

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

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral.

MHRA-101785-PIP01-25-M01

Scope of the Application

Active Substance(s)

Repinatrabit

Condition(s)

Treatment of hyperphenylalaninemia

Pharmaceutical Form(s)

Film-coated tablet; Granules; Age appropriate oral formulation

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Otsuka Pharmaceutical Netherlands B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Otsuka Pharmaceutical Netherlands B.V. submitted to the licensing authority on 22/05/2026 20:04 BST an application for a Modification

The procedure started on 15/06/2026 10:04 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 accept change(s) to the agreed paediatric investigation plan and to the 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-101785-PIP01-25-M01

Of 02/09/2026 09:47 BST

On the adopted decision for Repinatrabit (MHRA-101785-PIP01-25-M01) 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 modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan).

This decision applies to a Modification for Repinatrabit, Film-coated tablet; Granules; Age appropriate oral formulation , ORAL USE .

This decision is addressed to Otsuka Pharmaceutical Netherlands B.V., Herikerbergweg 292, Amsterdam, NETHERLANDS, 1101 CT

ANNEX I

1. Waiver

1.1 Condition:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of hyperphenylalaninemia

2.2 Indication(s) targeted by the PIP:

Reduction of plasma phenylalanine concentrations in patients with phenylketonuria who have uncontrolled plasma phenylalanine concentrations.

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

All subsets of the paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film-coated tablet Granules Age-appropriate oral formulation

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of age-appropriate oral formulation for use in children below 12 years of age or adolescents unable to swallow tablets.
Non-Clinical Studies	1	Study 2 Definitive juvenile animal study
Clinical Studies	3	Study 3 (JNT517-201) Randomised, double-blind, placebo-controlled study to evaluate efficacy and safety of repinatrabit in adolescents from 12 years to less than 18 years of age with phenylketonuria (PKU). Study 4 (JNT517-301) Randomised, double-blind, placebo-controlled, two-part study to evaluate efficacy and safety of repinatrabit in children from 4 years to less than 18 years of age (and adults) with PKU. Study 5 (JNT517-302) Open-label, single-arm study to evaluate safety and efficacy of repinatrabit in paediatric participants from birth to less than 4 years of age with PKU.
Extrapolation, Modeling & Simulation Studies	3	Study 6 (JNT517-M&S-1) Modelling and simulation analyses to support dosing of repinatrabit in children from 4 years to less than 18 years of age with PKU. Study 7 (JNT517-M&S-2) Modelling and simulation analyses to support dosing of repinatrabit in children from birth to less than 4 years of age with PKU.

		Extrapolation Plan Studies 3, 4 and 5 are part of the extrapolation plan of efficacy data from adults, adolescents and paediatric patients to the paediatric population from birth to less than 4 years of age with hyperphenylalaninemia.
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:	30/06/2033
Deferral of one or more studies contained in the paediatric investigation plan:	Yes