EMA/65204/2022

European Medicines Agency decision

P/0043/2022

of 10 February 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral for acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) (IB1001) (EMEA-002796-PIP01-20) 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 application submitted by IntraBio Ltd. on 24 February 2020 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 21 January 2022, in accordance with Article 17 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 agreement of a paediatric investigation plan and on the granting of a deferral.

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

(3) It is therefore appropriate to adopt a decision granting a 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

A paediatric investigation plan for acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) (IB1001), age-appropriate oral liquid dosage form, 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

A deferral for acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) (IB1001), age-appropriate oral liquid dosage form, 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 granted.

Article 3

This decision is addressed to IntraBio Ltd., 10 Earlsfort Terrace, D02 T380 - Dublin 2, Ireland.

EMA/PDCO/611624/2021
Amsterdam, 21 January 2022

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

EMEA-002796-PIP01-20

Scope of the application

Active substance(s):

acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) (IB1001)

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 16(1) of Regulation (EC) No 1901/2006 as amended, IntraBio Ltd. submitted for agreement to the European Medicines Agency on 24 February 2020 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 31 March 2020.

Supplementary information was provided by the applicant on 18 October 2021. The applicant proposed modifications to the paediatric investigation plan.

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;
- to grant a deferral in accordance with Article 21 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 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	**Number of measures**	**Description**
Quality-related studies	2	**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	0	Not applicable.
Clinical studies	3	**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.

		Study 5 (IB1001-401): Observer-blind, non-comparative trial to evaluate pharmacokinetics, safety and activity of the active substance in children from birth to less than 4 years of age with Niemann-Pick Disease Type C.
Extrapolation, modelling and simulation studies	1	**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	0	Not applicable
Other measures	0	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 April 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes