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-100505-PIP01-22-M01

Scope of the Application

Active Substance(s)

copanlisib dihydrochloride

Condition(s)

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue), Treatment of mature B-cell neoplasms

Pharmaceutical Form(s)

Powder for concentrate for solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Bayer plc

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Bayer plc submitted to the licensing authority on 26/05/2022 13:20 BST an application for a Modification

The procedure started on 12/12/2022 11:27 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 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-100505-PIP01-22-M01

Of 15/12/2022 16:44 GMT

On the adopted decision for copanlisib dihydrochloride (MHRA-100505-PIP01-22-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 copanlisib dihydrochloride, Powder for concentrate for solution for infusion , INTRAVENOUS USE .

This decision is addressed to Bayer plc, 400 South Oak Way, Reading, UNITED KINGDOM, RG2 6AD

ANNEX I

1. Waiver

1.1 Condition:

Condition 1: Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 months of age Pharmaceutical form(s): Powder for concentrate for solution for infusion Route(s) of administration: INTRAVENOUS 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). Reason for Refusing Waiver: Not Applicable Condition 2: Treatment of mature B-cell neoplasms The waiver applies / applied to: Paediatric Subset(s): All subsets of the paediatric population from birth to less than 18 years of age Pharmaceutical form(s): Powder for concentrate for solution for infusion Route(s) of administration: INTRAVENOUS 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 all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

2.2 Indication(s) targeted by the PIP:

Treatment of children with a relapsed or refractory neuroblastoma, Ewing sarcoma, osteosarcoma or rhabdomyosarcoma including at first relapse, in combination with chemotherapy

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

The paediatric population from 6 months to less than 18 years of age

2.4 Pharmaceutical Form(s):

Powder for concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	4	Study 1 Non-clinical in vitro pharmacology testing (single agent). Study 2 Non-clinical in vivo pharmacology testing (single agent). Study 3 Non-clinical in vitro pharmacology testing (combination). Study 4 Non-clinical tolerability study for combination therapy.
Clinical Studies	1	Study 5 Open-label, non-controlled, dose escalating trial to evaluate the pharmacokinetics, pharmacodynamics, safety and activity of copanlisib in children from 6 months to less than 18 years of age (and young adults less than 22 years of age) with a relapsed or refractory solid malignant tumour or a lymphoma. Study 6 Deleted during procedure MHRA-100505-PIP01-22-M01.
Extrapolation, Modeling & Simulation Studies	0	Study 7 Deleted during procedure MHRA-100505-PIP01-22-M01.

		Study 8 Deleted during procedure MHRA-100505-PIP01-22-M01.
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:	31/12/2023
Deferral of one or more studies contained in the paediatric investigation plan:	No