EMA/267114/2024

European Medicines Agency decision

P/0211/2024

of 14 June 2024

on the acceptance of a modification of an agreed paediatric investigation plan for calcifediol (Rayaldee), (EMEA-002093-PIP02-17-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/0339/2018 issued on 8 November 2018 and the decision P/0503/2020 issued on 22 December 2020,

Having regard to the application submitted by Vifor Fresenius Medical Care Renal Pharma France on 17 January 2024 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 26 April 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 and to the waiver.

(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 and to the waiver.

[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 calcifediol (Rayaldee), prolonged-release capsule, oral use, including changes to the deferral and to the waiver, 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/47042/2024
Amsterdam, 26 April 2024

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

EMEA-002093-PIP02-17-M02

Scope of the application

Active substance(s):

Calcifediol

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of secondary hyperparathyroidism

Pharmaceutical form(s):

Prolonged-release capsule

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Vifor Fresenius Medical Care Renal Pharma France

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 17 January 2024 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 and the decision P/0503/2020 issued on 22 December 2020.

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

The procedure started on 26 February 2024.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified. A waiver for a new paediatric subset has been added. The pharmaceutical form has been amended.

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 and to the waiver 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 secondary hyperparathyroidism (SHPT)

The waiver applies to:

- the paediatric population from birth to less than 8 years of age;
- 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 (SHPT)

2.1.1. Indication(s) targeted by the PIP

Treatment of secondary parathyroidism 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 8 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Prolonged-release capsule

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Study deleted during procedure EMEA-002093-PIP02-17-M02
Non-clinical studies	Not applicable
Clinical studies	**Study 2 (CTAP101-CL-2015)** Open-label, randomised, multiple dose study to evaluate pharmacokinetics, pharmacodynamics, safety and tolerability of calcifediol in children and adolescents from 8 years to less than 18 years of age with secondary hyperparathyroidism with stage 3 or 4 chronic kidney disease and low vitamin D levels.

	Study 3 Study deleted during procedure EMEA-002093-PIP02-17-M02
Extrapolation, modelling and simulation studies	**Study 4** Population PK study to evaluate the use of the product in children from 8 years to less than 18 years of age with secondary hyperparathyroidism and to support extrapolation in children and adolescents from 8 years to less than 18 years of age
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 February 2029
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 secondary hyperparathyroidism (SHPT)

Authorised indication(s):

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

  - Invented name(s): Rayaldee
  - Authorised pharmaceutical form(s): Prolonged-release capsule, soft
  - Authorised route(s) of administration: Oral use
  - Authorised via decentralised procedure