EMA/472044/2018

European Medicines Agency decision

P/0263/2018

of 15 August 2018

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for ferric pyrophosphate citrate (EMEA-002261-PIP01-17) 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 Rockwell Medical, Inc. on 13 November 2017 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 29 June 2018, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation 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 deferral 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 deferral.

(4) 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 ferric pyrophosphate citrate, solution for infusion, solution for haemodialysis, intravenous use, haemodialysis, 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 ferric pyrophosphate citrate, solution for infusion, solution for haemodialysis, intravenous use, haemodialysis, 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

A waiver for ferric pyrophosphate citrate, solution for infusion, solution for haemodialysis, intravenous use, haemodialysis, 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 4

This decision is addressed to Rockwell Medical, Inc., 30142 Wixom Road, 48393 - Wixom, MI 48393, United States.

EMA/PDCO/248709/2018
London, 29 June 2018

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

EMEA-002261-PIP01-17

Scope of the application

Active substance(s):

Ferric pyrophosphate citrate

Condition(s):

Treatment of anaemia of chronic kidney disease

Pharmaceutical form(s):

Solution for infusion

Solution for haemodialysis

Route(s) of administration:

Intravenous use

Haemodialysis

Name/corporate name of the PIP applicant:

Rockwell Medical, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Rockwell Medical, Inc. submitted for agreement to the European Medicines Agency on 13 November 2017 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 3 January 2018.

Supplementary information was provided by the applicant on 5 April 2018. The applicant proposed modifications to the paediatric investigation plan and 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 17(1) of said Regulation;
   - to grant a deferral in accordance with Article 21 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)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset of the paediatric population.

   The Norwegian Paediatric Committee member agrees 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 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.

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 anaemia of chronic kidney disease

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- solution for infusion, solution for haemodialysis, intravenous use, haemodialysis;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset.

2. Paediatric investigation plan

2.1. Condition

Treatment of anaemia of chronic kidney disease

2.1.1. Indication(s) targeted by the PIP

Treatment of anaemia in patients with haemodialysis-dependent chronic kidney disease

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

From 6 months to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for infusion

Solution for haemodialysis

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable.
Clinical studies	3	**Study 1** Multicentre, open-label, 2-period, single-dose study to determine the pharmacokinetics of intravenous administration of ferric pyrophosphate citrate in paediatric patients with stage 5 haemodialysis-dependent chronic kidney disease.

		Study 2 Single-centre, open-label, randomized-sequence, single-dose bioavailability study to determine the amount of ferric pyrophosphate citrate that is administered via haemodialysate to adult subjects with haemodialysis-dependent stage 5 chronic kidney disease. **Study 3** Multi-centre, open-label study to assess the safety of ferric pyrophosphate citrate administered via dialysate or intravenously, compared to standard anaemia practice in paediatric patients with haemodialysis-dependent chronic kidney disease.
Extrapolation, modelling and simulation studies	1	**Study 4** Pharmacokinetic modelling and simulation study to evaluate the use of the product in the anaemia in haemodialysis-dependent chronic kidney disease in children from 6 months to less than 18 years of age with anaemia from chronic kidney disease.
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:	No
Date of completion of the paediatric investigation plan:	By September 2020
Deferral for one or more measures contained in the paediatric investigation plan:	Yes