively addressed by a more ambitious EU collective action**, encompassing joint commissioning<sup>9</sup> as well as workforce planning. Specifically, enhanced cooperation is required when one or more of the following three factors emerge:

1. Lack of sufficient cases to adequately assess patient needs at national level: very small and infrequent affected patient population results in lack of sufficient cases to understand needs at national level or through devolved sub-national commissioning structures.
2. Safety risks & poor clinical viability: There are very few expert teams able to provide the service across the EU with the sufficient experience to provide a safe and sustainable service.
3. Limited financial capacity of individual countries. Individual health systems lack the financial and innovation capacities required to provide and maintain high-cost, highly specialised and innovative healthcare services, including workforce training & adequate replacement rates.

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<sup>4</sup> René Vonlanthen et al., "Toward a Consensus on Centralization in Surgery," *Annals of Surgery* 268, no. 5 (November 2018): 712–24, <https://doi.org/10.1097/SLA.0000000000002965>.

<sup>5</sup> "RARE IMPACT Phase 2 Workstream 3: Methods & Models for the Selection, Assessment and Approval of Advanced Therapy Medicinal Products (ATMPs) ATMP Treatment Centres," December 2021.

<sup>6</sup> Agathe Roubertie et al., "Gene Therapy for Aromatic L-amino Acid Decarboxylase Deficiency: Requirements for Safe Application and Knowledge-generating Follow-up," *Journal of Inherited Metabolic Disease*, July 17, 2023, <https://doi.org/10.1002/jimd.12649>.

<sup>7</sup> "RARE IMPACT Phase 2 Workstream 3: Methods & Models for the Selection, Assessment and Approval of Advanced Therapy Medicinal Products (ATMPs) ATMP Treatment Centres."

<sup>8</sup> Andrew R. Champion et al., "Managing Access to Advanced Therapy Medicinal Products: Challenges for NHS Wales," *British Journal of Clinical Pharmacology* 87, no. 6 (June 3, 2021): 2444–49, <https://doi.org/10.1111/bcp.14286>.

<sup>9</sup> The term "commissioning" refers to the process by which health and care services are planned, purchased and monitored (Source: [What is Commissioning and how is it changing?](#). The Kings Fund, July 2023.)

EU action in this domain is not only a moral imperative to guarantee equal **access** and universal health coverage for people living with very rare conditions, but it also represents an opportunity for the EU to be at the forefront of **healthcare innovation**.

Following this reasoning, it is recommended to take collective EU action to organise healthcare services and workforce planning to meet the needs of:

- people living with a disease that affects less than 500 people across the EU;
- people that require complex interventions where the number of procedures performed each year is below 500 across the EU;
- people that require innovative treatments that are typically delivered in a few centres across the EU.

Central to this proposal would be to integrate planning of highly specialised healthcare services with other decision-making processes linked to contracting, delivery and workforce planning. Practically speaking, it would mean the creation of a **European Union for highly specialised healthcare services**.

Such an approach is also in line with current political trends that call for enhanced EU cooperation in health and healthcare, greater competitiveness, and a broad understanding of the European Health Union mission<sup>10</sup>. Moreover, it would help to materialise the implementation of Principle 16 of the [European Pillar of Social Rights](#) “everyone has the right to timely access to affordable, preventive and curative healthcare of good quality”, and **make the European Health Union a tangible reality**.

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<sup>10</sup> Frank Vandenbroucke, “The Promise of a European Health Union,” *Journal of the European Observatory on Health Systems and Policies EUROHEALTH* 29, no. 3 (2023), <http://www.healthobservatory.euhttps://www.lse.ac.uk/lse-health>.

## 2. ACHIEVING FULL REACH

Only through greater solidarity and enhanced cooperation, EU countries will be able to commission certain highly specialised healthcare services on an optimal population size to ensure safe, accessible and sustainable highly specialised healthcare for all.

Achieving full reach will require establishing a European commissioning system to plan, fund and contract from a few European Expert Centres (“European Rare Disease Lighthouses”), connected to European and international networks, the delivery of highly specialised interventions and treatments for:

- people living with a rare disease where there is <500 cases EU-wide;
- people requiring complex interventions where the annual caseload is <500 EU-wide;
- people requiring innovative treatments that are only delivered in a few centres in the EU.

