EMA/74598/2023

European Medicines Agency decision

P/0079/2023

of 10 March 2023

on the acceptance of a modification of an agreed paediatric investigation plan for inotuzumab ozogamicin (Besponsa), (EMEA-001429-PIP01-13-M07) 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 726/2004[1],

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 Agency[2],

Having regard to the European Medicines Agency's decision P/0333/2016 issued on 2 December 2016, the decision P/0402/2017 issued on 19 December 2017, the decision P/0062/2020 issued on 10 February 2020, the decision P/0478/2021 issued on 3 December 2021, the decision P/0134/2022 issued on 13 April 2022 and the decision P/0398/2022 issued on 9 September 2022,

Having regard to the application submitted by Pfizer Europe MA EEIG on 17 October 2022 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 20 January 2023, 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 inotuzumab ozogamicin (Besponsa), 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 - Bruxelles, Belgium.

EMA/PDCO/853666/2022
Amsterdam, 20 January 2023

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

EMEA-001429-PIP01-13-M07

Scope of the application

Active substance(s):

Inotuzumab ozogamicin

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of B cell acute lymphoblastic 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 Europe MA EEIG

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 17 October 2022 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/0333/2016 issued on 2 December 2016, the decision P/0402/2017 issued on 19 December 2017, the decision P/0062/2020 issued on 10 February 2020, the decision P/0478/2021 issued on 3 December 2021, the decision P/0134/2022 issued on 13 April 2022 and the decision P/0398/2022 issued on 9 September 2022.

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

The procedure started on 21 November 2022.

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 Paediatric Committee member of Norway 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 B cell acute lymphoblastic leukaemia

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- powder for concentrate for solution for infusion, intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Treatment of B cell acute lymphoblastic leukaemia

2.1.1. Indication(s) targeted by the PIP

Treatment of first relapse of precursor B cell acute lymphoblastic leukaemia

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

From 1 year 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	**Description**
Quality-related studies	Not applicable.
Non-clinical studies	Not applicable.
Clinical studies	**Study 1:** Open-label, multiple dose, two-strata trial to establish the maximum tolerated dose of inotuzumab ozogamicin used as single agent and to determine the recommended dose of inotuzumab ozogamicin as add-on to modified regimen from trial UKALL-R3 in children from 1 year to less than 18 years of age with CD22-positive relapsed/refractory acute lymphoblastic leukaemia. **Study 2:** Open-label, randomised superiority trial to evaluate safety and efficacy of inotuzumab ozogamicin monotherapy over standard

	UKALL-R3 regimen in patients from 1 year to less than 18 years of age (and adults) with high-risk first relapse of CD22 positive B cell precursor acute lymphoblastic leukaemia.
Extrapolation, modelling and simulation studies	Not applicable.
Other measures	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long-term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By October 2026
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

Condition(s) and authorised indication(s)

1. Treatment of B cell acute lymphoblastic leukaemia

Authorised indication(s):

- Besponsa is indicated as monotherapy for the treatment of adults with relapsed or refractory CD22-positive B cell precursor acute lymphoblastic leukaemia (ALL). Adult patients with Philadelphia chromosome positive (Ph+) relapsed or refractory B cell precursor ALL should have failed treatment with at least 1 tyrosine kinase inhibitor (TKI).
  - Invented name(s): Besponsa
  - Authorised pharmaceutical form(s): Powder for concentrate for solution for infusion
  - Authorised route(s) of administration: Intravenous use
  - Authorised via centralised procedure