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 (MHRA-102400-PIP01-26-M02) and to the deferral

MHRA-102400-PIP01-26-M03

Scope of the Application

Active Substance(s)

ODEVIXIBAT SESQUIHYDRATE

Condition(s)

Treatment of Alagille syndrome (ALGS)

Pharmaceutical Form(s)

Capsule, hard

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Ipsen Pharma

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Ipsen Pharma submitted to the licensing authority on 16/03/2026 14:00 GMT an application for a Modification

The procedure started on 30/03/2026 08:18 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-102400-PIP01-26-M03

Of 22/05/2026 16:23 BST

On the adopted decision for ODEVIXIBAT SESQUIHYDRATE (MHRA-102400-PIP01-26-M03) 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 ODEVIXIBAT SESQUIHYDRATE, Capsule, hard , ORAL USE .

This decision is addressed to Ipsen Pharma, 70 rue Balard, Paris, FRANCE, 75015

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Alagille syndrome

2.2 Indication(s) targeted by the PIP:

Treatment of Alagille syndrome

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):

Capsule, hard

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Compatibility study to determine, when mixing pellets with food, the recovery of drug substance after dispersion in semi-liquids or liquids. Study 2 Deleted during procedure MHRA-102400-PIP01-26-M03.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 3 (A4250-012) Double-blind, randomised, placebo-controlled trial to evaluate the safety and efficacy of odevixibat in children from birth to less than 18 years of age (and adults) with Alagille syndrome.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
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:	31/12/2022
Deferral of one or more studies contained in the paediatric investigation plan:	No