

MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral

MHRA-102069-PIP01-25

Scope of the Application

Active Substance(s)

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

Condition(s)

Treatment of ataxia telangiectasia

Pharmaceutical Form(s)

Granules for oral solution

Route(s) of Administration

ORAL USE GASTRIC USE

Name / Corporate name of the PIP applicant

Intrabio Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Intrabio Limited submitted to the licensing authority on 04/08/2025 20:03 BST an application for a Paediatric Investigation Plan

The procedure started on 02/09/2025 19:23 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a deferral

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-102069-PIP01-25

Of 03/02/2026 10:41 GMT

On the adopted decision for acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) (MHRA-102069-PIP01-25) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for acetyl-L-leucine ((s)-(acetylamino)-4-methylpentanoic acid) , Granules for oral solution , ORAL USE GASTRIC USE .

This decision is addressed to Intrabio Limited, 10 Earlsfort Terrace, Dublin 2, IRELAND, D02 T380

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of ataxia telangiectasia

2.2 Indication(s) targeted by the PIP:

Treatment of ataxia telangiectasia

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Granules for oral solution

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Study to assess compatibility of the L-acetyl leucine formulation (age-appropriate oral liquid dosage form i.e. granules for oral suspension) with the feeding tube. Study 2 Development of a lower strength and volume L-acetyl leucine formulation appropriate for dosing children weighing under 5 kg.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	3	Study 3 (IB1001-203) Open-label, assessor-blind trial to evaluate pharmacokinetics (PK), safety, and activity of L-acetyl leucine in children from 6 years to less than 18 years of age (and adults) with ataxia-telangiectasia (A-T), with a 110-week extension phase. Study 4 (IB1001-303- Parent study) Double-blind, randomised, placebo controlled, crossover trial to evaluate pharmacokinetics (PK), efficacy, and safety of L-acetyl leucine compared to placebo in children from 4 years to less than 18 years (and in adults) with ataxia-telangiectasia (A-T). Study 5 (IB1001-303- Extension study) Open-label extension study to evaluate the safety, PK, and activity of L-acetyl leucine in children from birth to less than 18 years of age (and in adults) with ataxia-telangiectasia (A-T).
Extrapolation, Modeling & Simulation Studies	1	Study 6 (Study AT-PopPK) Use of population PK (PopPK) to confirm or modify the dosing regimen of L-acetyl leucine for children from birth

		to less than 4 years of age compared to the regimen used in clinical trials.
Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	30/06/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes