

EN
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Answer given by Mr Várhelyi
on behalf of the European Commission
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While Member States are responsible for the organisation of health services and medical care, the Commission has taken several steps to facilitate access for rare disease patients to effective diagnostics and therapies.

The Commission supports the European Reference Networks (ERN)¹; cross-border networks that bring together European hospital centres of expertise and reference to tackle rare diseases. ERN EYE² focuses on rare eye diseases.

The Healthier Together initiative³ supports Member States in addressing NCDs such as geographic atrophy. The Commission supports collaborative action and the sharing of best practices⁴ with EU4Health funding⁵. The Expert Group on Public Health⁶ provides a forum for exchange with the Member States on NCDs.

Following an application, the European Medicines Agency (EMA) recommends whether a medicine can be authorised based on the assessment of its benefit risk balance. The EU pharmaceutical legislation reform⁷ will make the EU system more attractive, increase competitiveness, and reduce red tape contributing to savings of EUR 300 million annually for industry and Member States. Simpler procedures and faster authorisation times will be introduced. Better use of real-world evidence and expedited pathways will accelerate access to new treatments. Regulatory sandboxes and adapted frameworks will enable early testing of innovative therapies, positioning the EU as a global innovation leader.

The EU supports research on advanced therapies through the EU Research and Innovation Framework Programmes. It has funded 34 collaborative projects with EUR 220 million in Horizon Europe⁸. Remaining calls in Horizon Europe under Work programmes 2025 and 2026/2027 will also target advanced therapy approaches.

¹ Rare diseases and European Reference Networks https://health.ec.europa.eu/rare-diseases-and-european-reference-networks_en

² ERN-EYE, a European Reference Network dedicated to rare eye diseases <https://www.ern-eye.eu/>

³ https://health.ec.europa.eu/non-communicable-diseases/healthier-together-eu-non-communicable-diseases-initiative_en

⁴ <https://webgate.ec.europa.eu/dyna/bp-portal/>

⁵ https://health.ec.europa.eu/funding/eu4health-programme-2021-2027-vision-healthier-european-union_en

⁶ https://health.ec.europa.eu/non-communicable-diseases/expert-group-public-health_en

⁷ https://health.ec.europa.eu/medicinal-products/pharmaceutical-strategy-europe/reform-eu-pharmaceutical-legislation_en

⁸ https://research-and-innovation.ec.europa.eu/funding/funding-opportunities/funding-programmes-and-open-calls/horizon-europe_en