EMADOC-1700519818-2040316

# European Medicines Agency decision

EMA/PE/0000229973

of 07 April 2025

on the acceptance of a modification of an agreed paediatric investigation plan for fosigotifator sodium tromethamine 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0354/2023 issued on 8 September 2023 and the decision P/0306/2024 issued on 16 August 2024,

Having regard to the application submitted by Abbvie Limited on 18 October 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 31 January 2025, 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) of Regulation (EC) No 1901/2006, 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, 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, 2 Vanwall Road, Maidenhead - SL6 4UB, United Kingdom.

EMADOC-1700519818-1740187
Amsterdam, 31 January 2025

# Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMA/PE/0000229973

## 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, AbbVie Limited submitted to the European Medicines Agency on 18 October 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 and the decision P/0306/2024 issued on 16 August 2024.

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

The procedure started on 25 November 2024.

## 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**<br>Development of granule formulation for paediatric use in children from 6 months of age.<br>**Study 2**<br>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)**<br>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)**<br>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)**<br><br>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.<br><br>**Study 6 (M20-475)**<br><br>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**<br><br>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 December 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.**