EUROPEAN
COMMISSION

Brussels, 29.5.2024
C(2024) 3739 (final)

COMMISSION IMPLEMENTING DECISION

of 29.5.2024

granting, in exceptional circumstances, marketing authorisation under Regulation (EC) No 726/2004 of the European Parliament and of the Council for "Qalsody - tofersen", 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 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) 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 the application submitted by Biogen Netherlands B.V., on 1 December 2022, under Article 4(1) of Regulation (EC) No 726/2004,

Having regard to the opinions of the European Medicines Agency, formulated on 22 February 2024 and on 19 April 2024 by the Committee for Medicinal Products for Human Use,

Whereas:

(1) Commission Decision C(2016)5639(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 "Synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid" as an orphan medicinal product.

(2) The orphan medicinal product "Qalsody - tofersen" 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[3] .

(3) Following consultations with the applicant, the Committee for Medicinal Products for Human Use has pointed out, as set out in Annex IV, the existence, in this instance, of exceptional circumstances, these being that in the present state of scientific knowledge, comprehensive information on the efficacy and safety of the medicinal

[1] OJ L 136, 30.4.2004, p. 1.
[2] OJ L 18, 22.1.2000, p. 1.
[3] OJ L 311, 28.11.2001, p. 67.

product under normal conditions of use cannot be provided, which justify making the requested authorisation conditional on the fulfilment of certain requirements.

(4) Authorisation for the placing on the market of "Qalsody - tofersen" should therefore be granted subject to certain requirements, in accordance with Article 14(8) of Regulation (EC) No 726/2004.

(5) The Committee for Medicinal Products for Human Use considered that "tofersen" 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 marketing authorisation provided for in Article 3 of Regulation (EC) No 726/2004 is granted for the orphan medicinal product "Qalsody - tofersen", the characteristics of which are summarised in Annex I to this Decision. "Qalsody - tofersen" shall be registered in the Union Register of Medicinal Products under number EU/1/23/1783.

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 marketing authorisation shall be five years from the date of notification of this Decision.

Article 5

This Decision is addressed to Biogen Netherlands B.V., Prins Mauritslaan 13, 1171 LP Badhoevedorp, Nederland.

Done at Brussels, 29.5.2024

For the Commission

Sandra GALLINA
Director-General