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 (MHRA-100959-PIP01-23-M01) and to the deferral

MHRA-100959-PIP01-23-M02

Scope of the Application

Active Substance(s)

DOSTARLIMAB

Condition(s)

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies)

Pharmaceutical Form(s)

Concentrate for solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

GlaxoSmithKline UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, GlaxoSmithKline UK Limited submitted to the licensing authority on 27/03/2024 12:48 GMT an application for a Modification

The procedure started on 04/06/2024 10:50 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-100959-PIP01-23-M02

Of 03/09/2024 06:50 BST

On the adopted decision for DOSTARLIMAB (MHRA-100959-PIP01-23-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 (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for DOSTARLIMAB, Concentrate for solution for infusion , INTRAVENOUS USE .

This decision is addressed to GlaxoSmithKline UK Limited, 980 Great West Road Brentford, Middlesex, UNITED KINGDOM, TW8 9GS

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid malignancies).

2.2 Indication(s) targeted by the PIP:

Treatment of paediatric patients from birth to less than 18 years old with neuroblastoma and/or osteosarcoma

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

The paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	3	Study 1 Open-label, multiple dose, two part trial to evaluate pharmacokinetics, safety, activity and acceptability of niraparib when given in combination with dostarlimab in children from 6 months to less than 18 years of age with recurrent/ refractory solid tumours, excluding central nervous system (CNS) tumours in part 1a and 1b and with recurrent/ refractory osteosarcoma and recurrent/ refractory neuroblastoma in Part 2. Study 2 Open label, randomised controlled, active comparator trial to evaluate efficacy and safety of niraparib in combination with dostarlimab against current standard of care in children from 6 months of age to less than 18 years of age with relapsed/ refractory osteosarcoma and/or neuroblastoma. Study 3 Open label, randomised controlled, active comparator trial to evaluate efficacy and safety of niraparib in combination with dostarlimab against current standard of care in children from birth to less than 18 years of age with newly diagnosed high risk osteosarcoma and/or Stage 4 neuroblastoma.

Extrapolation, Modeling & Simulation Studies	1	Study 4 Modelling and simulation study, to evaluate the use of niraparib and dostarlimab in the proposed paediatric indications in children from birth to less than 18 years of age.
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:	30/06/2040
Deferral of one or more studies contained in the paediatric investigation plan:	Yes