



European Medicines Agency

Doc. Ref. EMEA/352210/2009
P/132/2009

EUROPEAN MEDICINES AGENCY DECISION

of 17 July 2009

on the agreement of a Paediatric Investigation Plan and on the granting of a deferral and on the granting of a waiver for recombinant human C1 inhibitor (EMEA-000367-PIP01-08) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council as amended

(ONLY THE ENGLISH TEXT IS AUTHENTIC)

DISCLAIMER: This Decision does not entitle to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006, as amended.

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THE EUROPEAN MEDICINES AGENCY,

Having regard to the Treaty establishing the European Community,

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 as amended and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004¹,

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 Agency²,

Having regard to the application submitted by Pharming Group N.V. on 15 September 2008 under Article 16(1) of Regulation (EC) No 1901/2006 as amended also requesting a waiver under Article 13 of said Regulation and a deferral under Article 20 of said Regulation,

Having regard to the Opinion of the Paediatric Committee of the European Medicines Agency, issued on 29 May 2009, in accordance with Article 18 of Regulation (EC) No 1901/2006 as amended, and Article 13 of said Regulation and Article 21 of said Regulation,

Having regard to Article 25 of Regulation (EC) No 1901/2006 as amended,

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 granting 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 recombinant human C1 inhibitor, powder for solution for injection, 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 recombinant human C1 inhibitor, powder for solution for injection, 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 recombinant human C1 inhibitor, powder for solution for injection, 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 Pharming Group N.V., Darwinweg 24, 2333 CR, Leiden, Netherlands.

Done at London, 17 July 2009

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Doc. Ref. EMEA/PDCO/284186/2009
EMEA-000367-PIP01-08

OPINION OF THE PAEDIATRIC COMMITTEE ON THE AGREEMENT OF A PAEDIATRIC INVESTIGATION PLAN AND DEFERRAL AND A WAIVER

Scope of the application

Active substance(s):

Recombinant human C1 inhibitor

Condition(s):

Hereditary angioedema

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.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Pharming Group N.V. submitted for agreement to the EMEA on 15 September 2008 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 October 2008.

Supplementary information was provided by the applicant on 19 March 2009.

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 Agency, together with its annex(es) and appendix.

London, 29 May 2009

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman

(Signature on file)

ANNEX I

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

CONDITION(S)

Hereditary angioedema

B. WAIVER

- **Condition**

Hereditary angioedema

The waiver applies to:

- Preterm newborn infants, term newborn infants (from birth to less than 28 days), infants and toddlers (from 28 days to less than 24 months), for recombinant human C1 inhibitor, 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(s) are not feasible.

C. PAEDIATRIC INVESTIGATION PLAN

- **Condition to be investigated**

Hereditary angioedema

- **Proposed PIP indication**

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

- **Subset(s) of the paediatric population concerned by the paediatric development**

From 2 to less than 18 years.

- **Formulation(s)**

Powder for solution for injection, intravenous use, 2100 U (150 U/mL)

- **Studies**

Area	Number of studies	Description
Quality		Not applicable
Non-clinical		Not applicable.
Clinical		1. Open-label, single arm trial to evaluate the safety and immunogenicity of recombinant human C1 inhibitor for the treatment of acute attacks in adolescents with hereditary angioedema, from Tanner pubertal stage 2 to less than 18 years old. 2. Open label, single-arm trial to evaluate the safety and immunogenicity of recombinant human C1 inhibitor for the treatment of acute attacks in prepubertal children with hereditary angioedema, from 2 years of age (Tanner stage I).

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 2012
Deferral for some or all studies contained in the paediatric investigation plan:	Yes