



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/430903/2017

European Medicines Agency decision

P/0219/2017

of 9 August 2017

on the acceptance of a modification of an agreed paediatric investigation plan for luspatercept (EMA-001521-PIP01-13-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.



European Medicines Agency decision

P/0219/2017

of 9 August 2017

on the acceptance of a modification of an agreed paediatric investigation plan for luspatercept (EMA-001521-PIP01-13-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 726/2004¹,

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 European Medicines Agency's decision P/0245/2014 issued on 29 September 2014,

Having regard to the application submitted by Celgene Europe Ltd on 31 March 2017 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 23 June 2017, in accordance with Article 22 of Regulation (EC) No 1901/2006 and Article 20(1) of said Regulation and Article 11(1) 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 on the refusal of the changes to the deferral and on the refusal of the changes to the waiver.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan and refusal of the changes to the deferral and refusal of the 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 luspatercept, solution for injection, subcutaneous 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

Changes to the deferral and to the waiver for luspatercept, solution for injection, subcutaneous use, are hereby refused in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 3

This decision is addressed to Celgene Europe Ltd, 1 Longwalk Road, Stockley Park, UB11 1DB – Uxbridge, United Kingdom.

EMA/PDCO/241599/2017

London, 23 June 2017

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001521-PIP01-13-M01

Scope of the application

Active substance(s):

Luspatercept

Condition(s):

Treatment of myelodysplastic syndromes

Treatment of beta-thalassaemia

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name / corporate name of the PIP applicant:

Celgene Europe Ltd

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Celgene Europe Ltd submitted to the European Medicines Agency on 31 March 2017 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/0245/2014 issued on 29 September 2014.

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

The procedure started on 25 April 2017.

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 in the scope set out in the Annex I of this opinion;
 - to refuse changes to the deferral as it does not meet the grounds detailed in Article 20(1) of said Regulation;
 - to refuse changes to the waiver for one of the above mentioned condition(s) as it does not meet the grounds detailed in Article 11(1) of said Regulation.

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 annex 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 myelodysplastic syndromes

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

1.2. Condition

Treatment of beta-thalassaemia

The waiver applies to:

- the paediatric population from birth to less than 6 months;
- solution for injection, subcutaneous 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 beta-thalassaemia

2.1.1. Indication(s) targeted by the PIP

Treatment of anaemia in patients with beta-thalassaemia intermedia and major

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

From 6 months 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	1	Study 1 Development of an age-appropriate formulation in a self-administration device for subcutaneous use, allowing sufficient accuracy of dose measurement and delivery in all age groups to be treated.
Non-clinical studies	2	Study 2 (1383657025333) Dose range-finding juvenile toxicity study Study 3 (1381154982795) Definitive juvenile toxicity study
Clinical studies	2	Study 4 (1378937733170) Two-part, double-blind, randomised, placebo controlled trial to evaluate pharmacokinetics, safety and efficacy of luspatercept in children from 6 months to less than 18 years of age with transfusion-dependent beta-thalassaemia. Study 5 (1406216840211) Two-part, double-blind, randomised, placebo controlled trial to evaluate pharmacokinetics, safety and efficacy of luspatercept in children from 6 months to less than 18 years of age with non-transfusion-dependent beta-thalassaemia.
Extrapolation, modelling and simulation studies	0	Not applicable.
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 June 2019
Deferral for one or more measures contained in the paediatric investigation plan:	Yes