EMA/666027/2017

# European Medicines Agency decision

P/0326/2017

of 31 October 2017

on the acceptance of a modification of an agreed paediatric investigation plan for gemtuzumab ozogamicin (EMEA-001733-PIP02-15-M01) 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.**

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
**Telephone** +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
**Contact EMA** www.ema.europa.eu/contact

# European Medicines Agency decision

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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 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0078/2016 issued on 18 March 2016,

Having regard to the application submitted by Pfizer Limited on 8 June 2017 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 15 September 2017, 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.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

***Article 1***

Changes to the agreed paediatric investigation plan for gemtuzumab ozogamicin, powder for concentrate for solution for infusion, intravenous use, 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 Limited, Ramsgate Road, CT13 9NJ - Sandwich, United Kingdom.

EMA/PDCO/392061/2017
London, 15 September 2017

# Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-001733-PIP02-15-M01

## 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 opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Pfizer Limited submitted to the European Medicines Agency on 8 June 2017 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.

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

The procedure started on 18 July 2017.

## Scope of the modification

A timeline of the paediatric investigation plan has been modified.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
**Telephone** +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5510
**Send a question via our website** www.ema.europa.eu/contact

## 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 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 annex 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**<br>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**<br><br>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, modelling and simulation studies | 1 | **Study 3**<br><br>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**<br><br>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 2022 |
| Deferral for one or more measures contained in the paediatric investigation plan: | Yes |