EMA/73051/2021

European Medicines Agency decision

P/0099/2021

of 19 March 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for imetelstat (EMEA-001910-PIP03-20) 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 Geron Corporation on 14 May 2020 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 29 January 2021, in accordance with Article 17 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 imetelstat, 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 imetelstat, 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 imetelstat, 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 Geron Corporation, 919 E. Hillsdale Blvd, Suite 250, CA 94404 - Foster City, United States.

EMA/PDCO/579693/2020
Amsterdam, 29 January 2021

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

EMEA-001910-PIP03-20

Scope of the application

Active substance(s):

Imetelstat

Condition(s):

Treatment of acute myeloid leukaemia

Treatment of myelodysplastic syndromes, including juvenile myelomonocytic leukaemia

Pharmaceutical form(s):

Powder for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Geron Corporation

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Geron Corporation submitted for agreement to the European Medicines Agency on 14 May 2020 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 6 July 2020.

Supplementary information was provided by the applicant on 22 October 2020. The applicant proposed modifications to the paediatric investigation plan and to the waiver.

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;
   - 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 (AML)

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).

1.2. Condition:

Treatment of myelodysplastic syndromes (MDS), including juvenile myelomonocytic leukaemia (JMML)

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 (AML)

2.1.1. Indication(s) targeted by the PIP

Treatment of paediatric patients with relapsed or refractory AML or MDS, including JMML

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	1	**Study 1** Development of an age appropriate presentation of a powder for solution for infusion
Non-clinical studies	2	**Study 2** Ex-vivo pharmacology study to evaluate the anti-leukaemic activity of imetelstat **Study 3** In-vivo pharmacology study to evaluate the anti-leukaemic activity of imetelstat
Clinical studies	2	**Study 4** Open-label, single arm, two-stage trial to evaluate dose, safety, pharmacokinetics, pharmacodynamics and activity of imetelstat as monotherapy in children from 1 year to less than 18 years of age with relapsed or refractory AML or MDS, including JMML. **Study 5** Open-label, single arm, confirmatory trial to evaluate safety and activity of imetelstat as monotherapy in children from 28 days to less than 18 years of age with relapsed or refractory AML or MDS, including JMML.
Extrapolation, modelling and simulation studies	1	**Study 6** Modelling and simulation study to support dose finding of imetelstat as monotherapy in children from 28 days to less than 18 years of age with relapsed or refractory AML or MDS, including JMML.
Other studies	0	Not applicable
Other measures	0	Not applicable

2.2. Condition:

Treatment of myelodysplastic syndromes (MDS), including juvenile myelomonocytic leukaemia (JMML)

2.2.1. Indication(s) targeted by the PIP

Treatment of paediatric patients with relapsed or refractory AML or MDS, including JMML

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

From 28 days to less than 18 years of age

2.2.3. Pharmaceutical form(s)

Powder for solution for infusion

2.2.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Same as study 1 for condition of treatment of AML
Non-clinical studies	0	Not applicable
Clinical studies	2	**Study 2** Same as study 4 for condition of treatment of AML **Study 3** Same as study 5 for condition of treatment of AML
Extrapolation, modelling and simulation studies	1	**Study 4** Same as study 6 for condition of treatment of AML
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 July 2030
Deferral for one or more measures contained in the paediatric investigation plan:	Yes