EMA/360346/2021

European Medicines Agency decision

P/0246/2021

of 9 July 2021

on the agreement of a paediatric investigation plan for odevixibat, (EMEA-002054-PIP03-20) 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 Albireo AB on 10 August 2020 under Article 16(1) of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 21 May 2021, in accordance with Article 17 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 agreement of a paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

[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 odevixibat, capsule, hard, age-appropriate oral liquid dosage form, 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

This decision is addressed to Albireo AB, Arvid Wallgrens backe 20, 413 46 – Gothenburg, Sweden.

EMA/PDCO/136061/2021
Amsterdam, 21 May 2021

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan

EMEA-002054-PIP03-20

Scope of the application

Active substance(s):

Odevixibat

Condition(s):

Treatment of Alagille syndrome

Pharmaceutical form(s):

Capsule, hard

Age-appropriate oral liquid dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Albireo AB

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Albireo AB submitted for agreement to the European Medicines Agency on 10 August 2020 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 15 September 2020.

Supplementary information was provided by the applicant on 11 February 2021. 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.

   The Norwegian Paediatric Committee member 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 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

Not applicable.

1.1. Condition:

Treatment of Alagille syndrome

2. Paediatric investigation plan

2.1. Condition:

Treatment of Alagille syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of Alagille syndrome

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)

Capsule, hard

Age-appropriate oral liquid dosage form

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	2	**Study 1** (identical to quality-related study included in opinion for EMEA-002054-PIP02-18) Compatibility study to determine, when mixing pellets with food, the recovery of drug substance after dispersion in semi-liquids or liquids. **Study 2** (identical to quality-related study included in opinion for EMEA-002054-PIP02-18) Development of an age appropriate oral liquid formulation.
Non-clinical studies	0	Not applicable.

Clinical studies	1	**Study 3** Double-blind, randomised, placebo-controlled trial to evaluate the safety and efficacy of odevixibat in children from birth to less than 18 years of age (and adults) with Alagille syndrome.
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:	No