EMA/344720/2014

European Medicines Agency decision

P/0175/2014

of 11 July 2014

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for obeticholic acid (6 alpha-ethylchenodeoxycholic acid), (EMEA-001304-PIP02-13) 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 application submitted by Intercept Italia s.r.l. on 2 August 2013 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 23 May 2014, in accordance with Article 18 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 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for obeticholic acid (6 alpha-ethylchenodeoxycholic acid), coated tablet, tablet, oral suspension, oral use, 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 for obeticholic acid (6 alpha-ethylchenodeoxycholic acid), coated tablet, tablet, oral suspension, oral use, 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 3

A waiver for obeticholic acid (6 alpha-ethylchenodeoxycholic acid), coated tablet, tablet, oral suspension, oral use, 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 4

This decision is addressed to Intercept Italia s.r.l., c/o Studio Enrico Pellegrini, Via Settevalli, 556, 06129 - Perugia, Italy.

Done at London, 11 July 2014

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/146486/2014

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

EMEA-001304-PIP02-13

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 16(1) of Regulation (EC) No 1901/2006 as amended, Intercept Italia s.r.l. submitted for agreement to the European Medicines Agency on 2 August 2013 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 12 September 2013.

Supplementary information was provided by the applicant on 3 March 2014. The applicant proposed modifications to the paediatric investigation plan and requested a waiver.

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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 18 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)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended occurs only in adult populations.

   The Icelandic and the Norwegian Paediatric Committee members agree 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 annex and appendix.

London, 23 May 2014

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