EMA/628702/2020

# European Medicines Agency decision

P/0503/2020

of 22 December 2020

on the acceptance of a modification of an agreed paediatric investigation plan for calcifediol (Rayaldee) (EMEA-002093-PIP02-17-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.**

# 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 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 European Medicines Agency's decision P/0339/2018 issued on 8 November 2018,

Having regard to the application submitted by Vifor Fresenius Medical Care Renal Pharma France on 30 July 2020 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 13 November 2020, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

***Article 1***

Changes to the agreed paediatric investigation plan for calcifediol (Rayaldee), age-appropriate oral liquid dosage form, prolonged-release capsule, 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 Vifor Fresenius Medical Care Renal Pharma France, 100-101 Terrasse Boieldieu, Tour Franklin La Défense 8, 92042 - Paris La Défense Cedex, France.

EMA/PDCO/440561/2020
Amsterdam, 13 November 2020

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

EMEA-002093-PIP02-17-M01

## Scope of the application

**Active substance(s):**

Calcifediol

**Invented name:**

Rayaldee

**Condition(s):**

Treatment of secondary hyperparathyroidism (SHPT)

**Authorised indication(s):**

See Annex II

**Pharmaceutical form(s):**

Age-appropriate oral liquid dosage form

Prolonged-release capsule

**Route(s) of administration:**

Oral use

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

Vifor Fresenius Medical Care Renal Pharma France

**Information about the authorised medicinal product:**

See Annex II

# Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Vifor Fresenius Medical Care Renal Pharma France submitted to the European Medicines Agency on 30 July 2020 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/0339/2018 issued on 8 November 2018.

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

The procedure started on 15 September 2020.

## 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 the paediatric investigation plan and to the deferral in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member 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 secondary hyperparathyroidism

The waiver applies to:

- the paediatric population from birth to less than 6 months;
- age-appropriate oral liquid dosage form, prolonged-release capsule, oral use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

# 2. Paediatric investigation plan

## *2.1. Condition:*

Treatment of secondary hyperparathyroidism

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

Treatment of secondary parathyroidism (SHPT) in non-dialysis chronic kidney disease (ND-CKD) patients with low serum 25-hydroxivitamin D levels

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

From 6 months to less than 18 years of age

### 2.1.3. Pharmaceutical form(s)

Age-appropriate oral liquid dosage form

Prolonged-release capsule

### 2.1.4. Measures

| Area | Number of measures | Description |
|---|---|---|
| Quality-related studies | 1 | **Study 1**<br>Development of an age-appropriate oral liquid formulation |
| Non-clinical studies | 0 | Not applicable |
| Clinical studies | 2 | **Study 2 - CTAP101-CL-3007**<br>Double-blind, randomised, multiple dose, placebo controlled study to evaluate pharmacokinetics, safety, efficacy, of calcifediol in children from 8 to less than 18 years of age with secondary hyperparathyroidism with stage 3 or 4 chronic kidney disease and low vitamin D |

| | | |
|---|---|---|
| | | levels.<br>**Study 3**<br>Open-label, multiple dose, non-controlled study to evaluate safety and efficacy of calcifediol in children from 6 months to less than 8 years of age with secondary hyperparathyroidism with stage 3 or 4 chronic kidney disease and low vitamin D levels |
| Extrapolation, modelling and simulation studies | 1 | **Study 4**<br>Population PK study to evaluate the use of the product in children from 6 months to less than 18 years of age with secondary hyperparathyroidism |
| 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 August 2027 |
| 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 secondary hyperparathyroidism (SHPT)

Authorised indication:

- Treatment of secondary hyperparathyroidism (SHPT) in adults with chronic kidney disease (CKD) Stage 3 or 4 and vitamin D insufficiency or deficiency.

## Authorised pharmaceutical form(s):

Prolonged-release capsule, soft

## Authorised route(s) of administration:

Oral use