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 and grant a waiver

MHRA-101278-PIP01-23

Scope of the Application

Active Substance(s)

oveporexton

Condition(s)

Treatment of narcolepsy., Treatment of idiopathic hypersomnia.

Pharmaceutical Form(s)

Film coated tablet

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Takeda UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Takeda UK Limited submitted to the licensing authority on 31/01/2024 10:19 GMT an application for a Paediatric Investigation Plan

The procedure started on 06/08/2024 15:28 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 and grant a waiver

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-101278-PIP01-23

Of 23/04/2025 17:30 BST

On the adopted decision for oveporexton (MHRA-101278-PIP01-23) 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 oveporexton, Film coated tablet , ORAL USE .

This decision is addressed to Takeda UK Limited, 1 Kingdom Street, London, UNITED KINGDOM, W2 6BD

ANNEX I

1. Waiver

1.1 Condition:

Condition 1: Treatment of narcolepsy The waiver applies / applied to: Paediatric Subset(s): All subsets of the paediatric population from birth to less than 6 years of age Pharmaceutical form(s): Film coated tablet Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s). Condition 2: Treatment of idiopathic hypersomnia. The waiver applies / applied to: Paediatric Subset(s): All subsets of the paediatric population from birth to less than 18 years of age Pharmaceutical form(s): Film coated tablet Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be ineffective.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of narcolepsy

2.2 Indication(s) targeted by the PIP:

Treatment of narcolepsy type 1 (NT1)

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

The paediatric population from 6 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film coated tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Acceptability/palatability study.
Non-Clinical Studies	2	Study 2 (SBL010-546) Dose-range finding juvenile study in rats. Study 3 (SBL010-561) Definitive juvenile toxicology study in rats.
Clinical Studies	2	Study 4 (TAK-861-1012) Open-label single dose trial to evaluate pharmacokinetics, safety and acceptability /palatability of oveporexton in children from 6 years to less than 18 years of age with narcolepsy type 1 (NT1). Study 5 (TAK-861-3004) Double-blind, randomised, multiple dose, placebo controlled withdrawal trial to evaluate pharmacokinetics, safety and efficacy of oveporexton in children from 6 years to less than 18 years of age with narcolepsy type 1 (NT1), with an open label extension period to evaluate long term safety and effect maintenance.
Extrapolation, Modeling & Simulation Studies	3	Study 6 Development of a physiologically based pharmacokinetic model to support initial paediatric dose determination. Study 7 Development of a population pharmacokinetic model to predict paediatric doses to be used in planned clinical studies. Study

		8 Development of an exposure-response model to predict paediatric doses to be used in planned clinical studies.
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:	31/12/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes