EMA/408699/2016

European Medicines Agency decision

P/0178/2016

of 8 July 2016

on the agreement of a paediatric investigation plan and on the granting of a deferral for eleclazine (EMEA-001697-PIP02-14) 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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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 Gilead Sciences International Ltd on 7 August 2015 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 27 May 2016, 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.

(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 eleclazine, age-appropriate dosage form, film-coated tablet, tablet, 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 eleclazine, age-appropriate dosage form, film-coated tablet, tablet, 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 Gilead Sciences International Ltd, Flowers Building, Granta Park, Abington, CB21 6GT – Cambridge, United Kingdom.

Done at London, 8 July 2016

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

EMA/PDCO/197216/2016
London, 27 May 2016

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

EMEA-001697-PIP02-14

Scope of the application

Active substance(s):

Eleclazine

Condition(s):

Treatment of hypertrophic cardiomyopathy

Pharmaceutical form(s):

Age-appropriate dosage form

Film-coated tablet

Tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Gilead Sciences International Ltd

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Gilead Sciences International Ltd submitted for agreement to the European Medicines Agency on 7 August 2015 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 15 September 2015.

Supplementary information was provided by the applicant on 4 March 2016. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

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.

   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 hypertrophic cardiomyopathy

2.1.1. Indication(s) targeted by the PIP

Treatment of symptomatic hypertrophic cardiomyopathy

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)

Age-appropriate dosage form

Film-coated tablet

Tablet

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	2	Study 1 Development of a reduced-dose strength tablet appropriate for children from 6 years to less than 12 years of age (same study as in PIP EMEA-001697-PIP01-14). Study 2 Development of an age-appropriate pharmaceutical form for use in paediatric subjects less than 6 years of age and those who are unable to swallow tablets (same study as in PIP EMEA-001697-PIP01-14).
Non-clinical studies	1	Study 3 Juvenile toxicology study to include standard toxicology endpoints, including landmarks of sexual maturation, as well as evaluations of learning, behaviour, and memory (same study as in PIP EMEA-001697-PIP01-14).

Area	Number of measures	Description
Clinical studies	2	Study 4 Comparative bioavailability study between the age appropriate formulation developed in study 2 and the adult tablet (same study as in PIP EMEA-001697-PIP01-14). Study 5 Double-blind, randomised, dose ranging, placebo controlled study to evaluate safety, tolerability, activity and pharmacokinetics in children from birth to less than 18 years of age with symptomatic hypertrophic cardiomyopathy.
Extrapolation, modelling and simulation studies	1	Study 6 Population PK modelling and simulation study to predict eleclazine exposure in children from birth to less than 18 years of age (same study as in PIP EMEA-001697-PIP01-14).
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 April 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes