

**Question for written answer E-002234/2024
to the Commission**

Rule 144

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Subject: Placing Ryplazim on the market in Europe

Severe hypoplasminogenemia (HPG), or plasminogen deficiency type 1 (PLGD-1) is a rare systemic disease characterised by abnormal extracellular fibrinolysis. It affects 0.02 in 10 000 people in the EU, usually manifesting in early childhood.

There exists only one effective treatment, now available in the form of Ryplazim, an experimental drug already approved in 2019 by the US Food and Drug Administration and available on the American market since May 2024.

The medicine was so successful that the manufacturer Kedrion received the prestigious Industry Innovation Award from the American National Organization for Rare Diseases (NORD).

However, the adoption of Ryplazim has not yet been authorised by the European Medicines Agency (EMA), which in 2015 granted orphan designation to ProMetic BioTherapeutics Ltd for human plasminogen with opinion EU/3/15/1511.

Not having the possibility of using Ryplazim in national healthcare systems means outdated therapies will be used instead.

In view of the above:

1. Was the Commission aware of this situation?
2. What concrete steps does the Commission intend to take with regard to the EMA in order to ensure that people suffering from severe hypoplasminogenemia have safe access to life-saving medicines at the dosage necessary for treatment, even if experimental?

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