EMA/28037/2018

European Medicines Agency decision

P/0012/2018

of 30 January 2018

on the acceptance of a modification of an agreed paediatric investigation plan for matrix applied characterised autologous cultured chondrocytes (MACI), (EMEA-000979-PIP01-10-M02) 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 European Medicines Agency's decision P/111/2011 issued on 6 May 2011 and the decision P/0294/2013 issued on 29 November 2013,

Having regard to the application submitted by Vericel Denmark ApS on 25 September 2017 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 December 2017, 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 matrix applied characterised autologous cultured chondrocyte (MACI), implant, implant 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 Vericel Denmark ApS, Amaliegade 10, DK-1256 - Copenhagen K, Denmark.

EMA/PDCO/643109/2017
London, 15 December 2017

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

EMEA-000979-PIP01-10-M02

Scope of the application

Active substance(s):

Matrix applied characterised autologous cultured chondrocytes

Invented name:

MACI

Condition(s):

Treatment of cartilage disorders

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Implant

Route(s) of administration:

Implant use

Name/corporate name of the PIP applicant:

Vericel Denmark ApS

Information about the authorised medicinal product:

See Annex II

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Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Vericel Denmark ApS submitted to the European Medicines Agency on 25 September 2017 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/111/2011 issued on 6 May 2011 and the decision P/0294/2013 issued on 29 November 2013.

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

The procedure started on 17 October 2017.

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

Treatment of cartilage disorders

The waiver applies to:

- the paediatric population from birth to closure of the femoral epiphyseal growth plate;
- for implant use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric Investigation Plan

2.1. Condition:

Treatment of cartilage disorders

2.1.1. Indication(s) targeted by the PIP

Treatment of cartilage disorders

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

Paediatric population from closure of the femoral epiphyseal growth plate to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Implant

2.1.4. Measures

Area	Number of studies	Description
Quality		Not applicable.
Non-clinical		Not applicable.
Clinical	1	Study 1 (55-1702-1) Prospective, open-label, randomized concurrent active-controlled, longitudinal, multicentre, clinical study to assess the safety and efficacy of Matrix applied characterised autologous cultured chondrocytes (MACI) in patients aged 10 to less than 17 years of age with symptomatic chondral or osteochondral defects of the knee due to osteochondritis dissecans or acute trauma
Extrapolation, modelling and simulation studies	1	Study 2: Extrapolation of efficacy from adult patients of less than 35 years of age treated with MACI in the SUMMIT study to paediatric patients of less than 17 years of age.

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 2025
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. Treatment of cartilage disorders

Authorised indication:

MACI is indicated for the repair of symptomatic, full-thickness cartilage defects of the knee (grade III and IV of the Modified Outerbridge Scale) of 3-20 cm2 in skeletally mature adult patients.

Authorised pharmaceutical form(s):

Implantation matrix

Authorised route(s) of administration:

Implantation use

Note: the marketing authorisation of this product was suspended on 19 November 2014.