EMA/98347/2024

# European Medicines Agency decision

P/0102/2024

of 12 April 2024

on the acceptance of a modification of an agreed paediatric investigation plan for ruxolitinib (phosphate), (Opzelura, Jakavi), (EMEA-002618-PIP02-20-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 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/0145/2021 issued on 16 April 2021,

Having regard to the application submitted by Incyte Biosciences Distribution B.V. on 17 November 2023 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 23 February 2024, 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 ruxolitinib (phosphate), (Opzelura, Jakavi), cream, cutaneous 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 covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0349/2017 issued on 1 December 2017, and the decision P/0061/2018 issued on 16 March 2018, including subsequent modifications thereof.

***Article 3***

This decision is addressed to Incyte Biosciences Distribution B.V., Paasheuvelweg 25, 1105 BP – Amsterdam, The Netherlands.

EMA/PDCO/542177/2023
Amsterdam, 23 February 2024

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

EMEA-002618-PIP02-20-M01

## Scope of the application

**Active substance(s):**

Ruxolitinib (phosphate)

**Invented name and authorisation status:**

See Annex II

**Condition(s):**

Treatment of vitiligo

**Pharmaceutical form(s):**

Cream

**Route(s) of administration:**

Cutaneous use

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

Incyte Biosciences Distribution B.V.

## Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Incyte Biosciences Distribution B.V. submitted to the European Medicines Agency on 17 November 2023 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/0145/2021 issued on 16 April 2021.

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

The procedure started on 3 January 2024.

## 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 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 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 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

## *1.1. Condition:*

Treatment of vitiligo

The waiver applies to:

- the paediatric population from birth to less than 6 years of age;
- cream; cutaneous use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

# 2. Paediatric investigation plan

## *2.1. Condition:*

Treatment of vitiligo

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

Topical treatment of vitiligo

### 2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 6 years to less than 18 years of age

### 2.1.3. Pharmaceutical form(s)

Cream

### 2.1.4. Measures

| Area | Description |
|---|---|
| Quality-related studies | Not applicable. |
| Non-clinical studies | Not applicable. |
| Clinical studies | **Study 1 (INCB 18424-306)**<br>Double-blind, randomised, placebo-controlled trial to evaluate efficacy and safety of ruxolitinib cream in adolescents from 12 years of age (and adults) with non-segmental vitiligo<br>**Study 2 (INCB 18424-307)**<br>Double-blind, randomised, placebo-controlled trial to evaluate efficacy and safety of ruxolitinib cream in adolescents from 12 years of age (and adults) with non-segmental vitiligo |

| | |
|---|---|
| | **Study 3 (INCB 18424-308)**<br><br>Double-blind, randomised, placebo-controlled extension trial to evaluate long-term efficacy and safety of ruxolitinib cream in adolescents from 12 years of age (and adults) with non-segmental vitiligo<br><br>**Study 4 (INCB 18424-309)**<br><br>Double-blind, randomised, placebo-controlled trial to evaluate efficacy and safety of ruxolitinib cream in children from 6 years to less than 12 years of age with non-segmental vitiligo |
| 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: | Yes |
| Date of completion of the paediatric investigation plan: | By December 2027 |
| 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 vitiligo

Authorised indication(s):

- Opzelura is indicated for the treatment of non-segmental vitiligo with facial involvement in adults and adolescents from 12 years of age.
  - Invented name(s): Opzelura
  - Authorised pharmaceutical form(s): Cream
  - Authorised route(s) of administration: Cutaneous use
  - Authorised via centralised procedure
  - Marketing Authorisation Holder: Incyte Biosciences Distribution B.V.

2. Treatment of myelofibrosis

Authorised indication(s):

Jakavi is indicated for the treatment of disease-related splenomegaly or symptoms in adult patients with primary myelofibrosis (also known as chronic idiopathic myelofibrosis), post polycythaemia vera myelofibrosis or post essential thrombocythaemia myelofibrosis.

- Invented name(s): Jakavi
- Authorised pharmaceutical form(s): Tablet
- Authorised route(s) of administration: Oral use
- Authorised via centralised procedure
- Marketing Authorisation Holder: Novartis Europharm Limited

3. Treatment of polycythaemia vera

Authorised indication(s):

Jakavi is indicated for the treatment of adult patients with polycythaemia vera who are resistant to or intolerant of hydroxyurea.

- Invented name(s): Jakavi
- Authorised pharmaceutical form(s): Tablet
- Authorised route(s) of administration: Oral use
- Authorised via centralised procedure
- Marketing Authorisation Holder: Novartis Europharm Limited

4. Treatment of graft versus host disease

Authorised indication(s):

Jakavi is indicated for the treatment of patients aged 12 years and older with acute graft versus host disease or chronic graft versus host disease who have inadequate response to corticosteroids or other systemic therapies.

- Invented name(s): Jakavi
- Authorised pharmaceutical form(s): Tablet
- Authorised route(s) of administration: Oral use
- Authorised via centralised procedure
- Marketing Authorisation Holder: Novartis Europharm Limited