EMA/206556/2022

# European Medicines Agency decision

P/0147/2022

of 13 May 2022

on the acceptance of a modification of an agreed paediatric investigation plan for odevixibat (Bylvay), (EMEA-002054-PIP01-16-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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# European Medicines Agency decision

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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/0306/2017 issued on 31 October 2017, decision P/0316/2019 issued on 11 September 2019, and decision P/0377/2020 issued on 11 September 2020,

Having regard to the application submitted by Albireo AB on 20 December 2021 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 25 March 2022, 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, 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, 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 Albireo AB, Arvid Wallgrens backe 20, 413 46 – Göteborg, Sweden.

EMA/PDCO/8331/2022
Amsterdam, 25 March 2022

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

EMEA-002054-PIP01-16-M03

## Scope of the application

**Active substance(s):**

Odevixibat

**Invented name:**

Bylvay

**Condition(s):**

Treatment of progressive familial intrahepatic cholestasis

**Authorised indication(s):**

See Annex II

**Pharmaceutical form(s):**

Capsule, hard

**Route(s) of administration:**

Oral use

**Name/corporate name of the PIP applicant:**

Albireo AB

**Information about the authorised medicinal product:**

See Annex II

## Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Albireo AB submitted to the European Medicines Agency on 20 December 2021 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0306/2017 issued on 31 October 2017, decision P/0316/2019 issued on 11 September 2019, and decision P/0377/2020 issued on 11 September 2020.

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

The procedure started on 31 January 2022.

## Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

A pharmaceutical form was removed.

## 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 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

# 2. Paediatric investigation plan

## *2.1. Condition*

Treatment of Progressive Familial Intrahepatic Cholestasis

### 2.1.1. Indication(s) targeted by the PIP

Treatment of Progressive Familial Intrahepatic Cholestasis

### 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

### 2.1.4. Measures

| Area | Description |
|---|---|
| Quality-related studies | **Study 1**<br>Study deleted in procedure EMEA-002054-PIP01-16-M03 |
| Non-clinical studies | Not applicable |
| Clinical studies | **Study 2**<br>Single and multiple dosing open-label study to evaluate the safety and efficacy of odevixibat in children from 1 year to <18 years with cholestatic pruritus (A4250-003).<br>**Study 3**<br>Double-blind, randomised, placebo-controlled study to demonstrate the efficacy and safety of repeated daily doses of odevixibat in children from 6 months to less than 18 years with Progressive Familial Intrahepatic Cholestasis types 1 and 2. (A4250-005)<br>**Study 4** (study deleted during procedure EMEA-002054-PIP01-16-M01)<br>**Study 5** (new study added during procedure EMEA-002054-PIP01-16-M01)<br>Open-label extension study to evaluate the long-term efficacy and safety of odevixibat in children from birth to less than 18 years with |

| | |
|---|---|
| | progressive familial intrahepatic cholestasis (PFIC) types 1 and 2 from baseline to week 72. (A4250-008) |
| 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 March 2024 |
| 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 Progressive familial intrahepatic cholestasis (PFIC)

Authorised indication(s):

- Bylvay is indicated for the treatment of progressive familial intrahepatic cholestasis (PFIC) in patients aged 6 months or older (see sections 4.4 and 5.1).

## Authorised pharmaceutical form(s):

Capsule, hard

## Authorised route(s) of administration:

Oral use