

(English version)

**Question for written answer E-002031/25
to the Commission**

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(21 May 2025)

Subject: Development of life-saving medicines for rare diseases and bringing them to market

The process of researching, developing and bringing life-saving drugs to market is hampered by significant financial costs, which often hinder access to innovative treatments, particularly for rare diseases.

According to numerous industry experts and as documented in recent investigations, a significant number of promising drugs never reach the commercialisation stage because developers lack the funds needed to complete clinical trials, particularly for the transition phase from laboratory research to large-scale testing (known as ‘the valley of death’).

Despite efforts to boost research into medicines for rare diseases, the number of medicines developed for rare diseases remains too low relative to patient numbers and therapeutic needs: there are more than 7 000 rare diseases and millions of Europeans have them.

The Commission has a duty to ensure that everyone in the European Union has access to proper care, including life-saving therapies for rare diseases, through inclusive and sustainable health policies.

In light of the above:

1. What does the Commission have to say about the difficulties faced when researching and developing life-saving medicines?
2. Could it inform Parliament how it plans to tackle this issue?

Submitted: 21.5.2025

Answer given by Ms Zaharieva on behalf of the European Commission
(24 September 2025)

The Commission acknowledges the challenges in developing new treatments for rare diseases (RDs). The EU regulation on orphan medicinal products, (EC) No 141/2000 ⁽¹⁾ catalysed the development of medicines for people living with a rare disease, as reported in the public registry of orphan designated products in the EU ⁽²⁾.

In an effort to tackle this challenge, the Commission supports research and innovation (R&I) on rare diseases (RDs) ⁽³⁾. Over the past 25 years, through the EU’s framework programs for R&I, it has allocated more than EUR 5 billion to single and multi-partner RD research projects ⁽⁴⁾.

Notably, the development of new RD treatments has been the focus of several EC research consortia, whose work led to new RD therapies ⁽⁵⁾, ⁽⁶⁾ approved in the EU.

Under Horizon Europe ⁽⁷⁾ (2021-2027), the Health cluster ⁽⁸⁾ continues to support RD research, through collaborative projects developing RD therapies ⁽⁹⁾ and optimising the RD clinical trials through public-private consortia ⁽¹⁰⁾.

The flagship initiative, the European Partnership on Rare Diseases, ERDERA ⁽¹¹⁾, that started in 2024 as a co-funded Partnership with EU Member States, provides further research funding opportunities, such under its 2025 ⁽¹²⁾ call dedicated to pre-clinical therapy studies for RDs.

⁽¹⁾ Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, (OJ L 18, 22.1.2000, p. 1.1, ELI: <http://data.europa.eu/eli/reg/2000/141/oj>).

⁽²⁾ Union Register of medicinal products — Public health — European Commission: https://ec.europa.eu/health/documents/community-register/html/reg_od_act.htm?sort=a.

⁽³⁾ EU-Factsheet (edition Feb. 2025) ‘Collaboration: A key to unlock the challenges of rare diseases research’: <https://op.europa.eu/en/web/eu-law-and-publications/publication-detail/-/publication/c85cfbb5-f58f-11ef-b7db-01aa75ed71a1/language-en>.

⁽⁴⁾ More info can be found via searches at cordis dissemination database: <https://cordis.europa.eu/projects>.

⁽⁵⁾ ALPHA-MAN (2010-2014) project developed a new orphan medicine for the ultra-rare disease alpha-mannosidosis: <https://cordis.europa.eu/project/id/261331/news>.

⁽⁶⁾ VISION-DMD (<https://cordis.europa.eu/project/id/667078>) (2016-2021) project led to a new treatment of Duchenne Muscular Dystrophy for patients from the age of 4 that was approved in the EU in December 2023.

⁽⁷⁾ https://research-and-innovation.ec.europa.eu/funding/funding-opportunities/funding-programmes-and-open-calls/horizon-europe_en.

⁽⁸⁾ https://research-and-innovation.ec.europa.eu/funding/funding-opportunities/funding-programmes-and-open-calls/horizon-europe/cluster-1-health_en.

⁽⁹⁾ https://cordis.europa.eu/programme/id/HORIZON_HORIZON-HLTH-2022-DISEASE-06-04-two-stage/en.

⁽¹⁰⁾ Such as RealiseD under the Innovative Health Initiative <https://cordis.europa.eu/project/id/101165912>.

⁽¹¹⁾ <https://erdera.org/> (2024-2031).

⁽¹²⁾ <https://erdera.org/funding/>.

Moreover, future opportunities addressing clinical research, are envisaged under the recently adopted Life Science Strategy ⁽¹³⁾ (LSS) that sets out a coordinated approach across the entire life sciences value chain.

LSS introduces a new set of actions, including flagship initiatives, such as an Investment Plan for multi-country clinical trials to boost research, innovation and market access of new medicines.

⁽¹³⁾ COM/2025/525 final 'Choose Europe for life sciences: A strategy to position the EU as the world's most attractive place for life sciences by 2030'.