EUROPEAN
COMMISSION

Brussels, 28.3.2025
C(2025) 2025 final

COMMISSION IMPLEMENTING DECISION

of 28.3.2025

refusing the renewal of the conditional marketing authorisation for the medicinal product for human use "Translarna - ataluren", granted by Decision C(2014)5619 final

(Text with EEA relevance)

(ONLY THE ENGLISH TEXT IS AUTHENTIC)

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THE EUROPEAN COMMISSION,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[1], and in particular Article 10(2) and 14-a thereof,

Having regard to Commission Regulation (EC) No 507/2006 on the conditional marketing authorisation for medicinal products for human use falling within the scope of Regulation (EC) No 726/2004 of the European Parliament and of the Council[2],

Having regard to the application submitted by PTC Therapeutics International Limited, on 3 February 2023, under Article 6(2) of Regulation (EC) No 507/2006, with a view to the annual renewal of the conditional marketing authorisation for the medicinal product "Translarna - ataluren",

Having regard to the opinions of the European Medicines Agency, formulated on 27 June 2024 and on 17 October 2024 by the Committee for Medicinal Products for Human Use (CHMP),

Whereas:

(1) The medicinal product "Translarna - ataluren", entered in the Union Register of Medicinal Products under the number EU/1/13/902 and authorised by Commission Decision C(2014)5619 final of 31 July 2014, does not comply with the requirements of Article 14-a of Regulation (EC) No 726/2004 of the European Parliament and of the Council, and Regulation (EC) No 507/2006, for the reasons set out in the Annex to this Decision.

(2) In particular, the CHMP assessed the benefit-risk of Translarna, taking into account the totality of available data, and concluded that the efficacy was not established in the authorised indication or in any subpopulation of this indication. Given that a favourable benefit-risk balance was not confirmed, the CHMP did not recommend the renewal of the Conditional Marketing Authorisation.

[1] OJ L 136, 30.4.2004, p. 1.
[2] OJ L 92, 30.3.2006, p. 6.

(3) The conditional marketing authorisation should therefore not be renewed.

(4) The Union Register of Medicinal Products should be updated.

(5) During the Standing Committee meeting held on 12 December 2024, several Member States, while agreeing with the negative opinion from the CHMP, expressed concerns about the high unmet medical need of patients suffering from the life-threatening and debilitating Duchenne muscular dystrophy disease and the unique circumstances for those patients being treated with Translarna already for some time, particularly in view of the current absence of alternative therapeutic options.

(6) The European Commission took note that those Member States acknowledged that, independently from the present Decision not to renew the authorisation for Translarna at EU level, it would be possible for Member States at national level to adopt exceptional measures under Articles 117(3) or Article 5(1) of Directive 2001/83/EC during a transitional period, as it is recognised that some patients have been receiving treatment with Translarna for some time and that a close collaboration between those patients, their caregivers and healthcare professionals is essential, in order to discuss the possible treatment options.

(7) The measures provided for in this Decision are in accordance with the opinion of the Standing Committee on Medicinal Products for Human Use,

HAS ADOPTED THIS DECISION:

Article 1

The renewal of the conditional marketing authorisation for the medicinal product "Translarna - ataluren" is refused on the basis of the scientific conclusions set out in the Annex to this Decision.

Article 2

This Decision is addressed to PTC Therapeutics International Limited, Unit 1, 52-55 Sir John Rogerson's Quay, Dublin 2, D02 NA07, Ireland.

Done at Brussels, 28.3.2025

For the Commission
Sandra GALLINA
Director-General

CERTIFIED COPY
For the Secretary-General

Martine DEPREZ
Director
Decision-making & Collegiality
EUROPEAN COMMISSION