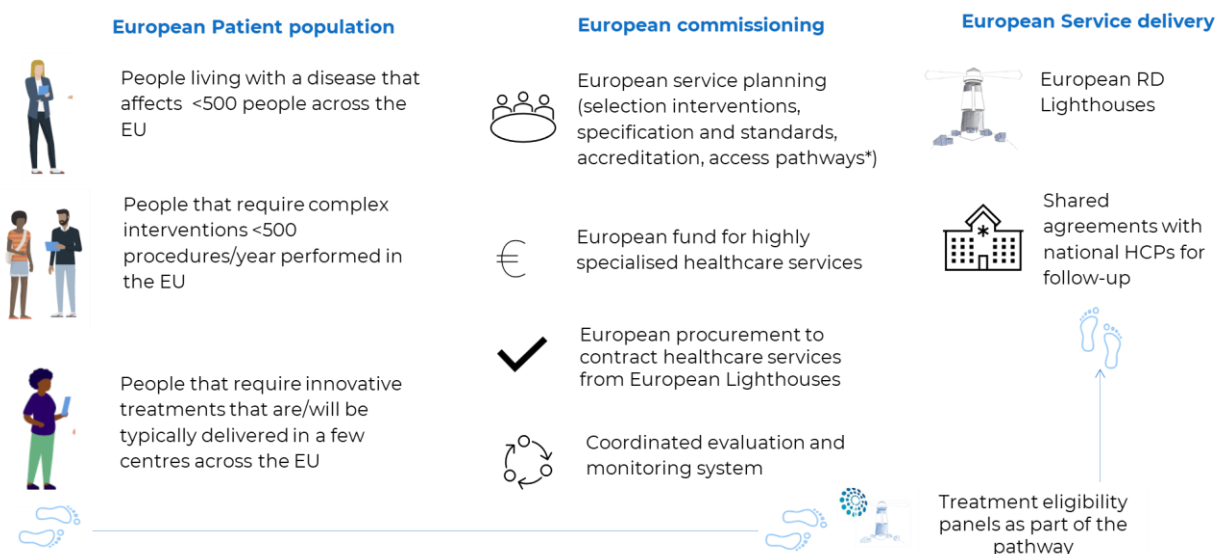


Figure 2. A European Health Union for Highly Specialised Healthcare Services

Commissioning of highly specialised healthcare services at EU level would require articulating the right mechanisms to ensure **equal access**, given the small number of Expert Centres and having shared agreements with local providers to ensure continuity of care. Importantly, emotional and practical support, (housing, translation, schooling, etc) should be bundled with the healthcare services contracted through this new mechanism. To address these and other relevant aspects, commissioning could be organised following a cyclical set of activities structured under 3 phases:

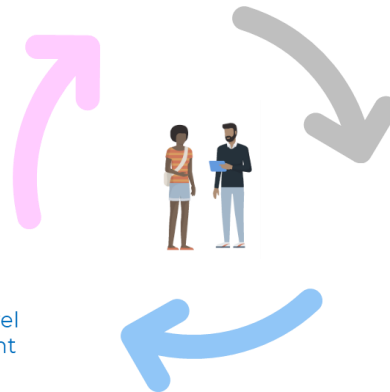
- Strategic planning, including the access policies and referral pathway
- Procurement and Implementation
- Monitoring and Evaluation, to inform re-programming and workforce planning.

### 3. COORDINATED EVALUATION AND MONITORING OF SERVICE DELIVERY AND OUTCOMES

### 2. PROCUREMENT AND CONTRACTING OF SERVICES

Including supporting families to travel and cover expenses during treatment

Recommendations on workforce planning, and also training of health professionals at local level where needed, could be incorporated to the tenders, or inform other EU initiatives if this is more appropriate.



### 1. STRATEGIC PLANNING

- Selection of interventions/treatments, needs assessment and priority setting
- Agreeing on service specifications and standards of care for EU Rare Disease Lighthouses Centres – including criteria for hospitals' general services & structure as well as for facilities to provide emotional and practical support to families
- Developing access policies per service (care pathway, inclusion/exclusion criteria, etc). As part of the care pathway, shared care agreements with local health services will be captured to coordinate care and follow-up.

Figure 3. EU end-to-end approach to commission and deliver highly specialised healthcare services.  
Source: adapted from the Planning Spiral <sup>11</sup>

Greater EU solidarity and collective action in this domain will contribute to:

1. Universal health coverage, fairness and equity by ensuring timely access to cross-border highly specialised healthcare.
2. Improved health outcomes by ensuring timely access to the experts, technologies and treatments needed as well as adequate follow-up.
3. Position the EU at the forefront of healthcare innovation and make the European Health Union a tangible reality.
4. Secure a sustainable workforce by integrating service and workforce planning, allowing for long-term planning of workforce needs, addressing training needs, workforce shortages and guaranteeing an adequate replacement rate.
5. Enhance the responsiveness of healthcare services that will be designed based on a better understanding of the patient population needs.

<sup>11</sup> Andrew Green, *An Introduction to Health Planning for Developing Health Systems, An Introduction to Health Planning for Developing Health Systems*, 2009, <https://doi.org/10.1093/acprof:oso/9780198571346.001.0001>.