

EMA/193744/2022

European Medicines Agency decision

P/0134/2022

of 13 April 2022

on the acceptance of a modification of an agreed paediatric investigation plan for inotuzumab ozogamicin (Besponsa), (EMA-001429-PIP01-13-M05) 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/0304/2014 issued on 24 November 2014, decision P/0333/2016 issued on 2 December 2016, decision P/0402/2017 issued on 19 December 2017, decision P/0062/2020 issued on 10 February 2020 and the decision P/0478/2021 issued on 3 December 2021,

Having regard to the application submitted by Pfizer Europe MA EEIG on 19 November 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 25 February 2022, 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, 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, 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/715055/2021
Amsterdam, 25 February 2022

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001429-PIP01-13-M05

Scope of the application

Active substance(s):

Inotuzumab ozogamicin

Invented name:

Besponsa

Condition(s):

Treatment of B cell acute lymphoblastic 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 19 November 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/0304/2014 issued on 24 November 2014, decision P/0333/2016 issued on 2 December 2016, decision P/0402/2017 issued on 19 December 2017, decision P/0062/2020 issued on 10 February 2020 and the decision P/0478/2021 issued on 3 December 2021.

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

The procedure started on 4 January 2022.

Scope of the modification

Some measures 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 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 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 January 2026
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 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).

Authorised pharmaceutical form(s):

Powder for concentrate for solution for infusion

Authorised route(s) of administration:

Intravenous use