



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1944371

European Medicines Agency decision

EMEA-003553-PIP01-23

of 19 March 2025

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for ovesporexton 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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on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for oreporexton in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Takeda Pharma A/S on 14/12/2023 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 31 January 2025, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 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 on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.
- (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.
- (4) It is therefore appropriate to adopt a decision granting a waiver.

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 for ovesporexton, 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 ovesporexton, 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

A waiver for ovesporexton, 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 4

This decision is addressed to Takeda Pharma A/S, Delta Park 45, 2665 Vallensbaek Strand, Hovedstaden, Denmark.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1775654
Amsterdam, 31 January 2025 Corr¹

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

EMA-003553-PIP01-23

Scope of the application

Active substance(s):

Oveporexton

Invented name and authorisation status:

See Annex II

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 Pharma A/S

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Takeda Pharma A/S submitted for agreement to the European Medicines Agency on 14 December 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 22 January 2024.

¹ Correction 14 February 2025



Supplementary information was provided by the applicant on 17 October 2024. 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;
 - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients and with Article 11(1)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver 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 annexes 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

1.1. Condition:

Treatment of narcolepsy

The waiver applies to:

- all subsets of the paediatric population from birth to less than 6 years of age;
- film-coated tablet, oral use;
- 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).

1.2. Condition:

Treatment of idiopathic hypersomnia

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- all pharmaceutical form(s), all route(s) of administration;
- on the grounds that the specific medicinal product is likely to be ineffective.

2. Paediatric investigation plan

2.1. Condition:

Treatment of narcolepsy

2.1.1. Indication(s) targeted by the PIP

Treatment of narcolepsy type 1 (NT1)

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 6 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet, oral use

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Acceptability/palatability study
Non-clinical studies	Study 2

	<p>Dose-range finding juvenile study in rats.</p> <p>Study 3</p> <p>Definitive juvenile toxicology study in rats.</p>
Clinical studies	<p>Study 4</p> <p>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).</p> <p>Study 5</p> <p>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.</p>
Modelling and simulation analyses	<p>Study 6</p> <p>Development of a physiologically based pharmacokinetic model to support initial paediatric dose determination</p> <p>Study 7</p> <p>Development of a population pharmacokinetic model to predict paediatric doses to be used in planned clinical studies</p> <p>Study 8</p> <p>Development of an exposure-response model to predict paediatric doses to be used in planned clinical studies</p>
Other studies	Not applicable
Extrapolation plan	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 December 2031
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.