EMA/253072/2012

European Medicines Agency decision

P/0079/2012

of 27 April 2012

on the acceptance of a modification of an agreed paediatric investigation plan for human normal immunoglobulin (Gammagen) (EMEA-000415-PIP01-08-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/165/2009 issued on 14 August 2009,

Having regard to the application submitted by ORFAGEN on 23 January 2012 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 March 2012, 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 human normal immunoglobulin (Gammagen), solution for infusion, intravenous 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 ORFAGEN, CRDPF Langlade, 3 avenue Hubert Curien, BP13562, 31035 Toulouse Cedex 01, France.

Done at London, 27 April 2012

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

EMA/PDCO/120105/2012

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

EMEA-000415-PIP01-08-M01

Scope of the application

Active substance(s):

Human normal immunoglobulin

Invented name:

Gammagen

Condition(s):

Treatment of dermatopolymyositis

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

ORFAGEN

Information about the authorised medicinal product:

See Annex I

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, ORFAGEN submitted to the European Medicines Agency on 23 January 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/165/2009 issued on 14 August 2009.

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

The procedure started on 15 February 2012.

Scope of the modification

Timelines of the paediatric investigation plan has been modified, and administrative errors corrected.

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 in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

   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.

2. 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 March 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 dermatopolymyositis

The waiver applies to:

- The paediatric population from birth to less than 2 years of age;
- for solution for infusion for intravenous use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan

2.1. Condition: Treatment of dermatopolymyositis

2.1.1. Indication(s) targeted by the PIP

Treatment of Juvenile Dermatomyositis (JDM), Polymyositis in children and Overlap Myositis in children.

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

From 2 to less than 18 years.

2.1.3. Pharmaceutical form(s)

Solution for infusion for intravenous use.

2.1.4. Studies

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	1	Efficacy and safety of a human normal immunoglobulin product for intravenous administration (IVIg) in the treatment of juvenile dermatomyositis (JDM), juvenile polymyositis (PM) and juvenile overlap myositis: prospective, randomised, double-blind, comparative, two-parallel group, multicentric study in children from 2 to less than 18 years of age.

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

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of dermatopolymyositis

   Authorised indications:

   - none

2. Treatment of primary immunodeficiency syndromes

   Authorised indications:

   - Replacement therapy in primary immunodeficiency syndromes such as:
     - congenital agammaglobulinaemia and hypogammaglobulinaemia;
     - common variable immunodeficiency;
     - severe combined immunodeficiency;
     - Wiskott Aldrich syndrome.

3. Treatment of secondary immunodeficiency syndromes

   Authorised indications:

   - Myeloma or chronic lymphocytic leukaemia with severe secondary hypogammaglobulinaemia and recurrent infections.
   - Children with congenital AIDS and recurrent infections.

4. Immunomodulation

   Authorised indications:

   - 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.
   - Allogeneic bone marrow transplantation

Invented name	**Strength**	**Pharmaceutical form**	**Route of administration**
Ig VENA	50 g/l	Solution for infusion	Intravenous use