

**Question for written answer E-002693/2025
to the Commission**
Rule 144
Valentina Palmisano (The Left)

Subject: Equal access to orphan drugs for compassionate under the EU regulatory framework

Article 83 of Regulation (EC) No 726/2004 allows for the compassionate use of medicinal products not authorised for serious or life-threatening diseases, in the absence of alternative treatments. However, its application is left up to the Member States, which has created significant disparities in access across Europe.

A recent case highlights this problem: an Italian patient with recurrent and refractory Philadelphia positive acute lymphoblastic leukaemia (Ph+ ALL) urgently needs olverembatinib, a third generation TKI inhibitor approved in China and designated an orphan drug by the European Medicines Agency (EMA) in 2021. The patient has exhausted all the treatment options available under the national health system, but access to the drug is being hampered by the lack of harmonised procedures and an uncertain timeframe.

In the light of Commission proposal COM(2023)192, can the Commission answer the following questions:

1. Will it propose binding criteria, timeframes and procedural standards at EU level to harmonise compassionate use programmes in the Member States, as part of the ongoing pharmaceutical legislation reform?
2. Will it impose obligations on marketing authorisation holders or orphan designation holders to make medicines available through compassionate use programmes or early access in emergency clinical scenarios, where there are no alternatives?
3. Will it reinforce the role and mandate of the Committee for Medicinal Products for Human Use (CHMP) in the timely delivery of public scientific opinions on unauthorised medicines eligible for compassionate use, especially in rare and orphan diseases?

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