



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-2038334

European Medicines Agency decision

EMA/PE/0000238064

of 16 April 2025

on the acceptance of a modification of an agreed paediatric investigation plan for allogeneic bone marrow-derived pooled mesenchymal stromal cells ex-vivo expanded 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 allogeneic bone marrow-derived pooled mesenchymal stromal cells ex-vivo expanded 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 726/2004¹,

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 Agency²,

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, the decision P/0008/2023 issued on 30 January 2023 and the decision P/0276/2024 issued on 14 August 2024,

Having regard to the application submitted by Medac Gesellschaft für klinische Spezialpräparate mbH on 25 November 2024 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 28 February 2025, 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 pooled mesenchymal stromal cells ex-vivo expanded, 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, 6 Theaterstrasse, 22880 – Wedel, Germany.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1811085 ¹Corr
Amsterdam, 28 February 2025

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA/PE/0000238064

Scope of the application

Active substance(s):

Allogeneic bone marrow-derived pooled mesenchymal stromal cells ex-vivo expanded

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 25 November 2024 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/0453/2020 issued on 1 December 2020, the decision P/0033/2022 issued on 1 February 2022, the decision P/0008/2023 issued on 30 January 2023 and the decision P/0276/2024 issued on 14 August 2024.

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

The procedure started on 2 January 2025.

¹ 7 April 2025



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)

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)

Dispersion for infusion

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	Study 1 [MC-MS.C.1/aGvHD (IDUNN Trial)] Study deleted in EMA/PE/0000238064 of February 2025 Study 2 [(MC-MS.C.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.