EMA/136542/2017

European Medicines Agency decision

P/0052/2017

of 17 March 2017

on the acceptance of a modification of an agreed paediatric investigation plan for conestat alfa (Ruconest), (EMEA-000367-PIP01-08-M06) 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/132/2009 issued on 17 July 2009, the decision P/103/2011 issued on 3 May 2011, the decision P/0024/2013 issued on 26 February 2013, the decision P/0131/2014 issued on 10 June 2014, the decision P/0010/2015 issued on 30 January 2015 and the decision P/0004/2016 issued on 22 January 2016,

Having regard to the application submitted by Pharming Group N.V. on 7 November 2016 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 27 January 2017, 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 and to the deferral.

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

[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 conestat alfa (Ruconest), powder for solution for injection, intravenous use, including changes to the deferral, 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 Pharming Group N.V., Darwinweg 24, 2333 CR – Leiden, The Netherlands.

EMA/PDCO/740371/2016
London, 27 January 2017

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

EMEA-000367-PIP01-08-M06

Scope of the application

Active substance(s):

Conestat alfa

Invented name:

Ruconest

Condition(s):

Treatment of hereditary angioedema

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Pharming Group N.V.

Information about the authorised medicinal product:

See Annex II

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Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Pharming Group N.V. submitted to the European Medicines Agency on 7 November 2016 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/132/2009 issued on 17 July 2009, the decision P/103/2011 issued on 3 May 2011, the decision P/0024/2013 issued on 26 February 2013, the decision P/0131/2014 issued on 10 June 2014, the decision P/0010/2015 issued on 30 January 2015 and the decision P/0004/2016 issued on 22 January 2016.

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

The procedure started on 29 November 2016.

Scope of the modification

Some 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 deferral 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.

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 annexes 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 hereditary angioedema

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- for powder for solution for injection, intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2. Paediatric Investigation Plan

2.1. Condition:

Treatment of hereditary angioedema

2.1.1. Indication(s) targeted by the PIP

Treatment of acute attacks of angioedema associated with hereditary C1 esterase inhibitor deficiency

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

From 2 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder for solution for injection

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	2	**Study 1** Open-label, single arm trial to evaluate the safety and immunogenicity of conestat alfa for the treatment of acute attacks in adolescents (and adults) with hereditary angioedema from 14 to less than 18 years old **Study 2** Open label, single-arm trial to evaluate the safety and immunogenicity of conestat alfa for the

		treatment of acute attacks in children with hereditary angioedema from 2 to 14 years of age
Extrapolation, modelling & simulation studies	0	Not applicable
Other studies	0	Not applicable
Other measures	0	Not applicable

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By December 2017
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 hereditary angioedema

Authorised indication(s):

- Treatment of acute angioedema attacks in adults and adolescents with hereditary angioedema (HAE) due to C1 esterase inhibitor deficiency

Authorised pharmaceutical form(s):

Powder for solution for injection

Authorised route(s) of administration:

Intravenous use