EMA/430844/2017

European Medicines Agency decision

P/0213/2017

of 9 August 2017

on the acceptance of a modification of an agreed paediatric investigation plan for obeticholic acid (6 alpha-ethylchenodeoxycholic acid) (Ocaliva), (EMEA-001304-PIP02-13-M03) 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 726/2004[1],

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 Agency[2],

Having regard to the European Medicines Agency's decision P/0175/2014 issued on 11 July 2014, the decision P/0038/2015 issued on 20 March 2015 and the decision P/0310/2015 issued on 21 December 2015,

Having regard to the application submitted by Intercept Pharma Ltd. on 3 April 2017 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 23 June 2017, 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 obeticholic acid (6 alpha-ethylchenodeoxycholic acid) (Ocaliva), coated tablet, 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 is addressed to Intercept Pharma Ltd., 2 Pancras Square, N1C 4AG - London, United Kingdom.

EMA/PDCO/241491/2017
London, 23 June 2017

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-001304-PIP02-13-M03

Scope of the application

Active substance(s):

Obeticholic acid (6 alpha-ethylchenodeoxycholic acid)

Invented name:

Ocaliva

Condition(s):

Treatment of primary biliary cirrhosis

Treatment of biliary atresia

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Coated tablet

Tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Intercept Pharma Ltd.

Information about the authorised medicinal product:

See Annex II

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Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Intercept Pharma Ltd. submitted to the European Medicines Agency on 3 April 2017 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/0175/2014 issued on 11 July 2014, the decision P/0038/2015 issued on 20 March 2015 and the decision P/0310/2015 issued on 21 December 2015.

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

The procedure started on 25 April 2017.

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 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 primary biliary cirrhosis

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- coated tablet, tablet, oral use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition

Treatment of biliary atresia

2.1.1. Indication(s) targeted by the PIP

Treatment of biliary atresia

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Tablet

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	2	**Study 1** Development of an age appropriate oral solid dosage form (1.5 mg mini-tablet). **Study 2** Development of an age appropriate oral solid dosage form (0.1 mg mini-tablet).
Non-clinical studies	2	**Study 3** Dose range-finding juvenile toxicity study. **Study 4** Definitive juvenile toxicity study.

Clinical studies	3	**Study 5** Open label, single and multiple sequential dose study to evaluate safety, tolerability and pharmacokinetics of obeticholic acid in children and adolescents with biliary atresia. **Study 6** Deleted during procedure EMEA-001304-PIP02-13-M03 **Study 7** Deleted during procedure EMEA-001304-PIP02-13-M03 **Study 8** Natural history data collection study from biliary atresia registries. **Study 9** Randomised, placebo-controlled study to evaluate the efficacy, safety, tolerability, pharmacokinetics, and pharmacodynamics of obeticholic acid in children from birth to less than 18 years with biliary atresia, post-hepatoportoenterostomy.
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
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:	No
Date of completion of the paediatric investigation plan:	By December 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of primary biliary cirrhosis

Authorised indication(s):

OCALIVA is indicated for the treatment of primary biliary cholangitis (also known as primary biliary cirrhosis) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA.

Authorised pharmaceutical form(s):

Film coated tablets

Authorised route(s) of administration:

Oral use