EUROPEAN
COMMISSION

Brussels, 29.5.2019
C(2019) 4181 (final)

COMMISSION IMPLEMENTING DECISION

of 29.5.2019

granting a conditional marketing authorisation under Regulation (EC) No 726/2004 of the European Parliament and of the Council for "Zynteglo - autologous CD34^{+} cells encoding $\beta^{A\text{-}T87Q}$-globin gene", an orphan medicinal product for human use

(Text with EEA relevance)

(ONLY THE DUTCH 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) 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 Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products[3], 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[4],

Having regard to the application submitted by bluebird bio (Netherlands) B.V., on 4 October 2018, under Article 4(1) of Regulation (EC) No 726/2004,

Having regard to the opinions of the European Medicines Agency, formulated on 26 April 2019 by the Committee for Medicinal Products for Human Use and on 1 April 2019 by the Committee for Orphan Medicinal Products,

Whereas:

(1) Commission Decision C(2013)480(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 "Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human *beta$^{A\text{-}T87Q}$-globin* gene" as an orphan medicinal product.

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1 OJ L 136, 30.4.2004, p. 1.
2 OJ L 92, 30.3.2006, p. 6.
3 OJ L 18, 22.1.2000, p. 1.
4 OJ L 324, 10.12.2007, p. 121.

(2) "Zynteglo - autologous CD34^{+} cells encoding $\beta^{A\text{-}T87Q}$-globin gene" falls within the scope of Regulation (EC) No 507/2006, in particular Article 2(1) and 2(3). In addition, as set out in Annex IV, the medicinal product meets the requirements of Article 4 of this Regulation for the granting of a conditional marketing authorisation.

(3) Authorisation for the placing on the market of "Zynteglo - autologous CD34^{+} cells encoding $\beta^{A\text{-}T87Q}$-globin gene" should therefore be granted subject to certain requirements, in accordance with Article 14-a of Regulation (EC) No 726/2004 and Regulation (EC) No 507/2006.

(4) The medicinal product "Zynteglo - autologous CD34^{+} cells encoding $\beta^{A\text{-}T87Q}$-globin gene" 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] and in Regulation (EC) No 1394/2007.

(5) The Committee for Medicinal Products for Human Use considered that "Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human *beta$^{A\text{-}T87Q}$-globin* gene" is a new active substance.

(6) 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 conditional marketing authorisation provided for in Article 3 and 14-a of Regulation (EC) No 726/2004 is granted for the orphan medicinal product "Zynteglo - autologous CD34^{+} cells encoding $\beta^{A\text{-}T87Q}$-globin gene", the characteristics of which are summarised in Annex I to this Decision. "Zynteglo - autologous CD34^{+} cells encoding $\beta^{A\text{-}T87Q}$-globin gene" shall be registered in the Union Register of Medicinal Products under number EU/1/19/1367.

Article 2

The marketing authorisation concerning the orphan medicinal product referred to in Article 1 shall be subject to compliance with the requirements set out in Annex II. Those requirements shall be reviewed annually.

Article 3

The labelling and package leaflet concerning the orphan medicinal product referred to in Article 1 shall conform to Annex III.

Article 4

The period of validity of the authorisation shall be one year from the date of notification of this Decision.

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

Article 5

This Decision is addressed to bluebird bio (Netherlands) B.V., Stadsplateau 7, 3521 AZ Utrecht, Nederland.

Done at Brussels, 29.5.2019

For the Commission

Anne BUCHER
Director-General