EMA/160747/2015

European Medicines Agency decision

P/0038/2015

of 20 March 2015

on the acceptance of a modification of an agreed paediatric investigation plan for obeticholic acid (6 alpha-ethylchenodeoxycholic acid) (EMEA-001304-PIP02-13-M01) 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,

Having regard to the application submitted by Intercept Italia s.r.l. on 12 November 2014 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 13 February 2015, 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), coated tablet, tablet, oral suspension, 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 Italia s.r.l., c/o Studio Enrico Pellegrini, Via Settevalli, 556, 06129 - Perugia, Italy.

Done at London, 20 March 2015

For the European Medicines Agency
Jordi Llinares Garcia
Head of Division (ad interim)
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/740382/2014

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

EMEA-001304-PIP02-13-M01

Scope of the application

Active substance(s):

Obeticholic acid (6 alpha-ethylchenodeoxycholic acid)

Condition(s):

Treatment of primary biliary cirrhosis

Treatment of biliary atresia

Pharmaceutical form(s):

Coated tablet

Tablet

Oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Intercept Italia s.r.l.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Intercept Italia s.r.l. submitted to the European Medicines Agency on 12 November 2014 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 application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 16 December 2014.

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Scope of the modification

Some 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.

London, 13 February 2015

On behalf of the Paediatric Committee
Dr Dirk Mentzer, Chairman
(Signature on file)

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 suspension, 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

Oral suspension

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	2	**Study 1** Development of an age appropriate oral solid dosage form. **Study 2** Development of an age appropriate oral liquid dosage form.
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** Randomised, double-blind, placebo controlled trial to assess safety and efficacy of obeticholic acid in children and adolescents from 2 years to less than 18 years with biliary atresia. **Study 7** Randomised, double-blind, placebo controlled trial to assess safety and efficacy of obeticholic acid in children from birth to less than 2 years with biliary atresia.
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 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes