EMA/195508/2016

European Medicines Agency decision

P/0107/2016

of 15 April 2016

on the acceptance of a modification of an agreed paediatric investigation plan for olesoxime (EMEA-001414-PIP01-12-M01) 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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on the acceptance of a modification of an agreed paediatric investigation plan for olesoxime (EMEA-001414-PIP01-12-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 European Medicines Agency's decision P/0044/2015 issued on 6 March 2015,

Having regard to the application submitted by Roche Registration Limited on 4 December 2015 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 February 2016, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan and to the deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the 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

Changes to the agreed paediatric investigation plan for olesoxime, age-appropriate oral liquid dosage form, oral use, gastric use, including changes to the deferral, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Roche Registration Limited, 6 Falcon Way. Shire Park, AL7 1TW - Welwyn Garden City, United Kingdom.

Done at London, 15 April 2016

For the European Medicines Agency
Zaïde Frias
Head of Division
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/851059/2015 Corr
London, 26 February 2016

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-001414-PIP01-12-M01

Scope of the application

Active substance(s):

Olesoxime

Condition(s):

Treatment of spinal muscular atrophy

Pharmaceutical form(s):

Age-appropriate oral liquid dosage form

Route(s) of administration:

Oral use

Gastric use

Name/corporate name of the PIP applicant:

Roche Registration Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Roche Registration Limited submitted to the European Medicines Agency on 4 December 2015 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0044/2015 issued on 6 March 2015.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 4 January 2016.

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Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan and to the deferral in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the 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.

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 liquid dosage form 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. (TRO19622TOEQ1501-1)
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). TRO19622CLEQ1115-1 (WP29845) **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). (TRO19622CLEQ1275-1) (WN29836)

		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) aged from birth to less than 3 years. (WP30095)
Extrapolation, modelling and simulation studies	3	**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. **Study 8** Population pharmacokinetic analysis of olesoxime in adult and paediatric SMA 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 December 2019
Deferral for one or more measures contained in the paediatric investigation plan:	Yes