



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/592849/2016

European Medicines Agency decision

P/0262/2016

of 5 October 2016

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for copanlisib (EMEA-001757-PIP02-15) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency²,

Having regard to the application submitted by Bayer Pharma AG on 6 October 2015 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 19 August 2016, in accordance with Article 18 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation 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 deferral 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 deferral.
- (4) 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 copanlisib, 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 deferral for copanlisib, 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

A waiver for copanlisib, 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 4

This decision is addressed to Bayer Pharma AG, Müllerstrasse 178, 13353 - Berlin, German.

EMA/PDCO/400966/2016
London, 19 August 2016

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

EMA-001757-PIP02-15

Scope of the application

Active substance(s):

Copanlisib

Condition(s):

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

Treatment of mature B-cell neoplasm

Pharmaceutical form(s):

Powder for solution for infusion

Route(s) of administration:

Intravenous use

Name / corporate name of the PIP applicant:

Bayer Pharma AG

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Bayer Pharma AG submitted for agreement to the European Medicines Agency on 6 October 2015 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 30 November 2015.

Supplementary information was provided by the applicant on 17 May 2016. 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 18 of said Regulation;
- to grant a deferral in accordance with Article 21 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 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 all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

The waiver applies to:

- the paediatric population from birth to less than 6 months;
- powder 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).

1.2. Condition:

Treatment of mature B-cell neoplasm

The waiver applies to:

- All subsets of the paediatric population from birth to less than 18 years;
- Powder 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 all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

2.1.1. Indication(s) targeted by the PIP

Treatment of children with a relapsed or refractory neuroblastoma, Ewing sarcoma, osteosarcoma or rhabdomyosarcoma including at first relapse, in combination with chemotherapy.

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

From 6 months to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Powder for solution for infusion, intravenous use

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	4	Study 1 Non-clinical <i>in vitro</i> pharmacology testing (single-agent) Study 2 Non-clinical <i>in vivo</i> pharmacology testing (single-agent) Study 3 Non-clinical <i>in vitro</i> pharmacology testing (combination) Study 4 Non-clinical tolerability study for combination therapy
Clinical studies	2	Study 5 Open-label, non-controlled, dose escalating trial to evaluate the pharmacokinetics, pharmacodynamics, safety and activity of copanlisib in children from 6 months to less than 18 years of age (and young adults) with a relapsed or refractory solid malignant tumour or a lymphoma and an extension phase in children from 6 months to less than 18 years of age (and young adults) with a relapsed or refractory neuroblastoma, osteosarcoma, rhabdomyosarcoma or Ewing sarcoma Study 6 Randomized, controlled trial to evaluate the safety and efficacy of copanlisib in combination with anti-cancer therapy in paediatric patients from 6 months to less than 18 years of age (and young adults) with a relapsed or refractory neuroblastoma, osteosarcoma, rhabdomyosarcoma or Ewing sarcoma
Extrapolation, modelling and simulation studies	2	Study 7 Physiology-based pharmacokinetic (PBPK) study to predict PK properties in paediatric patients Study 8 Population PK model for estimating the PK parameters of Copanlisib in paediatric patients
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 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes