EMA/149068/2022

European Medicines Agency decision

P/0103/2022

of 21 March 2022

on the agreement of a paediatric investigation plan for censavudine (EMEA-003075-PIP01-21) 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/0103/2022

of 21 March 2022

on the agreement of a paediatric investigation plan for censavudine (EMEA-003075-PIP01-21) 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Transposon Therapeutics, Inc. on 12 July 2021 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 25 February 2022, in accordance with Article 17 of Regulation (EC) No 1901/2006,

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.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for censavudine, age-appropriate oral solid dosage form, age-appropriate oral suspension formulation, oral 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

This decision is addressed to Transposon Therapeutics, Inc., 2765 Sand Hill Road, 94025 - Menlo Park CA, United States.

EMA/PDCO/713367/2021
Amsterdam, 25 February 2022

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

EMEA-003075-PIP01-21

Scope of the application

Active substance(s):

Censavudine

Condition(s):

Treatment of Aicardi-Goutières syndrome

Pharmaceutical form(s):

Age-appropriate oral solid dosage form

Age-appropriate oral suspension formulation

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Transposon Therapeutics, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Transposon Therapeutics, Inc. submitted for agreement to the European Medicines Agency on 12 July 2021 an application for a paediatric investigation plan and a deferral under Article 20 of said Regulation for the above mentioned medicinal product.

The procedure started on 17 August 2021.

Supplementary information was provided by the applicant on 19 November 2021. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a deferral.

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.

   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 Aicardi-Goutières Syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of Aicardi-Goutières 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)

Age-appropriate oral solid dosage form

Age-appropriate oral suspension formulation

2.1.4. Measures

Area	Description
Quality-related studies	Study 1: Stability study of the age appropriate oral solid dosage form according to the ICH Q1A(R2) guideline. Study 2: Development of an age-appropriate oral suspension formulation for use in children below 6 months of age.
Non-clinical studies	Study 3 (TPN101.TX029): Definitive juvenile toxicity study in Sprague-Dawley rats over 21 weeks to support evaluation of the safety of censavudine when used in children from birth to less than 18 years of age.
Clinical studies	Study 4 (TPN-101-AGS-201): Open-label, non-comparative, historical-controlled trial to evaluate pharmacokinetics, safety and activity of censavudine in children from birth to less than 18 years of age (and adults) with Aicardi-Goutières syndrome. Study 5 (TPN-101-AGS-301):

	Double-blind, randomised, placebo-controlled study to evaluate safety and efficacy of censavudine in children from birth to less than 18 years of age with Aicardi-Goutières syndrome.
Extrapolation, modelling and simulation studies	Study 6: Modelling and simulation population pharmacokinetic study to evaluate the use of censavudine in children from birth to less than 18 years of age with the Aicardi-Goutières syndrome.
Other studies	Not applicable.
Other measures	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 November 2028
Deferral for one or more measures contained in the paediatric investigation plan:	No