EMADOC-1700519818-1759915

European Medicines Agency decision

EMA/PE/0000183137

of 6 December 2024

on the acceptance of a modification of an agreed paediatric investigation plan for encaleret 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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on the acceptance of a modification of an agreed paediatric investigation plan for encaleret 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 European Medicines Agency's decision P/0324/2023 issued on 11 August 2023,

Having regard to the application submitted by BridgeBio Europe B.V. on 8 July 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 18 October 2024, in accordance with Article 22 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 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 encaleret, 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 BridgeBio Europe B.V., Weerdestein 97, 1083 GG – Amsterdam The Netherlands.

EMADOC-1700519818-1620892 Corr[1]
Amsterdam, 18 October 2024

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

EMA/PE/0000183137

Scope of the application

Active substance(s):

Encaleret

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of hypoparathyroidism

Pharmaceutical form(s):

Film-coated tablet

Age-appropriate oral dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

BridgeBio Europe B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, BridgeBio Europe B.V. submitted to the European Medicines Agency on 8 July 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/0324/2023 issued on 11 August 2023.

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

[1] 27 November 2024

The procedure started on 19 August 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 paeditric 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 hypoparathyroidism

2.1.1. Indication(s) targeted by the PIP

Treatment of autosomal dominant hypocalcaemia type 1 (ADH1)

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 dosage form

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of an age-appropriate oral dosage form suitable for children from birth to less than 12 years of age
Non-clinical studies	**Study 2 (CLTX-TX-032)** Dose range-finding juvenile toxicity study to assess toxicokinetics of encaleret in juvenile Sprague-Dawley rats **Study 3 (CLTX-TX-033)** Definitive juvenile toxicity study of encaleret in juvenile Sprague-Dawley rats, including clinical pathology and toxicokinetic evaluation, bone (femur) growth measurements, ex vivo bone densitometry and microscopic sperm evaluation / staging
Clinical studies	**Study 4 (CLTX-305-303)** Open-label, historical-controlled trial to evaluate pharmacokinetics, safety and efficacy of encaleret in children from birth to less than 18 years of age with autosomal dominant hypocalcaemia Type 1 (ADH1)
Modelling and simulation studies	**Study 5** Modelling and simulation study to evaluate the use of encaleret in children from birth to less than 18 years of age with autosomal dominant hypocalcaemia Type 1 (ADH1)

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:	Yes
Date of completion of the paediatric investigation plan:	By December 2028
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.