EMA/76623/2020

European Medicines Agency decision

P/0115/2020

of 18 March 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral for soticlestat (EMEA-002572-PIP01-19) 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 Takeda Pharma A/S on 23 April 2019 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 31 January 2020, in accordance with Article 17 of Regulation (EC) No 1901/2006 and 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 soticlestat, film-coated tablet, age-appropriate oral formulation, oral use, gastric 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 soticlestat, film-coated tablet, age-appropriate oral formulation, oral use, gastric 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 Takeda Pharma A/S, Dybendal Alle 10, 2630 – Taastrup, Denmark.

EMA/PDCO/614257/2019
Amsterdam, 31 January 2020

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

EMEA-002572-PIP01-19

Scope of the application

Active substance(s):

Soticlestat

Condition(s):

Treatment of Chromosome 15q Duplication Syndrome

Treatment of Cyclin-Dependent Kinase-Like 5 deficiency disorder

Pharmaceutical form(s):

Film-coated tablet

Age-appropriate oral formulation

Route(s) of administration:

Oral use

Gastric use

Name/corporate name of the PIP applicant:

Takeda Pharma A/S

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Takeda Pharma A/S submitted for agreement to the European Medicines Agency on 23 April 2019 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 28 May 2019.

Supplementary information was provided by the applicant on 25 October 2019. 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 17(1) 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 Chromosome 15q Duplication Syndrome (Dup15q)

2.1.1. Indication(s) targeted by the PIP

Treatment of seizures associated with Chromosome 15q Duplication Syndrome

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)

Film-coated tablet

Age-appropriate oral formulation

2.1.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	2	**Study 1** Development of an age appropriate oral formulation for neonates and children below two years of age **Study 2** Study to demonstrate feasibility of administration of the drug product through the G-tube/feeding tube
Non-clinical studies	0	Not applicable

Clinical studies	4	**Study 3** Multi-centre, open-label, pilot study in patients from 2 to less than 18 years of age (and adults) with Chromosome 15q Duplication Syndrome or CDKL5 Deficiency Disorder to investigate the effect of soticlestat on the frequency of motor seizures (TAK-935-18-002 (OV935)) **Study 4** Open-label extension study to assess the long-term safety, tolerability and effect on seizure frequency of soticlestat as adjunctive therapy in patients with rare epilepsy (TAK-935-18-001 (OV935)) **Study 5** Multi-centre, randomized, double-blind, placebo-controlled, parallel-group, study in patients from 2 to less than 18 years of age (and adults) with Chromosome 15q Duplication Syndrome, to assess the percent change from baseline in motor seizure frequency per 28 days compared to placebo (TAK-935-20-yyy (OV935)) **Study 8** Multi-centre, open-label, safety, efficacy, and tolerability study of soticlestat in patients from birth to less than 2 years of age with Chromosome 15q Duplication Syndrome (TAK-935-20-www (OV935))
Extrapolation, modelling and simulation studies	0	Not applicable
Other studies	0	Not applicable
Other measures	0	Not applicable

2.2. Condition:

Treatment of Cyclin-Dependent Kinase-Like 5 deficiency disorder (CDD)

2.2.1. Indication(s) targeted by the PIP

Treatment of seizures associated with Cyclin-Dependent Kinase-Like 5 deficiency disorder

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

From birth to less than 18 years of age.

2.2.3. Pharmaceutical form(s)

Film-coated tablet

Age-appropriate oral formulation

2.2.4. Measures

Area	Number of measures	Description
Quality-related studies	2	**Study 1** *The same study as for the condition "treatment of Chromosome 15q Duplication Syndrome (Dup15q))"* **Study 2** *The same study as for the condition "treatment of Chromosome 15q Duplication Syndrome (Dup15q)"*
Non-clinical studies	0	Not applicable
Clinical studies	4	**Study 3** *The same study as for the condition "treatment of Chromosome 15q Duplication Syndrome (Dup15q)"* **Study 4** *The same study as for the condition "treatment of Chromosome 15q Duplication Syndrome (Dup15q)"* **Study 6** Multi-centre, randomized, double-blind, placebo-controlled, parallel-group, study in patients from 2 to less than 18 years of age (and adults) with CDKL5 Deficiency Disorder, to assess the percent change from baseline in motor seizure frequency per 28 days compared to placebo (TAK-935-20-xxx (OV935)) **Study 7** Multi-centre, open-label, safety, efficacy, and tolerability study of soticlestat in patients from birth to less than 2 years of age with CDKL5 Deficiency Disorder (TAK-935-20-zzz (OV935))
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:	No
Date of completion of the paediatric investigation plan:	By December 2026
Deferral for one or more measures contained in the paediatric investigation plan:	Yes