EMA/408691/2016

European Medicines Agency decision

P/0194/2016

of 15 July 2016

on the agreement of a paediatric investigation plan and on the granting of a deferral for metreleptin (EMEA-001701-PIP01-14) 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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on the agreement of a paediatric investigation plan and on the granting of a deferral for metreleptin (EMEA-001701-PIP01-14) 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 726/2004[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 application submitted by Aegerion Pharmaceuticals Ltd on 9 October 2014 under Article 16(1) of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 27 May 2016, in accordance with Article 18 of Regulation (EC) No 1901/2006, and of its own motion in accordance with Article 21 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.

(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.

[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 metreleptin, powder for solution for injection, subcutaneous 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 metreleptin, powder for solution for injection, subcutaneous 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

This decision is addressed to Aegerion Pharmaceuticals Ltd, Lakeside House, 1 Furzeground Way, Stockley Park East, UB11 1BD – Uxbridge, United Kingdom.

Done at London, 15 July 2016

For the European Medicines Agency
Zaïde Frias
Head of Division
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/194292/2016
London, 27 May 2016

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

EMEA-001701-PIP01-14

Scope of the application

Active substance(s):

Metreleptin

Condition(s):

Treatment of lipodystrophy

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Aegerion Pharmaceuticals Ltd

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Aegerion Pharmaceuticals Ltd submitted for agreement to the European Medicines Agency on 9 October 2014 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 19 November 2014.

Supplementary information was provided by the applicant on 7 March 2016. The applicant proposed modifications to the paediatric investigation plan.

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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 on its own motion in accordance with Article 21 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 agreed paediatric investigation plan 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

Not applicable.

2. Paediatric investigation plan

2.1. Condition:

Treatment of lipodystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of lipodystrophy

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

From birth 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 measures	Description
Quality-related studies	2	Study 1 Development of a 2.5 mg and of a 5 mg presentation of metreleptin to facilitate dosing of paediatric patients. Study 2 Study to select an appropriate small capacity syringe appropriate to provide accurate dosing of metreleptin to paediatric patients to be available with the appropriate presentations.
Non-clinical studies	0	Not applicable.
Clinical studies	3	Study 3 (991265/20010769) Open-label uncontrolled trial to evaluate activity and safety of metreleptin in children from 7 months to less than 18 years of age (and adults) with lipodystrophy. Study 4 (FHA101) Open-label, uncontrolled trial to evaluate activity and

		safety of metreleptin in children from 5 years to less than 18 years of age (and adults) with lipodystrophy and associated diabetes mellitus and/or hypertriglyceridaemia. Study 5 Open-label, uncontrolled trial to evaluate PK, activity and safety of metreleptin in patients less than 6 years of age with generalised lipodystrophy and associated diabetes mellitus and/or hypertriglyceridaemia.
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 September 2020
Deferral for one or more measures contained in the paediatric investigation plan:	Yes