EMA/879/2024

# European Medicines Agency decision

P/0002/2024

of 3 January 2024

on the acceptance of a modification of an agreed paediatric investigation plan for mozafancogene autotemcel, (EMEA-002578-PIP01-19-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/0114/2020 issued on 18 March 2020,

Having regard to the application submitted by Rocket Pharmaceuticals, Inc. on 21 August 2023 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver and proposing a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 15 December 2023, 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 mozafancogene autotemcel, dispersion for infusion, intravenous 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 mozafancogene autotemcel, dispersion for infusion, intravenous 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 is addressed to Rocket Pharmaceuticals, Inc, 9 Cedarbrook Drive, 08512 - Cranbury, NJ, United States.

EMA/PDCO/419520/2023
Amsterdam, 15 December 2023

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

EMEA-002578-PIP01-19-M01

## Scope of the application

**Active substance(s):**

Mozafancogene autotemcel

**Invented name and authorisation status:**

See Annex II

**Condition(s):**

Treatment of Fanconi anaemia subtype A

**Pharmaceutical form(s):**

Dispersion for infusion

**Route(s) of administration:**

Intravenous use

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

Rocket Pharmaceuticals, Inc.

## Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Rocket Pharmaceuticals, Inc. submitted to the European Medicines Agency on 21 August 2023 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0114/2020 issued on 18 March 2020.

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

The procedure started on 16 October 2023.

## Scope of the modification

Some 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 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 Fanconi anaemia subtype A

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- dispersion for infusion, intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

# 2. Paediatric investigation plan

## *2.1. Condition:*

Treatment of Fanconi anaemia subtype A

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

Treatment of Fanconi anaemia subtype A

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

From 1 to less than 18 years of age

### 2.1.3. Pharmaceutical form(s)

Dispersion for infusion

### 2.1.4. Measures

| **Area** | **Number of measures** | **Description** |
|---|---|---|
| Quality-related studies | 0 | Not applicable. |
| Non-clinical studies | 0 | Not applicable. |
| Clinical studies | 4 | **Study 1**<br>Open-label, uncontrolled trial to evaluate safety and efficacy of autologous CD34+ enriched cells from patients with Fanconi anaemia subtype A transduced *ex vivo* with a lentiviral vector carrying the *FANCA* gene (PGK-FANCA-WPRE) [hereafter referred to as RP-L102] in children and adolescents from 1 year to less than 18 years of age (and young adults) with Fanconi anaemia subtype A (FANCOLEN-I). |

| | | |
|---|---|---|
| | | **Study 2**<br><br>Open-label, uncontrolled trial to evaluate safety and efficacy of RP-L102 in children and adolescents from 1 year to less than 18 years of age with Fanconi anaemia subtype A (FANCOLEN-II).<br><br>**Study 3**<br><br>Open-label, uncontrolled trial to evaluate safety and efficacy of RP-L102 in children from 1 year to less than 13 years of age with Fanconi anaemia subtype A (RP-L102-0418).<br><br>**Study 4**<br><br>Open-label, uncontrolled trial to evaluate safety and efficacy of RP-L102 in children and adolescents from 1 year to less than 18 years of age (and adults) with Fanconi anaemia subtype A (RP-L102-0319). |
| Extrapolation, modelling and simulation studies | 0 | Not applicable. |
| Other studies | 1 | **Study 5**<br><br>Comprehensive literature-based review of FA mosaic patients, as well as the spontaneous evolution of untreated FA patients**.** |
| 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: | Yes |
| Date of completion of the paediatric investigation plan: | By May 2026 |
| 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:***

**The product is not authorised anywhere in the European Community.**