



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/514793/2014

European Medicines Agency decision

P/0259/2014

of 1 October 2014

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for D-argininamide, N-acetyl-D-cysteinyl-D-alanyl-D-arginyl-D-arginyl-D-arginyl-D-alanyl-, disulfide with L-cysteine (AMG 416) (EMEA-001554-PIP01-13) 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 726/2004¹,

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 Amgen Europe B.V. on 11 November 2013 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 15 August 2014, 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 D-argininamide, N-acetyl-D-cysteinyl-D-alanyl-D-arginyl-D-arginyl-D-arginyl-D-alanyl-, disulfide with L-cysteine (AMG 416), 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 D-argininamide, N-acetyl-D-cysteinyl-D-alanyl-D-arginyl-D-arginyl-D-arginyl-D-alanyl-, disulfide with L-cysteine (AMG 416), 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 D-argininamide, N-acetyl-D-cysteinyl-D-alanyl-D-arginyl-D-arginyl-D-arginyl-D-alanyl-, disulfide with L-cysteine (AMG 416), 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 Amgen Europe B.V., Minervum 7061, 4817-ZK - Breda, The Netherlands.

Done at London, 1 October 2014

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

EMA/PDCO/337500/2014

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

EMA-001554-PIP01-13

Scope of the application

Active substance(s):

D-Argininamide, N-acetyl-D-cysteinyl-D-alanyl-D-arginyl-D-arginyl-D-arginyl-D-alanyl-, disulfide with L-cysteine (AMG 416)

Condition(s):

Treatment of hyperparathyroidism

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intravenous use

Name / corporate name of the PIP applicant:

Amgen Europe B.V.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Amgen Europe B.V. submitted for agreement to the European Medicines Agency on 11 November 2013 an application for a paediatric investigation plan for the above mentioned medicinal product and deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 18 December 2013.

Supplementary information was provided by the applicant on 27 May 2014. The applicant proposed modifications to the paediatric investigation plan.

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 Norwegian Paediatric Committee member agrees 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, 15 August 2014

On behalf of the Paediatric Committee
Dr Dirk Mentzer, 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 (PIP)

1. Waiver

1.1. Condition:

Treatment of hyperparathyroidism

The waiver applies to:

- Solution for infusion, intravenous use;
- the paediatric population from birth to less than 2 years;
- 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;

2. Paediatric investigation plan

2.1. Condition:

Treatment of hyperparathyroidism

2.1.1. Indication(s) targeted by the PIP

Treatment of secondary hyperparathyroidism in paediatric patients with chronic kidney disease receiving maintenance haemodialysis

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)

Solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	2	Study 1 Single arm, open-label, single-dose PK, PD and safety study in children and adolescents from 2 to 18 years with secondary hyperparathyroidism receiving maintenance haemodialysis

		Study 2 Single arm, open-label, multidose, titration, PK, PD and safety study in children and adolescents 2 to 18 years with secondary hyperparathyroidism receiving maintenance haemodialysis
Extrapolation, modelling and simulation studies	1	Comparative pharmacokinetic/pharmacodynamic modelling study between adult and paediatric patients with secondary hyperparathyroidism on maintenance haemodialysis
Other studies	0	Not applicable.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By January 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes