

EMA/18515/2022

European Medicines Agency decision

P/0014/2022

of 31 January 2022

on the acceptance of a modification of an agreed paediatric investigation plan for gemtuzumab ozogamicin (Mylotarg), (EMA-001733-PIP02-15-M02) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004¹,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency²,

Having regard to the European Medicines Agency's decision P/0078/2016 issued on 18 March 2016, and decision P/0326/2017 issued on 31 October 2017,

Having regard to the application submitted by Pfizer Europe MA EEIG on 9 September 2021 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 17 December 2021, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

- (1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

¹ OJ L 378, 27.12.2006, p.1, as amended.

² OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for gemtuzumab ozogamicin (Mylotarg), powder for concentrate for solution for infusion, intravenous use, including changes to the deferral, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Pfizer Europe MA EEIG, Boulevard de la Plaine 17, 1050 – Brussels, Belgium.

EMA/PDCO/530244/2021
Amsterdam, 17 December 2021

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001733-PIP02-15-M02

Scope of the application

Active substance(s):

Gemtuzumab ozogamicin

Invented name:

Mylotarg

Condition(s):

Treatment of acute myeloid leukaemia

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Powder for concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Pfizer Europe MA EEIG

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Pfizer Europe MA EEIG submitted to the European Medicines Agency on 9 September 2021 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0078/2016 issued on 18 March 2016, and decision P/0326/2017 issued on 31 October 2017.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 19 October 2021.

Scope of the modification

Some timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
 - to agree to changes to the paediatric investigation plan and to the deferral in the scope set out in the Annex I of this opinion.

The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of acute myeloid leukaemia

The waiver applies to:

- the paediatric population from birth to less than 1 month of age;
- powder for concentrate for solution for infusion, intravenous use;
- 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:

Treatment of acute myeloid leukaemia

2.1.1. 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

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 1 month to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder for concentrate for solution for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	2	Study 1 Randomized 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, <i>de novo</i> or secondary acute myeloid leukaemia
Extrapolation, modelling and 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 PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By June 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of acute myeloid leukaemia

Authorised indication(s):

- Mylotarg is indicated for combination therapy with daunorubicin (DNR) and cytarabine (AraC) for the treatment of patients age 15 years and above with previously untreated, de novo CD33-positive acute myeloid leukaemia (AML), except acute promyelocytic leukaemia (APL)

Authorised pharmaceutical form(s):

Powder for concentrate for solution for infusion (powder for concentrate)

Authorised route(s) of administration:

Intravenous use