EUROPEAN
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# COMMISSION IMPLEMENTING DECISION

**of 22.11.2018**

**granting marketing authorisation under Regulation (EC) No 726/2004 of the European Parliament and of the Council for "Luxturna - voretigene neparvovec", an orphan medicinal product for human use**

(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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[1], and in particular Article 10(2) thereof,

Having regard to Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products[2], and in particular Article 5(12) thereof,

Having regard to Regulation (EC) No 1394/2007 of the European Parliament and of the Council of 13 November 2007 on advanced therapy medicinal products and amending Directive 2001/83/EC and Regulation (EC) No 726/2004[3],

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[4], and in particular Articles 28(3) and 45(3) thereof,

Having regard to the application submitted by Spark Therapeutics Ireland Ltd, on 17 August 2017, under Article 4(1) of Regulation (EC) No 726/2004,

Having regard to the opinions of the European Medicines Agency, formulated on 20 September 2018 by the Committee for Medicinal Products for Human Use and on 11 October 2018 by the Committee for Orphan Medicinal Products,

Whereas:

(1) Commission Decisions C(2012)2351(final) and C(2015)5424(final), adopted in accordance with Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products designated "Adenovirus-

[1] OJ L 136, 30.4.2004, p. 1.
[2] OJ L 18, 22.1.2000, p. 1.
[3] OJ L 324, 10.12.2007, p. 121.
[4] OJ L 378, 27.12.2006, p. 1.

associated viral vector serotype 2 containing the human *RPE65* gene" as an orphan medicinal product.

(2) Following the scientific discussions on the therapeutic indication at the European Medicines Agency, the Committee for Orphan Medicinal Products considered that the designated orphan conditions "Leber's congenital amaurosis" and "retinitis pigmentosa" should be renamed as "inherited retinal dystrophies". The Community Register of orphan medicinal products for human use is updated accordingly.

(3) The orphan medicinal product "Luxturna - voretigene neparvovec" complies with the requirements set out in Directive 2001/83/EC of the European Parliament and of the Council of 6 November 2001 on the Community code relating to medicinal products for human use[5].

(4) It is therefore appropriate to authorise its placing on the market.

(5) By Decision P/0221/2015, the European Medicines Agency agreed to the paediatric investigation plan for "Luxturna - voretigene neparvovec".

(6) It has been verified that the application complies with all measures contained in the agreed completed paediatric investigation plan, and that the relevant information on the results of studies is included in the Summary of Product Characteristics.

(7) It is therefore appropriate to include a statement of compliance in the marketing authorisation.

(8) For the purpose of the application of the Article 45(3) of Regulation (EC) No 1901/2006, it has been verified that all studies contained in the agreed paediatric investigation plan have been conducted after the entry into force of that Regulation.

(9) The development of this orphan medicinal product has complied with all measures in the agreed paediatric investigation plan P/0221/2015. Therefore, the market exclusivity period referred to in Article 8(1) of Regulation (EC) No 141/2000 should be extended to twelve years in accordance with Article 37 of Regulation (EC) No 1901/2006.

(10) The Committee for Medicinal Products for Human Use considered that "voretigene neparvovec" is a new active substance.

(11) 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 marketing authorisation provided for in Article 3 of Regulation (EC) No 726/2004 is granted for the medicinal product "Luxturna - voretigene neparvovec", the characteristics of which are summarised in Annex I to this Decision. "Luxturna - voretigene neparvovec" shall be registered in the Community register of medicinal products under number EU/1/18/1331.

[5] OJ L 311, 28.11.2001, p. 67.

*Article 2*

The development of this product has complied with all measures in the agreed paediatric investigation plan (Decision P/0221/2015). All studies were conducted after the entry into force of Regulation (EC) No 1901/2006.

The market exclusivity period referred to in Article 8(1) of Regulation (EC) No 141/2000 is extended to twelve years in accordance with Article 37 of Regulation (EC) No 1901/2006.

*Article 3*

The marketing authorisation concerning the medicinal product referred to in Article 1 shall be subject to compliance with the conditions set out in Annex II and, in particular, with those relating to manufacture and importation, control and issue.

*Article 4*

The labelling and package leaflet concerning the medicinal product referred to in Article 1 shall comply with the conditions set out in Annex III.

*Article 5*

The period of validity of the authorisation shall be five years from the date of notification of this Decision.

*Article 6*

This Decision is addressed to Spark Therapeutics Ireland Ltd, Studio G3,The Tower, Trinity Technology & Enterprise Campus, Pearse Street, Dublin 2, Ireland.

Done at Brussels, 22.11.2018

*For the Commission*

*Anne BUCHER*
*Director-General*

CERTIFIED COPY
For the Secretary-General,

Jordi AYET PUIGARNAU
Director of the Registry
EUROPEAN COMMISSION