EMA/307425/2024

European Medicines Agency decision

P/0276/2024

of 14 August 2024

on the acceptance of a modification of an agreed paediatric investigation plan for allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded (MC0518), (EMEA-002706-PIP01-19-M03) 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

P/0276/2024

of 14 August 2024

on the acceptance of a modification of an agreed paediatric investigation plan for allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded (MC0518), (EMEA-002706-PIP01-19-M03) 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/0453/2020 issued on 1 December 2020, the decision P/0033/2022 issued on 1 February 2022, and the decision P/0008/2023 issued on 30 January 2023,

Having regard to the application submitted by medac Gesellschaft für klinische Spezialpräparate mbH on 20 March 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 28 June 2024, 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, 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 allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded (MC0518), dispersion for 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, Theaterstrasse 6, 22880 – Wedel, Germany.

EMA/PDCO/142425/2024
Amsterdam, 28 June 2024

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

EMEA-002706-PIP01-19-M03

Scope of the application

Active substance(s):

Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded (MC0518)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of acute graft-versus-host disease

Pharmaceutical form(s):

Dispersion for 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 20 March 2024 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0453/2020 issued on 1 December 2020, the decision P/0033/2022 issued on 1 February 2022, and the decision P/0008/2023 issued on 30 January 2023.

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

The procedure started on 29 April 2024.

Scope of the modification

Some measures 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 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 graft-versus-host disease

The waiver applies to:

- the paediatric population from birth to less than 28 days of age;
- dispersion for infusion, intravenous use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of acute graft-versus-host disease

2.1.1. Indication(s) targeted by the PIP

Treatment of steroid-refractory acute graft-versus-host disease (SR-aGvHD)

From 28 days to less than 18 years of age

2.1.2. Pharmaceutical form(s)

Dispersion for infusion

2.1.3. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1** [MC-MSC.1/aGvHD (IDUNN Trial)] Randomised, controlled, open-label trial to demonstrate the superiority of MC0518 to the first used best available therapy (BAT) with respect to overall response rate (ORR) and / or Overall Survival (OS) at Visit Day 28 in paediatric patients from 12 to less than 18 years of age (and adults) with steroid-refractory acute graft-versus-host disease **Study 2 [**(MC-MSC.2/aGvHD) (BALDER trial)] Multicentre, open-label, randomised (2:1), controlled Phase 2 of first line treatment with MC0518 versus best available therapy study to compare the overall response rate (ORR) in paediatric patients from 28 days to less than 18 years of age with steroid-refractory acute graft-versus-host disease at Visit Day 28 after treatment with MC0518 or best available therapy (BAT)

Extrapolation, modelling and simulation studies	Not applicable
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 September 2028
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.