EMA/478268/2012

European Medicines Agency decision

P/0200/2012

of 24 August 2012

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the refusal of a waiver for human fibrinogen (EMEA-001208-PIP01-11) 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 Octapharma GmbH on 7 October 2011 under 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 6 July 2012, in accordance with Article 18 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 refusal 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 refusing 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 human fibrinogen, powder and solvent for solution for infusion, intravenous 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 deferral for human fibrinogen, powder and solvent for solution for infusion, intravenous 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

A waiver for human fibrinogen, powder and solvent for solution for infusion, intravenous 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 refused.

Article 4

This decision is addressed to Octapharma GmbH, Elisabeth-Selbert-Str. 11, 40764 - Langenfeld, Germany

Done at London, 24 August 2012

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

EMA/PDCO/400661/2012 Corr

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

EMEA-001208-PIP01-11

Scope of the application

Active substance(s):

Human Fibrinogen

Condition(s):

Treatment of congenital fibrinogen deficiency

Pharmaceutical form(s):

Powder and solvent for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Octapharma GmbH

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Octapharma GmbH, submitted for agreement to the European Medicines Agency on 07 October 2011 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 16 November 2011.

Supplementary information was provided by the applicant on 13 April 2012.

A meeting with the Paediatric Committee took place on 4 June 2012.

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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 deferral in accordance with Article 21 of said Regulation,
   - to refuse the granting of a waiver in accordance with Article 13 of said Regulation, for some of the subsets of the paediatric population and the above mentioned condition as it does not meet the grounds detailed in Article 11(1) of said Regulation.

   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 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.

Langen, Germany, 6 July 2012

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

The waiver is refused for the following:

1.1. Condition: Treatment of congenital fibrinogen deficiency

The request for the waiver applied to:

- The paediatric population from birth to less than 6 years of age,
- For powder and solvent for solution for infusion, intravenous use.

The waiver request does not provide evidence to support the following grounds set out in Article 11(1) of Regulation (EC) No 1901/2006 that:

(a) the specific medicinal product is likely to be ineffective or unsafe in the paediatric population;

(b) the disease or condition for which the specific medicinal product is intended occurs only in adult populations;

(c) the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients;

because:

- measures would be justified by the expected therapeutic benefit and clinical trials may be feasible.

The waiver request is therefore refused by the PDCO.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of congenital fibrinogen deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment of congenital fibrinogen deficiency.

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)

Powder and solvent for solution for infusion.

2.1.4. Studies

Area	Number of studies	Description
Quality	1	Study 1 Development of a lower strength for the pharmaceutical form powder and solvent for solution for infusion: 0.5 g.

Area	Number of studies	Description
Non-clinical	0	Not applicable.
Clinical	3	Study 2 A prospective, controlled, randomised, cross-over study investigating the pharmacokinetic properties, surrogate efficacy and safety of Human fibrinogen compared to Haemocomplettan P/ RiaSTAP in paediatric (12 to less than 18 years of age) and adult patients with congenital fibrinogen deficiency. Study 3 Prospective, open-label, uncontrolled study to assess the efficacy and safety of Human fibrinogen for on-demand treatment of acute bleeding and perisurgical prophylaxis in paediatric (12 to less than 18 years of age) and adult patients with congenital fibrinogen deficiency. Study 4 Prospective, open-label, uncontrolled study to assess the pharmacokinetic properties, efficacy and safety of Human fibrinogen for on-demand treatment of acute bleeding and perisurgical prophylaxis in paediatric patients with congenital fibrinogen deficiency below 12 years of age.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes.
Date of completion of the paediatric investigation plan:	By December 2018.
Deferral for one or more studies contained in the paediatric investigation plan:	Yes.