EMA/908420/2011

European Medicines Agency decision

P/276/2011

of 25 November 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for human normal immunoglobulin (EMEA-001110-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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8670
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

European Medicines Agency decision

P/276/2011

of 25 November 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for human normal immunoglobulin (EMEA-001110-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 726/2004[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 Pharmazeutika Produktionsges.m.b.H on 14 January 2011 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 14 October 2011, 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 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 human normal immunoglobulin, 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 normal immunoglobulin, 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 normal immunoglobulin, 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 4

This decision is addressed to Octapharma Pharmazeutika Produktionsges.m.b.H, Oberlaaer Strasse 235, 1100 – Vienna, Austria.

Done at London, 25 November 2011

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

EMA/PDCO/645135/2011

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

EMEA-001110-PIP01-10

Scope of the application

Active substance(s):

Human normal immunoglobulin

Condition(s):

Treatment of primary immunodeficiency as model for replacement therapy

Treatment of idiopathic thrombocytopenic purpura as model for immunomodulation

Treatment of chronic inflammatory demyelinating polyradiculo-neuropathy

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Octapharma Pharmazeutika Produktionsges.m.b.H

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Octapharma Pharmazeutika Produktionsges.m.b.H submitted for agreement to the European Medicines Agency on 14 January 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 23 February 2011.

Supplementary information was provided by the applicant on 1 August 2011. The applicant proposed modifications to the paediatric investigation plan and requested a deferral and a waiver.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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 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 and appendix.

London, 14 October 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. 1.1 Condition: treatment of idiopathic thrombocytopenic purpura (ITP) as model for immunomodulation.

This condition covers:

- idiopathic thrombocytopenic purpura (ITP), in children or adults at high risk of bleeding or prior to surgery to correct the platelet count;
- Guillain Barré syndrome;
- Kawasaki disease.

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- for solution for infusion for intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

1.2. 1.2 Condition: treatment of chronic inflammatory demyelinating polyradiculo-neuropathy

The waiver applies to:

- paediatric patients from birth to less than 4 years of age;
- for solution for infusion for intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan

2.1. 2.1 Condition: treatment of primary immunodeficiency (PID) as model for replacement therapy.

2.1.1. 2.1.1. Indication(s) targeted by the PIP

Treatment of PID as a model for replacement therapy in:

- Primary immunodeficiency syndromes with failure of antibody production.
- Hypogammaglobulinaemia and recurrent bacterial infections in patients with chronic lymphocytic leukaemia, in whom prophylactic antibiotics have failed.
- Hypogammaglobulinaemia and recurrent bacterial infections in plateau phase multiple myeloma patients who have failed to respond to pneumococcal immunisation.
- Children and adolescents with congenital AIDS and recurrent bacterial infections.
- Hypogammaglobulinaemia in patients after allogenic haematopoietic stem cell transplantation (HSCT).

2.1.2. 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. 2.1.3 Pharmaceutical form(s)

Solution for infusion for intravenous use.

2.1.4. 2.1.4 Studies

Area	Number of studies	Description
Quality	Total number of quality studies	Not applicable.
Non-clinical	Total number of studies	Not applicable.
Clinical	2	Study 1: (NGAM-01) Prospective, open-label, non-controlled, non-randomised, multiple-dose, multicentre clinical study to evaluate the efficacy, pharmacokinetics and safety of immunoglobulin intravenous (human) 10% in patients with Primary Immunodeficiency Diseases. Study 2: (NGAM-05) Prospective, open-label, non-controlled, non-randomised, multiple-dose, multicentre clinical study to evaluate the safety and tolerability of immunoglobulin intravenous (human) (10%) administered at high infusion rates to patients with primary immunodeficiency diseases (extension of study NGAM-01).

2.2. 2.2 Condition: treatment of chronic inflammatory demyelinating polyradiculo-neuropathy

Treatment of chronic inflammatory demyelinating polyradiculo-neuropathy.

2.2.1. 2.2.1. Indication(s) targeted by the PIP

Chronic inflammatory demyelinating polyradiculo-neuropathy.

2.2.2. 2.2.2. Subset(s) of the paediatric population concerned by the paediatric development

From the age of 4 years to less than 18 years of age.

2.2.3. 2.2.3. Pharmaceutical form(s)

Solution for infusion for intravenous use.

2.2.4. 2.2.4. Studies

Area	Number of studies	Description
Quality	Total number of quality studies	Not applicable.
Non-clinical	Total number of studies	Not applicable.
Clinical	1	Study 3: (NGAM-07) Prospective, open label, non-controlled, multicenter clinical study to evaluate the efficacy and safety of human intravenous immunoglobulin intravenous (10%) in paediatric patients with chronic inflammatory demyelinating polyradiculo-neuropathy (CIDP).

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 December 2019
Deferral for one or more studies contained in the paediatric investigation plan:	Yes