MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept of change(s) to the agreed paediatric investigation plan

MHRA-100532-PIP01-22-M02

Scope of the Application

Active Substance(s)

BUROSUMAB

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

Kyowa Kirin Holdings BV

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Kyowa Kirin Holdings BV submitted to the licensing authority on 16/06/2022 15:22 BST an application for a Modification

The procedure started on 29/12/2022 22:15 GMT

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100532-PIP01-22-M02

Of 11/01/2023 20:34 GMT

On the adopted decision for BUROSUMAB (MHRA-100532-PIP01-22-M02) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan

This decision applies to a Modification for BUROSUMAB, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Kyowa Kirin Holdings BV, Bloemlaan 2, Hoofddorp, NETHERLANDS, 2132NP

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of X-linked hypophosphataemia

2.2 Indication(s) targeted by the PIP:

Treatment of X-linked hypophosphataemia

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

All subsets of the paediatric population from birth to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	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 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, Modeling & Simulation Studies	0	Not applicable

Other Studies	0	Not applicable
Other Measures	0	Not applicable

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	30/09/2023
Deferral of one or more studies contained in the paediatric investigation plan:	Yes