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-100252-PIP01-21-M01) and to the deferral

MHRA-100252-PIP01-21-M02

Scope of the Application

Active Substance(s)

GEMTUZUMAB OZOGAMICIN

Condition(s)

Treatment of acute myeloid leukaemia

Pharmaceutical Form(s)

Powder for concentrate for solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Pfizer Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Pfizer Limited submitted to the licensing authority on 19/08/2024 12:39 BST an application for a Modification

The procedure started on 25/10/2024 09:01 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-100252-PIP01-21-M02

Of 15/11/2024 15:27 GMT

On the adopted decision for GEMTUZUMAB OZOGAMICIN (MHRA-100252-PIP01-21-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 GEMTUZUMAB OZOGAMICIN, Powder for concentrate for solution for infusion , INTRAVENOUS USE .

This decision is addressed to Pfizer Limited, Ramsgate Road, Sandwich,, Kent, UNITED KINGDOM, CT13 9NJ

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): 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 acute myeloid leukaemia

2.2 Indication(s) targeted by the PIP:

Gemtuzumab ozogamicin is used in combination with induction regimens for the treatment of de novo and secondary newly diagnosed acute myeloid leukaemia in paediatric patients aged 28 days up to less than 18 years.

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

The paediatric population from 1 month 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	0	Not applicable.
Clinical Studies	2	Study 1 Randomised open-label multi-centre study to evaluate the safety and efficacy of gemtuzumab ozogamicin added to standard chemotherapy in children with newly diagnosed acute myeloid leukaemia from 1 month to less than 18 years of age (and young adults less than 30 years of age). Study 2 Randomised open-label multi-centre dose-escalating trial to evaluate pharmacokinetics, toxicity, safety and activity of gemtuzumab ozogamicin in combination with intensified induction regimens in paediatric patients from 1 month to less 18 years of age with newly diagnosed, de novo or secondary acute myeloid leukaemia.
Extrapolation, Modeling & Simulation Studies	1	Study 3 Population PK and PK/ PD Modelling of gemtuzumab ozogamicin in paediatric patients with acute myeloid leukaemia.
Other Studies	0	Not applicable.
Other Measures	1	Study 4 Systematic review of studies evaluating safety, activity and / or efficacy of gemtuzumab ozogamicin

		in paediatric patients with relapse of, or progressive acute myeloid leukaemia.

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

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	30/06/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes