EMA/912385/2022

European Medicines Agency decision

P/0515/2022

of 6 December 2022

on the acceptance of a modification of an agreed paediatric investigation plan for odevixibat (Bylvay), (EMEA-002054-PIP03-20-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 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0246/2021 issued on 9 July 2021 and the decision P/0368/2022 issued on 9 September 2022,

Having regard to the application submitted by Albireo AB on 8 August 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan and proposing a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 11 November 2022, in accordance with Article 22 of Regulation (EC) No 1901/2006 and Article 21 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 acceptance of changes to the agreed paediatric investigation plan and on the granting of a deferral.

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

(3) It is therefore appropriate to adopt a decision granting a deferral.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for odevixibat (Bylvay), capsule, hard, age-appropriate oral liquid dosage form , oral use, 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

A deferral for odevixibat (Bylvay), capsule, hard, age-appropriate oral liquid dosage form, oral use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0306/2017 issued on 31 October 2017, including subsequent modifications thereof.

Article 4

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

EMA/PDCO/687161/2022
Amsterdam, 11 November 2022

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

EMEA-002054-PIP03-20-M02

Scope of the application

Active substance(s):

Odevixibat

Invented name and authorisation status:

See Annex II

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 22 of Regulation (EC) No 1901/2006 as amended, Albireo AB submitted to the European Medicines Agency on 8 August 2022 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0246/2021 issued on 9 July 2021 and the decision P/0368/2022 issued on 9 September 2022.

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

The procedure started on 12 September 2022.

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 in the scope set out in the Annex I of this opinion;
   - to grant a deferral, the details of which are set out in the Annex I of this opinion.

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

2. The measures and timelines of the paediatric investigation plan 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

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	Description
Quality-related studies	**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	Not applicable
Clinical studies	**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	Not applicable
Other studies	Not applicable
Other measures	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

Information provided by the applicant:

Condition(s) and authorised indication(s)

1. Treatment of progressive familial intrahepatic cholestasis

Authorised indication(s):

- Treatment of progressive familial intrahepatic cholestasis (PFIC) in patients aged 6 months or older
  - Invented name(s): Bylvay
  - Authorised pharmaceutical form(s): Hard capsule
  - Authorised route(s) of administration: Oral use
  - Authorised via centralised procedure