EMA/86502/2015

European Medicines Agency decision

P/0044/2015

of 6 March 2015

on the agreement of a paediatric investigation plan and on the granting of a deferral for olesoxime (EMEA-001414-PIP01-12) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This Decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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The European Medicines Agency,

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

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[1],

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[2],

Having regard to the application submitted by TROPHOS SA on 15 February 2013 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 16 January 2015, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 of said Regulation,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the agreement of a paediatric investigation plan and on the granting of a deferral.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for olesoxime, oral suspension, oral use, gastric use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for olesoxime, oral suspension, oral use, gastric use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to TROPHOS SA, Parc Scientifique de Luminy, Luminy Biotech Entreprises, Case 931, 13288 – Marseille, France.

Done at London, 6 March 2015

For the European Medicines Agency
Jordi Llinares Garcia
Head of Division (ad interim)
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/658066/2014 corr

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral

EMEA-001414-PIP01-12

Scope of the application

Active substance(s):

Olesoxime

Condition(s):

Treatment of spinal muscular atrophy

Pharmaceutical form(s):

Oral suspension

Route(s) of administration:

Oral use

Gastric use

Name/corporate name of the PIP applicant:

TROPHOS SA

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, TROPHOS SA submitted for agreement to the European Medicines Agency on 15 February 2013 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 22 May 2013.

Supplementary information was provided by the applicant on 20 October 2014. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

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Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 18 of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation.

   The Icelandic and the Norwegian Paediatric Committee members agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex and appendix.

London, 16 January 2015

On behalf of the Paediatric Committee
Dr Dirk Mentzer, Chairman
(Signature on file)

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

Not applicable.

2. Paediatric investigation plan

2.1. Condition:

Treatment of spinal muscular atrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of 5q spinal muscular atrophy in paediatrics patients

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From birth to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Oral suspension

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of an age-appropriate oral suspension of olesoxime.
Non-clinical studies	1	**Study 2** Evaluation of the effects of olesoxime on the embryo-fetal and peri and post-natal development of the rat and subsequent reproductive performance of the offspring.
Clinical studies	3	**Study 3** Open-label, single and multiple dose study to assess safety and pharmacokinetics of olesoxime in patients with spinal muscular atrophy (SMA Type 1b, 2 and 3) aged from 6 to less than 12 years (and adults). **Study 4** Multicentre, randomised, adaptive, double-blind, placebo controlled study to assess safety and efficacy of olesoxime in non-ambulant patients with spinal muscular atrophy (SMA) type 2 or type 3 aged from 3 to less than 18 years (and adults).

Area	Number of measures	Description
		Study 5 Open-label, multi-centre, descriptive/exploratory, repeated dose study to assess safety, tolerability and pharmacokinetics, and collect efficacy data of olesoxime 10 mg/kg/day in patients with spinal muscular atrophy (SMA) type 1 aged from birth to less than 4 years.
Extrapolation, modelling and simulation studies	2	**Study 6** Extrapolation study, to support the use of olesoxime for treatment of SMA in children from birth to less than 4 years of age and for treatment of SMA type 1. **Study 7** Extrapolation study, to support the use of olesoxime for treatment of SMA in ambulant paediatric patients.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By June 2018
Deferral for one or more measures contained in the paediatric investigation plan:	Yes