

EN

E-000886/2025

Answer given by Mr Várhelyi
on behalf of the European Commission
(26.6.2025)

Challenges around orphan drugs are manifold. Medical requirements of patients with rare diseases (RD) are not adequately met, affordability of products is a growing challenge for individuals and health systems, and the existing regulatory framework does not sufficiently cater for innovation.

The Commission has taken strong actions on RD notably with the European Reference Networks (ERN), making expertise travel and disseminate. To maximise the benefits already offered by the ERNs, their integration into national healthcare systems is needed. A Joint Action is underway until 2027 to achieve this goal¹, providing a clearer picture of the RD landscape in the EU and informing future decisions.

The ongoing revision of pharmaceutical legislation² promotes the development of treatments for RD via regulatory incentives and enhanced scientific support from the European Medicines Agency. It focuses on areas where patients have no treatment options³, facilitating faster access to innovative medicines and affordable medicines.

The Commission supports ORPHANET's work on RD nomenclature and high-quality information on RD codification⁴.

ERNs for RD⁵ have conducted over 4,000 virtual panels on RD patients since 2017.

Collaborative research on RD has been a Commission priority⁶ since 2006, when it first coordinated research funding with national agencies⁷. Recently it launched its fourth successor, the European Partnership ERDERA⁸, and at global level⁹. The Innovative Health Initiative also supports RD research¹⁰. Gaps are tackled from various angles: treatments¹¹, diagnoses¹², newborn screening¹³, Health Technology Assessment¹⁴, specific regulatory needs¹⁵, inclusion of RD patients, and through relevant projects¹⁶.

¹ JARDIN Joint Action <https://jardin-ern.eu/>.

² Reform of the EU pharmaceutical legislation https://health.ec.europa.eu/medicinal-products/pharmaceutical-strategy-europe/reform-eu-pharmaceutical-legislation_en.

³ EU Pharmaceutical reform: Steering innovation to address unmet medical needs https://health.ec.europa.eu/document/download/93406a57-1be5-4005-a63f-de5fb1de4748_en?filename=factsheet_umn_en.pdf.

⁴ Nomenclature: ORPHAcodes <https://www.orpha.net/>.

⁵ European Reference Networks https://health.ec.europa.eu/rare-diseases-and-european-reference-networks/european-reference-networks_en.

⁶ https://research-and-innovation.ec.europa.eu/research-area/health/rare-diseases_en.

⁷ E-Rare <https://www.era-learn.eu/network-information/networks/e-rare>.

⁸ <https://cordis.europa.eu/project/id/101156595>.

⁹ Through the International Rare Diseases Research Consortium <https://irdirc.org/who-we-are-2/>.

¹⁰ <https://www.ih.europa.eu/projects-results/project-factsheets/realised>; <https://www.ih.europa.eu/projects-results/project-factsheets/ardat> and more.

¹¹ https://cordis.europa.eu/programme/id/HORIZON_HORIZON-HLTH-2022-DISEASE-06-04-two-stage/en; https://cordis.europa.eu/programme/id/H2020_PHC-14-2015/en and previous calls.

¹² https://cordis.europa.eu/programme/id/H2020_SC1-PM-03-2017/en and previous calls.

EU funding enables better treatment for RD patients. While a financial solidarity instrument is not currently in the pipeline, in 2023 the Commission funded via EU4Health the ERNs for another four years with EUR 77.4 million. Besides, Directive 2011/24/EU¹⁷ supports cross-border healthcare, including for RD. Its evaluation in 2027 will guide next steps.

¹³ <https://www.ih.europa.eu/projects-results/project-factsheets/screen4care>.

¹⁴ <https://cordis.europa.eu/project/id/825162>.

¹⁵ https://cordis.europa.eu/programme/id/HORIZON_HORIZON-HLTH-2023-IND-06-04/en and previous calls.

¹⁶ SOLVE-RD <https://solve-rd.eu/>; ImmunAID <https://cordis.europa.eu/project/id/779295/reporting>;

Connect4Children <https://connect4children.org/>; Screen4Care <https://screen4care.eu/>; REMEDI4All

<https://remedi4all.org/>; REPO4EU <https://repo4.eu/>.

¹⁷ <http://data.europa.eu/eli/dir/2011/24/oj>.