



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/9335/2025

European Medicines Agency decision P/0003/2025

of 28 January 2025

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for heterologous intestinal microbiota from healthy donors (MBK-01), (EMA-003635-PIP01-24) 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 726/2004¹,

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 application submitted by Mikrobiomik Healthcare Company, S.L. on 26 April 2024 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 December 2024, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a deferral.
- (4) It is therefore appropriate to adopt a decision granting a waiver.

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

A paediatric investigation plan for heterologous intestinal microbiota from healthy donors (MBK-01), gastro-resistant capsule, hard, oral use, gastric use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for heterologous intestinal microbiota from healthy donors (MBK-01), gastro-resistant capsule, hard, oral use, gastric use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for heterologous intestinal microbiota from healthy donors (MBK-01), gastro-resistant capsule, hard, oral use, gastric use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to Mikrobiomik Healthcare Company, S.L., Astondo Bidea, 612, 48160 – Derio, Spain.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/434779/2024
Amsterdam, 13 December 2024

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMA-003635-PIP01-24

Scope of the application

Active substance(s):

Heterologous intestinal microbiota from healthy donors (MBK-01)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of *Clostridioides difficile* infection

Pharmaceutical form(s):

Gastro-resistant capsule, hard

Route(s) of administration:

Oral use, gastric use

Name/corporate name of the PIP applicant:

Mikrobiomik Healthcare Company, S.L.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Mikrobiomik Healthcare Company, S.L. submitted for agreement to the European Medicines Agency on 26 April 2024 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 27 May 2024.

Supplementary information was provided by the applicant on 9 September 2024. The applicant proposed modifications to the paediatric investigation plan.



Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
- to grant a deferral in accordance with Article 21 of said Regulation;
- to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The Paediatric Committee members of Liechtenstein and Norway agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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 *Clostridioides difficile* infection

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- gastro-resistant capsule, hard, oral use and gastric use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of *Clostridioides difficile* infection

2.1.1. Indication(s) targeted by the PIP

Treatment of paediatric patients with primary refractory and recurrent *Clostridioides difficile* infection (CDI)

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

From 1 year to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Gastro-resistant capsule, hard

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable.
Non-clinical studies	Not applicable.
Clinical studies	Study 1 Open label, two stage, dose finding clinical trial in paediatric patients with recurrent <i>Clostridioides difficile</i> infection (CDI), to evaluate the efficacy and safety of different doses of lyophilised faecal microbiota (MBK-01) via oral capsules or via nasojejunal (NJ) tubes compared with standard of care antibiotic treatment Study 2 Confirmatory clinical study with MBK-01 to confirm the efficacy and safety results generated in Study 1 with the selected doses of MBK-01

	established from Stage 1 Study 1 in the recurrent CDI paediatric population
Modelling and simulation analyses	Not applicable.
Other studies	Not applicable.
Extrapolation plan	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By December 2041
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.