EMA/415145/2020

European Medicines Agency decision

P/0346/2020

of 9 September 2020

on the acceptance of a modification of an agreed paediatric investigation plan for treosulfan (Trecondi), (EMEA-000883-PIP01-10-M05) 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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on the acceptance of a modification of an agreed paediatric investigation plan for treosulfan (Trecondi), (EMEA-000883-PIP01-10-M05) 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 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 European Medicines Agency's decision P/122/2011 issued on 7 June 2011, the decision P/0104/2013 issued on 30 April 2013, the decision P/0033/2014 issued on 4 March 2014, the decision P/0059/2017 issued on 17 March 2017 and the decision P/0197/2017 issued on 14 July 2017,

Having regard to the application submitted by medac Gesellschaft für klinische Spezialpräparate mbH on 20 April 2020 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 24 July 2020, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for treosulfan (Trecondi), 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 medac Gesellschaft für klinische Spezialpräparate mbH, Theaterstr 6, 22880 – Wedel, Germany.

EMA/PDCO/232898/2020
Amsterdam, 24 July 2020

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

EMEA-000883-PIP01-10-M05

Scope of the application

Active substance(s):

Treosulfan

Invented name:

Trecondi

Condition(s):

Conditioning treatment prior to haematopoietic progenitor cell transplantation

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Powder for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

medac Gesellschaft für klinische Spezialpräparate mbH

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, medac Gesellschaft für klinische Spezialpräparate mbH submitted to the European Medicines Agency on 20 April 2020 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/122/2011 issued on 7 June 2011, the decision P/0104/2013 issued on 30 April 2013, the decision P/0033/2014 issued on 4 March 2014, the decision P/0059/2017 issued on 17 March 2017 and the decision P/0197/2017 issued on 14 July 2017.

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

The procedure started on 26 May 2020.

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 Norwegian Paediatric Committee member 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:

Conditioning treatment prior to haematopoietic progenitor cell transplantation

The waiver applies to:

- children from birth to less than 1 months of age;
- powder for solution for infusion, intravenous use;
- on the grounds that clinical studies cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset.

2. Paediatric Investigation Plan

2.1. Condition:

Conditioning treatment prior to haematopoietic progenitor cell transplantation

2.1.1. Indication(s) targeted by the PIP

Treosulfan in combination with a fludarabine-containing myeloablative conditioning regimen to treat a paediatric patient with a malignant disease, which requires an allogeneic haematopoietic stem cell transplant

Treosulfan in combination with a fludarabine-containing myeloablative conditioning regimen to treat a paediatric patient with a non-malignant disease, which requires an allogeneic haematopoietic stem cell transplant

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

From 1 months 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 studies	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	2	**Study 1** Juvenile toxicity study **Study 2** CNS pharmacokinetic study

Clinical studies	2	**Study 4** Randomised, active-controlled, open-label, multicentre trial to evaluate pharmacokinetics, activity and safety of treosulfan compared with busulfan in children with a non-malignant disease **Study 5** Multicentre, non-controlled, open-label trial to evaluate pharmacokinetics, activity and safety of treosulfan in children with a malignant disease
Other studies	1	**Study 3** Study to analyse of all data on paediatric uses of treosulfan

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By January 2021
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Conditioning treatment prior to haematopoietic progenitor cell transplantation

Authorised indication(s):

- Treosulfan in combination with fludarabine is indicated as part of conditioning treatment prior to allogeneic haematopoietic stem cell transplantation (alloHSCT) in adult patients with malignant andnon-malignant diseases, and in paediatric patients older than one month with malignant diseases.

Authorised pharmaceutical form(s):

Powder for solution for infusion

Authorised route(s) of administration:

Intravenous use