

EMA/973213/2011

European Medicines Agency decision P/307/2011

of 20 December 2011

on the agreement of a paediatric investigation plan and on the granting of a waiver for deferiprone (EMEA-001126-PIP01-10) 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 agreement of a paediatric investigation plan and on the granting of a waiver for deferiprone (EMA-001126-PIP01-10) 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Consorzio per Valutazioni Biologiche e Farmacologiche on 14 February 2011 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 11 November 2011, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 13 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 waiver.
- (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 waiver.

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 deferiprone, oral solution, oral 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 waiver for deferiprone, oral solution, oral 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 Consorzio per Valutazioni Biologiche e Farmacologiche (CVBF), Via Palestro, 26, 27100 Pavia, Italy.

Done at London, 20 December 2011

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/815594/2011

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

EMA-001126-PIP01-10

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 16(1) of Regulation (EC) No 1901/2006 as amended, Consorzio per Valutazioni Biologiche e Farmacologiche submitted for agreement to the European Medicines Agency on 14 February 2011 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 23 March 2011.

Supplementary information was provided by the applicant on 26 August 2011. The applicant proposed modifications to the paediatric investigation plan and to the waiver.

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 waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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 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(es) and appendix.

London, 11 November 2011

On behalf of the Paediatric Committee
Dr Daniel Brasseur, 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

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 and iron chelation.

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. Studies

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	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): 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.

Area	Number of studies	Description
		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.

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 December 2015
Deferral for one or more studies contained in the paediatric investigation plan:	No