

EMA/472032/2018

European Medicines Agency decision

P/0252/2018

of 15 August 2018

on the acceptance of a modification of an agreed paediatric investigation plan for osilodrostat (EMEA-000315-PIP02-15-M02) 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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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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency²,

Having regard to the European Medicines Agency's decision P/0011/2016 issued on 29 January 2016 and the decision P/0064/2018 issued on 16 March 2018,

Having regard to the application submitted by Novartis Europharm Limited on 9 April 2018 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 29 June 2018, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

¹ OJ L 378, 27.12.2006, p.1.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for osilodrostat, film-coated tablet, oral use, including changes to the deferral, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0011/2016 issued on 29/01/2016, including subsequent modifications thereof.

Article 3

This decision is addressed to Novartis Europharm Limited, Vista Building Elm Park, Merrion Road, 4 – Dublin, Ireland.

EMA/PDCO/237328/2018

London, 29 June 2018

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-000315-PIP02-15-M02

Scope of the application

Osilodrostat

Condition(s):

Treatment of adrenal cortical hyperfunction

Pharmaceutical form(s):

Film-coated tablet

Route(s) of administration:

Oral use

Name / corporate name of the PIP applicant:

Novartis Europharm Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Novartis Europharm Limited submitted to the European Medicines Agency on 9 April 2018 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0011/2016 issued on 29 January 2016 and the decision P/0064/2018 issued on 16 March 2018.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 2 May 2018.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
 - to agree to changes to the paediatric investigation plan and to the deferral in the scope set out in the Annex I of this opinion.

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

2. The measures and timelines of the 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 annex 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 adrenal cortical hyperfunction

The waiver applies to:

- the paediatric population from birth to less than 6 years of age;
- film-coated tablet, oral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition

Treatment of adrenal cortical hyperfunction

2.1.1. Indication(s) targeted by the PIP

Treatment of Cushing's disease in adolescents and children

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

From 6 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	2	Study 1 4-week dose-ranging study in juvenile animals Study 2 10-week toxicity study in juvenile animals
Clinical studies	1	Study 3 Open-label, non-comparative study to evaluate the tolerability, pharmacokinetics and pharmacodynamics of osilodrostat in children and adolescent patients with Cushing disease.

Extrapolation, modelling and simulation studies	1	Study 4 Population PK modelling and simulation study to evaluate the use of osilodrostat in children and adolescent patients from 6 to 18 years of age.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By February 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes