EMA/469914/2012

European Medicines Agency decision

P/0138/2012

of 20 July 2012

on the acceptance of a modification of an agreed paediatric investigation plan for human normal immunoglobulin (EMEA-001110-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.

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

European Medicines Agency decision

P/0138/2012

of 20 July 2012

on the acceptance of a modification of an agreed paediatric investigation plan for human normal immunoglobulin (EMEA-001110-PIP01-10-M01) 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 European Medicines Agency's decision P/276/2011 issued on 25 November 2011,

Having regard to the application submitted by Octapharma Pharmazeutika Produktionsges.m.b.H on 26 March 2012 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 8 June 2012, in accordance with Article 22 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 acceptance of changes to the agreed paediatric investigation plan and to the waiver.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the 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

Changes to the agreed paediatric investigation plan for human normal immunoglobulin, solution for infusion, intravenous use, including changes to the waiver, 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 Octapharma Pharmazeutika Produktionsges.m.b.H, Oberlaaer Strasse 235, 1100 – Vienna, Austria.

Done at London, 20 July 2012

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

EMA/469914/2012

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

EMEA-001110-PIP01-10-M01

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

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 22 of Regulation (EC) No 1901/2006 as amended, Octapharma Pharmazeutika Produktionsges.m.b.H submitted to the European Medicines Agency on 26 March 2012 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/276/2011 issued on 25 November 2011.

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

The procedure started on 16 April 2012.

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

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 and to the waiver in the scope set out in the Annex I of this opinion.

   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 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, 8 June 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

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.

2. Paediatric Investigation Plan

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

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 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)

Solution for infusion for intravenous use.

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).

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 October 2012
Deferral for one or more studies contained in the paediatric investigation plan:	Yes