

EMA/364886/2024

European Medicines Agency decision P/0306/2024

of 16 August 2024

on the acceptance of a modification of an agreed paediatric investigation plan for fosigotifator sodium tromethamine (EMA-003344-PIP01-22-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.

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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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0354/2023 issued on 8 September 2023,

Having regard to the application submitted by Calico Life Sciences LLC on 22 March 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 28 June 2024, in accordance with Article 22 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 acceptance of changes to the agreed paediatric investigation plan and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

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

Changes to the agreed paediatric investigation plan for fosigotifator sodium tromethamine, granules, age-appropriate dosage form , oral use, including changes to the deferral, 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

This decision is addressed to AbbVie Limited, AbbVie House, Vanwall Business Park, 2 Vanwall Road, Berkshire, SL6 4UB – Maidenhead, United Kingdom.

EMA/PDCO/145854/2024
Amsterdam, 28 June 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-003344-PIP01-22-M01

Scope of the application

Active substance(s):

Fosigotifator sodium tromethamine

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of vanishing white matter disease

Pharmaceutical form(s):

Granules

Age-appropriate dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

AbbVie Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Calico Life Sciences LLC submitted to the European Medicines Agency on 22 March 2024 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0354/2023 issued on 8 September 2023.

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

The procedure started on 29 April 2024.

On 13 April 2024 Calico Life Sciences LLC requested to transfer the paediatric investigation plan to AbbVie Limited.

Scope of the modification

Some measures and 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 and to the deferral in the scope set out in the Annex I of this opinion.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the 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 annexes 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 vanishing white matter disease

2.1.1. Indication(s) targeted by the PIP

Treatment of vanishing white matter disease (VWM)

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)

Granules

Age-appropriate dosage form

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of granule formulation for paediatric use in children from 6 months of age. Study 2 Feasibility assessment and formulation development of an age-appropriate formulation of ABBV-CLS-7262 for use in infants from birth to less than 6 months of age.
Non-clinical studies	Study 3 (TA21-009) Definitive juvenile toxicity study to support the evaluation of the use of ABBV-CLS-7262 in the paediatric population from birth to less than 18 years of age.
Clinical studies	Study 4 (M23-523) Open-label, uncontrolled trial to evaluate pharmacokinetics, safety, activity and acceptability/palatability of ABBV-CLS-7262 in children from 6 months to less than 18 years of age (and adults) with vanishing white matter (VWM) disease.

	Study 5 (M20-474) Double-blind, randomised, placebo controlled trial to evaluate pharmacokinetics, safety and efficacy of ABBV-CLS-7262 in children from 6 years to less than 18 years of age (and adults) with vanishing white matter (VWM) disease. Study 6 (M20-475) Open-label, historical controlled trial to evaluate pharmacokinetics, safety, efficacy, acceptability/palatability of ABBV-CLS-7262 in children from birth to less than 6 years of age with vanishing white matter (VWM) disease.
Modelling and simulation studies	Study 7 Modelling and simulation physiologically based pharmacokinetic (PBPK) study, to evaluate the use of the product in the treatment of vanishing white matter disease in children from birth to less than 18 years of age.
Other studies	Not applicable
Extrapolation plan	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 September 2029.
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.