EMA/911953/2022

European Medicines Agency decision

P/0517/2022

of 30 December 2022

on the acceptance of a modification of an agreed paediatric investigation plan for imetelstat (EMEA-001910-PIP03-20-M01) 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.

European Medicines Agency decision

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on the acceptance of a modification of an agreed paediatric investigation plan for imetelstat (EMEA-001910-PIP03-20-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0099/2021 issued on 19 March 2021,

Having regard to the application submitted by Geron Corporation on 8 August 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 11 November 2022, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan and to the deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

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

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for imetelstat, powder for solution for infusion, intravenous use, including changes to the deferral, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Geron Corporation, 919 E. Hillsdale Blvd, Suite 250, CA 94404 - Foster City, United States.

EMA/PDCO/687158/2022
Amsterdam, 11 November 2022

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-001910-PIP03-20-M01

Scope of the application

Active substance(s):

Imetelstat

Invented name and authorisation status:

See Annex II

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 22 of Regulation (EC) No 1901/2006 as amended, Geron Corporation submitted to the European Medicines Agency on 8 August 2022 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0099/2021 issued on 19 March 2021.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 12 September 2022.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan and to the deferral in the scope set out in the Annex I of this opinion.

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

2. The measures and timelines of the 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.

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	Description
Quality-related studies	**Study 1** Development of an age appropriate presentation of a powder for solution for infusion
Non-clinical studies	**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	**Study 4** Open-label, single armtrial to evaluate dose, safety, pharmacokinetics, pharmacodynamics of imetelstat in combination with fludarabine and cytarabine (FLA) chemotherapy in children from 1 year to less than 18 years of age with relapsed or refractory AML, including MDS or JMML **Study 5** Open-label, randomised controlled trial to evaluate safety, pharmacokinetics (PK) and activity of imetelstat in combination with fludarabine and cytarabine (FLA) chemotherapy against FLA chemotherapy in children from 28 days to less than 18 years of age with AML after first relapse or refractory to first line therapy, including MDS or JMML **Study *7 (added during EMEA-001910-PIP03-20-M01)*** Open-label, randomised controlled trial to evaluate safety, pharmacokinetics (PK) and efficacy of imetelstat in combination with fludarabine and cytarabine (FLA) chemotherapy against FLA chemotherapy in children from 28 days to less than 18 years of age with AML after first relapse or refractory to first line therapy, including MDS or JMML
Extrapolation, modelling and simulation studies	**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	Not applicable
Other measures	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	Description
Quality-related studies	**Study 1** Same as for condition of treatment of AML
Non-clinical studies	Not applicable
Clinical studies	**Study 4** Same as for condition of treatment of AML **Study 5** Same as for condition of treatment of AML **Study 7 *(added during EMEA-001910-PIP03-20-M01)*** Same as for condition of treatment of AML
Extrapolation, modelling and simulation studies	**Study 6** Same as for condition of treatment of AML
Other studies	Not applicable
Other measures	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 October 2035
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.