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 change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-100993-PIP01-23-M01

Scope of the Application

Active Substance(s)

QUIZARTINIB DIHYDROCHLORIDE

Condition(s)

Treatment of acute myeloid leukaemia (AML)

Pharmaceutical Form(s)

Powder for oral solution, Film-coated tablet

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Daiichi Sankyo UK Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Daiichi Sankyo UK Ltd submitted to the licensing authority on 25/07/2023 19:12 BST an application for a Modification

The procedure started on 09/08/2023 13:38 BST

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 and to the deferral

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-100993-PIP01-23-M01

Of 21/08/2023 13:11 BST

On the adopted decision for QUIZARTINIB DIHYDROCHLORIDE (MHRA-100993-PIP01-23-M01) 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 (including modification of a waiver or deferral included in that paediatric investigation plan).

This decision applies to a Modification for QUIZARTINIB DIHYDROCHLORIDE, Film-coated tablet; Powder for oral solution , ORAL USE .

This decision is addressed to Daiichi Sankyo UK Ltd, Building 4, Uxbridge Business Park, Sanderson Road, Uxbridge, UNITED KINGDOM, UB8 1DH

ANNEX I

1. Waiver

1.1 Condition:

Treatment of acute myeloid leukaemia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 month of age. Pharmaceutical form(s): Film-coated tablet, Powder for oral solution Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of acute myeloid leukaemia.

2.2 Indication(s) targeted by the PIP:

• For the treatment of paediatric patients aged from 1 month to less than 18 years of age with refractory or relapsed AML with FLT3-ITD mutations after failure of front line intensive chemotherapy regimen, in combination with standard chemotherapy. • For the treatment of paediatric patients aged from 1 month to less than 18 years of age with newly diagnosed AML with FLT3-ITD mutations and NPM1 wild-type.

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

From 1 month to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film-coated tablet Powder for oral solution

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	2	Study 1 Dose range-finding juvenile toxicity study. Study 2 Juvenile toxicity study.
Clinical Studies	2	Study 3 Open-label, single-arm trial to evaluate safety, pharmacokinetics, pharmacodynamics and efficacy of quizartinib in combination with FLA chemotherapy, with optional consolidation therapy, and as single-agent after high-dose therapy in paediatric patients with FLT3-ITD AML from 1 month to less than 18 years of age (and young adults). Study 4 Open-label, single-arm trial to evaluate safety, pharmacokinetics, pharmacodynamics and activity of quizartinib in combination with chemotherapy and as single-agent after high-dose therapy in paediatric patients newly-diagnosed with FLT3-ITD AML and NPM1 wild-type from 1 month to less than 18 years of age (and young adults).
Extrapolation, Modeling & Simulation Studies	1	Study 5 Exposure-response model.
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:	31/12/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes