

EMA/431300/2017

European Medicines Agency decision

P/0200/2017

of 21 July 2017

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for liposomal combination of cytarabine and daunorubicin (EMA-001858-PIP02-16) 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 Jazz Pharmaceuticals Ireland Limited on 11 July 2016 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 23 June 2017, 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 liposomal combination of cytarabine and daunorubicin, 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 liposomal combination of cytarabine and daunorubicin, 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 liposomal combination of cytarabine and daunorubicin, 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 Jazz Pharmaceuticals Ireland Limited, Fifth Floor, Waterloo Exchange, Waterloo Road, 4 Dublin, Ireland.

EMA/PDCO/589985/2016

London, 23 June 2017

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

EMA-001858-PIP02-16

Scope of the application

Active substance(s):

Liposomal combination of cytarabine and daunorubicin

Condition(s):

Treatment of acute myeloid leukaemia

Pharmaceutical form(s):

Powder for solution for infusion

Route(s) of administration:

Intravenous use

Name / corporate name of the PIP applicant:

Jazz Pharmaceuticals Ireland Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Jazz Pharmaceuticals Ireland Limited submitted for agreement to the European Medicines Agency on 11 July 2016 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 16 August 2016.

Supplementary information was provided by the applicant on 3 April 2017. 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 acute myeloid leukaemia

The waiver applies to:

- the paediatric population from birth to less than 28 days of age;
- 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 acute myeloid leukaemia

2.1.1. Indication(s) targeted by the PIP

Treatment of paediatric patients from 28 days to less than 18 years of age with relapsed/refractory acute myeloid leukaemia or in first relapse and with newly diagnosed acute myeloid leukaemia.

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

From 28 days 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	3	Study 1 Open-label, multiple dose trial to evaluate pharmacokinetics, safety and tolerability of liposomal combination of cytarabine and daunorubicin (CPX-351) in paediatric patients from 28 days to less than 18 years of age (and young adults) with a relapsed/refractory haematologic malignancy (CPX-MA-1201).

Area	Number of measures	Description
		Study 2 Open-label, multiple dose study to evaluate pharmacokinetics, safety, tolerability and anti-tumour activity of liposomal combination of cytarabine and daunorubicin (CPX-351) followed by fludarabine, cytarabine, and G-CSF (FLAG) in paediatric patients from 28 days to less than 18 years of age (and young adults) with relapsed/refractory acute myeloid leukaemia including a dose-finding phase and an expansion phase. (AAML1421). Study 3 Open-label, active-controlled study to evaluate efficacy, safety, tolerability and pharmacokinetics, of liposomal combination of cytarabine and daunorubicin (CPX-351) compared to standard of care in paediatric patients from 28 days to less than 18 years of age with newly-diagnosed acute myeloid leukaemia (CLTR0316-209).
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 September 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes