

EMA/576578/2016

European Medicines Agency decision

P/0265/2016

of 5 October 2016

on the acceptance of a modification of an agreed paediatric investigation plan for human recombinant IgG1 monoclonal antibody targeting fibroblast growth factor 23 (KRN23) (EMEA-001659-PIP01-15-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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0144/2016 issued on 23 May 2016,

Having regard to the application submitted by Ultragenyx Pharmaceutical Inc. on 27 May 2016 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 19 August 2016, 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.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

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 human recombinant IgG1 monoclonal antibody targeting fibroblast growth factor 23 (KRN23), solution for injection, subcutaneous 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

This decision is addressed to Ultragenyx Pharmaceutical Inc., 60 Leveroni Court, 94949 – Novato, United States.

EMA/PDCO/397224/2016
London, 19 August 2016

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001659-PIP01-15-M01

Scope of the application

Active substance(s):

Human recombinant IgG1 monoclonal antibody targeting fibroblast growth factor 23 (KRN23)

Condition(s):

Treatment of X-linked hypophosphataemia

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name / corporate name of the PIP applicant:

Ultragenyx Pharmaceutical Inc.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Ultragenyx Pharmaceutical Inc. submitted to the European Medicines Agency on 27 May 2016 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0144/2016 issued on 23 May 2016.

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

The procedure started on 21 June 2016.

Scope of the modification

Some measures 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.

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 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 annex 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 X-linked hypophosphataemia

2.1.1. Indication(s) targeted by the PIP

Treatment of X-linked hypophosphataemia

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)

Solution for injection

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 (UX023-CL201) Open-label, randomised, multicentre, multiple dose, uncontrolled study to evaluate pharmacokinetics, safety, activity and quality of life of human recombinant IgG1 monoclonal antibody targeting fibroblast growth factor 23 (KRN23) in children from 5 years to less than 13 years of age with X-linked hypophosphataemia. Study 2 (UX023-CL301) Open-label, randomised, multicentre, active controlled study to evaluate pharmacokinetics, safety, efficacy and quality of life of KRN23 compared to oral phosphate/active vitamin D therapy in children from 1 year to less than 13 years of age at the start of the study with X-linked hypophosphataemia.

Area	Number of measures	Description
		Study 3 (UX023-CL207) Open-label, multicentre, uncontrolled study to evaluate safety, pharmacodynamics and activity of KRN23 in children from birth to less than 1 year of age with X-linked hypophosphataemia. Study 4 (UX023-CL205) Open-label, multicentre, uncontrolled study to evaluate pharmacodynamics, safety and activity of KRN23 in children from 1 year to less than 5 years of age with X-linked hypophosphataemia.
Extrapolation, modelling and simulation studies	0	Not applicable.
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:	Yes
Date of completion of the paediatric investigation plan:	By September 2020
Deferral for one or more measures contained in the paediatric investigation plan:	Yes