EMA/775829/2022

European Medicines Agency decision

P/0411/2022

of 30 September 2022

on the acceptance of a modification of an agreed paediatric investigation plan for burosumab (Crysvita), (EMEA-001659-PIP01-15-M06) 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/0144/2016 issued on 23 May 2016, the decision P/0265/2016 issued on 5 October 2016, the decision P/0149/2017 issued on 7 June 2017, the decision P/0007/2018 issued on 30 January 2018, the decision P/0093/2020 issued on 18 March 2020 and the decision P/0491/2020 issued on 21 December 2020,

Having regard to the application submitted by Kyowa Kirin Holdings B.V. on 26 May 2022 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 9 September 2022, 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, 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 burosumab (Crysvita), 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 Kyowa Kirin Holdings B.V., 2 Bloemlaan, 2132NP – Hoofddorp, The Netherlands.

EMA/PDCO/566010/2022
Amsterdam, 9 September 2022

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

EMEA-001659-PIP01-15-M06

Scope of the application

Active substance(s):

Burosumab

Invented name:

Crysvita

Condition(s):

Treatment of X-linked hypophosphataemia

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Kyowa Kirin Holdings B.V.

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Kyowa Kirin Holdings B.V. submitted to the European Medicines Agency on 26 May 2022 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 decision P/0265/2016 issued on 5 October 2016, the decision P/0149/2017 issued on 7 June 2017, the decision P/0007/2018 issued on 30 January 2018, the decision P/0093/2020 issued on 18 March 2020 and the decision P/0491/2020 issued on 21 December 2020.

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

The procedure started on 11 July 2022.

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 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 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

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	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1 (UX023-CL201)** Open-label, randomised, multicentre, multiple dose, uncontrolled study to evaluate pharmacokinetics, safety, activity and quality of life of burosumab 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 burosumab 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 **Study 3 (UX023-CL207 (BUR-CL207))** Open-label, multicentre, uncontrolled study to evaluate safety, pharmacodynamics and activity of burosumab 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 burosumab in children from 1 year to less than 5 years of age with X-linked hypophosphataemia
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 September 2023
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):

- Treatment of X-linked hypophosphataemia

Authorised indication(s):

- Treatment of X-linked hypophosphataemia, in children and adolescents aged 1 to 17 years with radiographic evidence of bone disease, and in adults.

Authorised pharmaceutical form(s):

Solution for injection

Authorised route(s) of administration:

Subcutaneous use