EMA/985539/2011

European Medicines Agency decision

P/0023/2012

of 27 January 2012

on the acceptance of a modification of an agreed paediatric investigation plan for (S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt (FBS0701), (EMEA-001057-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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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/137/2011 issued on 10 June 2011 the decision,

Having regard to the application submitted by FerroKin BioSciences Ltd on 3 October 2011 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 9 December 2011, 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 (S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt (FBS0701), powder for oral solution, capsule, hard, 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 FerroKin BioSciences Ltd, 311 Shoreham Street, S2 4FA – Sheffield, United Kingdom.

Done at London, 27 January 2012

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

EMA/PDCO/847398/2011

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

EMEA-001057-PIP01-10-M01

Scope of the application

Active substance(s):

(S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt (FBS0701)

Condition(s):

Treatment of chronic iron overload

Pharmaceutical form(s):

Powder for oral solution

Capsule, hard

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

FerroKin BioSciences Ltd

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, FerroKin BioSciences Ltd submitted to the European Medicines Agency on 3 October 2011 an application for modification of the agreed paediatric investigation plan with a waiver and a deferral as set out in the European Medicines Agency's decision P/137/2011 issued on 10 June 2011.

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

The procedure started on 12 October 2011.

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Scope of the modification

Some measures and/or 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.

   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 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, 9 December 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:

- all subsets of the paediatric population from birth to less than 2 years of age;
- for powder for oral solution, oral use;
- for hard capsule, 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 transfusional iron overload.

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

From 2 to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Powder for oral solution.

Capsule, hard.

2.1.4. Studies

Area	Number of studies	Description
Quality	1	**Measure 1** Development of a powder for oral solution formulation for administering adequate doses to children down to 2 years of age.
Non-clinical	1	**Study 2** Juvenile toxicology and toxicokinetic study in rats.
Clinical	3	**Study 3** Open Label, Multi-Centre, Single-Dose Pharmacokinetics, and Multiple Dose Study of the Safety, Activity and Tolerability of FBS0701 in a Paediatric Population with Transfusional Iron Overload. **Study 4**

Area	Number of studies	Description
		Open-label, Randomised, Two-treatment, Two-period, Two-sequence, Cross-over, Adult Healthy Volunteer Study to Evaluate Safety, Pharmacokinetics and Relative Bioavailability following Oral Administration of the FBS0701 Capsule and Powder for Oral Solution Formulations. **Study 5** Comparator-Controlled Study of the Safety and Efficacy of FBS0701 in the Treatment of Adults and Children with Transfusional Iron Overload with Open Long-Term Safety Follow up.

3. Follow-up, completion and deferral of PIP

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