

EN
E-002234/2024
Answer given by Mr Várhelyi
on behalf of the European Commission
(6.1.2025)

1. The European Medicines Agency (EMA) adopted a positive opinion on 18 June 2015 recommending the granting of the designation of ‘Human plasminogen’ as an orphan medicinal product¹. On 28 July 2015, the orphan designation (EU/3/15/1511) was granted by the Commission to ProMetic BioTherapeutics Ltd, for human plasminogen for the treatment of plasminogen deficiency².

In December 2021, the orphan designation was transferred to Kedrion S.p.A.³. Since that date, no application for marketing authorisation of human plasminogen for the treatment of plasminogen deficiency has been submitted to the EMA by Kedrion S.p.A..

2. In accordance with Regulation (EC) 726/2004⁴, the availability of a new medicine in the EU requires the submission of an application for an authorisation complying with the substantive requirements laid out by Directive 2001/83/EC⁵, a positive outcome of the benefit/risk balance assessment conducted by the EMA and the adoption of a Commission decision granting marketing authorisation.

The EU Orphan Regulation promotes the development of treatments for rare diseases through regulatory incentives⁶. The recent proposal for a reform of the EU general pharmaceutical legislation⁷ focuses especially on areas where there are no treatment options for patients, including rare disease⁸.

¹ https://www.ema.europa.eu/en/documents/orphan-designation/eu3151511-public-summary-opinion-orphan-designation-human-plasminogen-treatment-plasminogen-deficiency_en.pdf

² https://ec.europa.eu/health/documents/community-register/2015/20150728132605/dec_132605_en.pdf

³ https://ec.europa.eu/health/documents/community-register/2021/20211220154340/dec_154340_en.pdf

⁴ <http://data.europa.eu/eli/reg/2004/726/oj>

⁵ <http://data.europa.eu/eli/dir/2001/83/oj>

⁶ eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:32000R0141&from=EN

⁷ Reform of the EU pharmaceutical legislation https://health.ec.europa.eu/medicinal-products/pharmaceutical-strategy-europe/reform-eu-pharmaceutical-legislation_en

⁸ https://health.ec.europa.eu/medicinal-products/pharmaceutical-strategy-europe/reform-eu-pharmaceutical-legislation_en