EMA/761533/2014

European Medicines Agency decision

P/0331/2014

of 22 December 2014

on the acceptance of a modification of an agreed paediatric investigation plan for deferiprone (EMEA-001126-PIP01-10-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 deferiprone (EMEA-001126-PIP01-10-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/307/2011 issued on 20 December 2011,

Having regard to the application submitted by Consorzio per Valutazioni Biologiche e Farmacologiche on 25 August 2014 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 14 November 2014, 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.

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

[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 deferiprone, oral solution, oral use, 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 Consorzio per Valutazioni Biologiche e Farmacologiche, Via Luigi Porta, 14, 27100 - Pavia, Italy.

Done at London, 22 December 2014

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

EMA/PDCO/530817/2014

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

EMEA-001126-PIP01-10-M01

Scope of the application

Active substance(s):

Deferiprone

Condition(s):

Treatment of chronic iron overload

Pharmaceutical form(s):

Oral solution

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Consorzio per Valutazioni Biologiche e Farmacologiche

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Consorzio per Valutazioni Biologiche e Farmacologiche submitted to the European Medicines Agency on 25 August 2014 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/307/2011 issued on 20 December 2011.

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

The procedure started on 16 September 2014.

Scope of the modification

Some measures andtimelines of the Paediatric Investigation Plan have been modified.

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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 in the scope set out in the Annex I of this opinion.

   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 paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver 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, 14 November 2014

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

1.1. Condition:

Treatment of chronic iron overload

The waiver applies to:

- Preterm and term newborn infants, from birth to less than 28 days;
- for the oral solution, for oral use;
- on the grounds that clinical studies cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the paediatric population.

2. Paediatric Investigation Plan

2.1. Condition:

Treatment of chronic iron overload

2.1.1. Indication(s) targeted by the PIP

Treatment of iron overload in paediatric patients affected by haemoglobinopathies requiring chronic transfusion.

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

From 1 month to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Oral solution

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	3	**Study 1** (DEEP-1): Multicentre, oral single dose experimental and modelling study to evaluate the pharmacokinetics (PK) of deferiprone in patients aged from 1 month to less than 6 years of age affected by transfusion-dependent haemoglobinopathies. **Study 2** (DEEP-2):

Area	Number of studies	Description
		Multicentre, randomised, open label, non-inferiority active-controlled trial to evaluate the efficacy and safety of deferiprone compared to deferasirox in paediatric patients aged from 1 month to less than 18 years of age affected by transfusion-dependent haemoglobinopathies. **Study 3** (DEEP-3): Long-term observational safety study to evaluate the nature and incidence of adverse effects of deferiprone treatment in children with beta-thalassaemia major, who are aged from 1 month to less than 18 years of age when deferiprone treatment commences.
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By April 2016
Deferral for one or more studies contained in the paediatric investigation plan:	No