EMA/19077/2021

European Medicines Agency decision

P/0016/2021

of 27 January 2021

on the agreement of a paediatric investigation plan and on the granting of a waiver for carfilzomib (Kyprolis), (EMEA-001806-PIP04-19) 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 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 application submitted by Amgen Europe BV on 21 February 2019 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 27 January 2021, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a waiver.

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

Has adopted this decision:

Article 1

A paediatric investigation plan for carfilzomib (Kyprolis), powder for solution for infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A waiver for carfilzomib (Kyprolis), powder for solution for infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Amgen Europe BV, Minervum 7061, 4817 ZK - Breda, The Netherlands.

EMA/PDCO/526004/2020 Corr
Amsterdam, 11 December 2020

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a waiver

EMEA-001806-PIP04-19

Scope of the application

Active substance(s):

Carfilzomib

Invented name:

Kyprolis

Condition(s):

Treatment of acute lymphoblastic leukaemia

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Powder for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Amgen Europe BV

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Amgen Europe BV submitted for agreement to the European Medicines Agency on 21 February 2019 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 1 April 2019.

Supplementary information was provided by the applicant on 11 September 2020. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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

2. The measures and timelines of the agreed 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.

1. Waiver

1.1. Condition:

Treatment of acute lymphoblastic leukaemia

The waiver applies to:

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

2. Paediatric investigation plan

2.1. Condition:

Treatment of acute lymphoblastic leukaemia

2.1.1. Indication(s) targeted by the PIP

Treatment for paediatric patients with relapsed or refractory T-cell acute lymphoblastic leukaemia or paediatric patients with relapsed or refractory B-cell acute lymphoblastic leukaemia who received prior targeted immune therapy

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 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	1	**Study 1 (20140106):** Part 1 (phase 1b): uncontrolled, dose-escalation study in patients from 1 year to less than 18 years of age (and adults), with relapsed or refractory T-cell or B-cell acute lymphoblastic leukaemia with or without extramedullary disease, to assess the safety and tolerability of carfilzomib, alone and in combination with induction chemotherapy, and to determine the optimal dose for the subsequent part 2 study of carfilzomib (CFZ) in combination with induction chemotherapy.

		Part 2 (phase 2): externally-controlled, single arm study of carfilzomib (CFZ) in combination with VXLD induction chemotherapy (vincristine, dexamethasone, PEG-asparaginase, and daunorubicin) from 1 month to less than 18 years (diagnosis must be prior to 18 years) of age (and adults) with relapsed or refractory T-cell ALL or B-cell acute lymphoblastic leukaemia, who must have a bone marrow relapse with or without extramedullary disease after receiving a targeted B-cell immune therapy as treatment for a prior relapse, to compare the rate of complete remission (CR) at the end of induction therapy to an external control.
Extrapolation, modelling and simulation studies	0	Not applicable
Other studies	0	Not applicable
Other measures	0	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 2024
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

Treatment of multiple myeloma in adults

Authorised indication(s):

Kyprolis in combination with either lenalidomide and dexamethasone or dexamethasone alone is indicated for the treatment of adult patients with multiple myeloma who have received at least one prior therapy.

Authorised pharmaceutical form(s):

Powder for solution for infusion

Authorised route(s) of administration:

Intravenous use