EMA/202483/2013

European Medicines Agency decision

P/0104/2013

of 30 April 2013

on the acceptance of a modification of an agreed paediatric investigation plan for treosulfan (EMEA-000883-PIP01-10-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.

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on the acceptance of a modification of an agreed paediatric investigation plan for treosulfan (EMEA-000883-PIP01-10-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 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,

Having regard to the application submitted by medac Gesellschaft für klinische Spezialpräparate mbH on 12 December 2012 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 15 March 2013, 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.

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

[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, powder for solution for injection / infusion, intravenous use, 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, Fehlandtstr. 3, 20354 – Hamburg, Germany.

Done at London, 30 April 2013

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/821084/2012

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

EMEA-000883-PIP01-10-M01

Scope of the application

Active substance(s):

Treosulfan

Condition(s):

Conditioning treatment prior to haematopoietic progenitor cell transplantation

Pharmaceutical form(s):

Powder for solution for injection / infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

medac Gesellschaft für klinische Spezialpräparate mbH

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 12 December 2012 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 application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 16 January 2013.

Scope of the modification

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

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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 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 annex and appendix.

London, 15 March 2013

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman
(Signature on file)

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

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;
- for powder for solution for injection / 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 injection / infusion

2.1.4. Measures

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	2	Measure 1: Juvenile toxicity study Measure 2: CNS pharmacokinetic study
Clinical	3	Measure 3: Analysis of all data on paediatric uses of treosulfan Measure 4: Randomised, active-controlled, open-label, multicenter trial to evaluate pharmacokinetics, activity and safety of treosulfan compared with busulfan in children with non-malignant diseases Measure 5: Multicentre, non-controlled, open-label

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 December 2016
Deferral for one or more measures contained in the paediatric investigation plan:	Yes