EMA/535417/2021

European Medicines Agency decision

P/0453/2021

of 29 October 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral for crisantaspase (EMEA-002934-PIP01-20) 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 Jazz Pharmaceuticals Ireland Ltd. on 27 November 2020 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 10 September 2021, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 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.

(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.

[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 crisantaspase, solution for injection, solution for infusion, intramuscular use, 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 crisantaspase, solution for injection, solution for infusion, intramuscular use, 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 Jazz Pharmaceuticals Ireland Ltd., Waterloo Exchange, Waterloo Road, D04 E5W7 – Dublin, Ireland.

EMA/PDCO/342742/2021
Amsterdam, 10 September 2021

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

EMEA-002934-PIP01-20

Scope of the application

Active substance(s):

Crisantaspase

Condition(s):

Treatment of acute lymphoblastic leukaemia

Treatment of lymphoblastic lymphoma

Pharmaceutical form(s):

Solution for injection

Solution for infusion

Route(s) of administration:

Intramuscular use

Intravenous use

Name/corporate name of the PIP applicant:

Jazz Pharmaceuticals Ireland Ltd.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Jazz Pharmaceuticals Ireland Ltd. submitted for agreement to the European Medicines Agency on 27 November 2020 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 4 January 2021.

Supplementary information was provided by the applicant on 3 June 2021. 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 deferral in accordance with Article 21 of said Regulation.

   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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of acute lymphoblastic leukaemia (ALL)

2.1.1. Indication(s) targeted by the PIP

Treatment of patients with ALL who have developed hypersensitivity or silent inactivation to E. coli-derived asparaginase.

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

From birth to less than 18 years of age.

2.1.3. Pharmaceutical form

Solution for injection

Solution for infusion

2.1.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	0	Not applicable
Non-clinical studies	1	***Study 1 (WIL-350510)*** A pharmacokinetic comparability study of recombinant crisantaspase and crisantaspase conjugate, administered as a single dose intravenous bolus in male CD-1 mice.
Clinical studies	2	***Study 2 (JZP458-201 Part A)*** Open-label, single arm, multiple dose trial to confirm dose and evaluate pharmacokinetics, safety, activity and immunogenicity of intramuscular (IM) crisantaspase in children from birth to less than 18 years of age (and adults) with ALL or lymphoblastic lymphoma (LBL) who are hypersensitive to E. coli-derived asparaginases (allergic reaction or silent inactivation). ***Study 3 (JZP458-201 Part B)*** Open-label, single arm, multiple dose trial to confirm dose and evaluate pharmacokinetics, safety, activity and immunogenicity of

		intravenous (IV) crisantaspase in children from birth to less than 18 years of age (and adults) with ALL or lymphoblastic lymphoma (LBL) who are hypersensitive to E. coli-derived asparaginases (allergic reaction or silent inactivation).
Extrapolation, modelling and simulation studies	1	***Study 4*** Modelling and simulation study to evaluate the use of the product with its two formulations (IM and IV) in the proposed paediatric indication in children from birth to less than 18 years of age with ALL or lymphoblastic lymphoma (LBL) who are hypersensitive to E. coli-derived asparaginases (allergic reaction or silent inactivation).
Other studies	0	Not applicable
Other measures	0	Not applicable

2.2. Condition:

Treatment of lymphoblastic lymphoma (LBL)

2.2.1. Indication(s) targeted by the PIP

Treatment of patients with LBL who have developed hypersensitivity or silent inactivation to E. coli-derived asparaginase.

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

From birth to less than 18 years of age.

2.2.3. Pharmaceutical form

Solution for injection

Solution for infusion

2.2.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	0	Not applicable
Non-clinical studies	1	***Study 1*** Same as for condition of treatment of acute lymphoblastic leukaemia
Clinical studies	2	***Study 2*** Same as for condition of treatment of acute lymphoblastic leukaemia

		Study 3 Same as for condition of treatment of acute lymphoblastic leukaemia
Extrapolation, modelling and simulation studies	1	***Study 4*** Same as for condition of treatment of acute lymphoblastic leukaemia
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 December 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes