EMA/28207/2024

European Medicines Agency decision

P/0043/2024

of 14 February 2024

on the acceptance of a modification of an agreed paediatric investigation plan for acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) (IB001) (EMEA-002796-PIP01-20-M02) 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/0043/2022 issued on 10 February 2022, and the decision P/0146/2023 issued on 21 April 2023,

Having regard to the application submitted by IntraBio Ltd on 6 October 2023 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 19 January 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 acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) (IB001), age-appropriate oral liquid 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 IntraBio Ltd., 10 Earlsfort Terrace, D02 T380 - Dublin 2, Ireland.

EMA/PDCO/479727/2023 Corr[1]
Amsterdam, 19 January 2024

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

EMEA-002796-PIP01-20-M02

Scope of the application

Active substance(s):

Acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) (IB001)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of Niemann-Pick disease type C

Pharmaceutical form(s):

Age-appropriate oral liquid dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

IntraBio Ltd

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, IntraBio Ltd submitted to the European Medicines Agency on 6 October 2023 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0043/2022 issued on 10 February 2022, and the decision P/0146/2023 issued on 21 April 2023.

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

The procedure started on 20 November 2023.

[1] 5 February 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.

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 Niemann-Pick disease type C

2.1.1. Indication(s) targeted by the PIP

Treatment of Niemann-Pick disease type C

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 liquid dosage form

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1:** A study to assess compatibility of the formulation with the feeding tube. **Study 2:** Development of a lower strength and volume formulation appropriate for dosing children weighing under 5 kg.
Non-clinical studies	Not applicable.
Clinical studies	**Study 3 (IB1001-201):** Observer-blind, non-comparative trial to evaluate pharmacokinetics, safety and activity of the active substance in children from 6 years to less than 18 years of age with Niemann-Pick Disease Type C. **Study 4 (IB1001-301):** Double-blind, randomised, placebo controlled, cross over trial to evaluate pharmacokinetics, safety and efficacy of active substance in children from 4 years to less than 18 years of age with Niemann-Pick Disease Type C followed by an open-label extension study including children from birth to less than 18 years.

	Study 5 (IB1001-401) *Removed in EMEA-002796-PIP01-20-M02*
Extrapolation, modelling and simulation studies	**Study 6 (TW-2020-IntraB-001):** Modelling and simulation study of population pharmacokinetics (PK) to evaluate the use of the product in children from birth to less than 4 years of age with Niemann-Pick Disease Type C.
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 March 2027
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.