EMADOC-1700519818-2115976

European Medicines Agency decision

EMA/PE/0000232767

of 16 May 2025

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for VE303-07: Bacilli, strain from cluster XVII, (closest relative: Clostridium_AQ innocuum) LiveVE303-07 / VE303-06: Clostridia, strain from cluster XIVa, (closest relative: Dorea_A longicatena) Live / VE303-05: Clostridia, strain from cluster XIVa, (closest relative: Blautia sp001304935) Live / VE303-03: Clostridia, strain from cluster XIVa, (closest relative: Sellimonas intestinalis) Live / VE303-02: Clostridia, strain from cluster IV, (closest relative: Anaerotruncus colihominis) LiveVE303-02 / VE303-01: Clostridia, strain from cluster XIVa (closest relative: Enterocloster bolteae) Live / VE303-08: Clostridia, strain from cluster IV, (closest relative: Flavonifractor plautii), Live / VE303-04: Clostridia, strain from cluster XIVa, (closest relative: Clostridium_Q symbiosum) Live (VE303) 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Vedanta Biosciences Inc. on 26 February 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 28 March 2025, 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 VE303-07: Bacilli, strain from cluster XVII, (closest relative: Clostridium_AQ innocuum) LiveVE303-07 / VE303-06: Clostridia, strain from cluster XIVa, (closest relative: Dorea_A longicatena) Live / VE303-05: Clostridia, strain from cluster XIVa, (closest relative: Blautia sp001304935) Live / VE303-03: Clostridia, strain from cluster XIVa, (closest relative: Sellimonas intestinalis) Live / VE303-02: Clostridia, strain from cluster IV, (closest relative: Anaerotruncus colihominis) LiveVE303-02 / VE303-01: Clostridia, strain from cluster XIVa (closest relative: Enterocloster bolteae) Live / VE303-08: Clostridia, strain from cluster IV, (closest relative: Flavonifractor plautii), Live / VE303-04: Clostridia, strain from cluster XIVa, (closest relative: Clostridium_Q symbiosum) Live (VE303), 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 VE303-07: Bacilli, strain from cluster XVII, (closest relative: Clostridium_AQ innocuum) LiveVE303-07 / VE303-06: Clostridia, strain from cluster XIVa, (closest relative: Dorea_A longicatena) Live / VE303-05: Clostridia, strain from cluster XIVa, (closest relative: Blautia sp001304935) Live / VE303-03: Clostridia, strain from cluster XIVa, (closest relative: Sellimonas intestinalis) Live / VE303-02: Clostridia, strain from cluster IV, (closest relative: Anaerotruncus colihominis) LiveVE303-02 / VE303-01: Clostridia, strain from cluster XIVa (closest relative: Enterocloster bolteae) Live / VE303-08: Clostridia, strain from cluster IV, (closest relative: Flavonifractor plautii), Live / VE303-04: Clostridia, strain from cluster XIVa, (closest relative: Clostridium_Q symbiosum) Live (VE303), 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 VE303-07: Bacilli, strain from cluster XVII, (closest relative: Clostridium_AQ innocuum) LiveVE303-07 / VE303-06: Clostridia, strain from cluster XIVa, (closest relative: Dorea_A longicatena) Live / VE303-05: Clostridia, strain from cluster XIVa, (closest relative: Blautia sp001304935) Live / VE303-03: Clostridia, strain from cluster XIVa, (closest relative: Sellimonas intestinalis) Live / VE303-02: Clostridia, strain from cluster IV, (closest relative: Anaerotruncus colihominis) LiveVE303-02 / VE303-01: Clostridia, strain from cluster XIVa (closest relative: Enterocloster bolteae) Live / VE303-08: Clostridia, strain from cluster IV, (closest relative: Flavonifractor plautii), Live / VE303-04: Clostridia, strain from cluster XIVa, (closest relative: Clostridium_Q symbiosum) Live (VE303), 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 Vedanta Biosciences Inc., 19 Blackstone Street, Cambridge, MA 02139-3709, United States.

EMADOC-1700519818-1892822

Amsterdam, 28 March 2025

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

EMA/PE/0000232767

Scope of the application

Active substance(s):

VE303-07: *Bacilli*, strain from cluster XVII, (closest relative: *Clostridium_AQ innocuum*) LiveVE303-07 / VE303-06: *Clostridia*, strain from cluster XIVa, (closest relative: *Dorea_A longicatena*) Live / VE303-05: *Clostridia*, strain from cluster XIVa, (closest relative: *Blautia sp001304935*) Live / VE303-03: *Clostridia*, strain from cluster XIVa, (closest relative: *Sellimonas intestinalis*) Live / VE303-02: *Clostridia*, strain from cluster IV, (closest relative: *Anaerotruncus colihominis*) LiveVE303-02 / VE303-01: *Clostridia*, strain from cluster XIVa (closest relative: *Enterocloster bolteae*) Live / VE303-08: *Clostridia*, strain from cluster IV, (closest relative: *Flavonifractor plautii*), Live / VE303-04: *Clostridia*, strain from cluster XIVa, (closest relative: Clostridium_Q symbiosum) Live (VE303)

Invented name and authorisation status:

See Annex II

Condition(s):

Prevention of *Clostridioides difficile* infection

Pharmaceutical form(s):

Capsule, hard

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Vedanta Biosciences Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Vedanta Biosciences Inc. submitted for agreement to the European Medicines Agency on 26/02/2024 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 02/04/2024.

Supplementary information was provided by the applicant on 30 January 2025. 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 clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

   The Paediatric Committee member of Norway agrees 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:

Prevention of *Clostridioides difficile* infection

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- hard capsule, oral use; age-appropriate formulation, oral use
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Prevention of *Clostridioides difficile* infection

2.1.1. Indication(s) targeted by the PIP

Prevention of recurrence of *Clostridioides difficile* infection in patients at high risk of recurrence following antibacterial treatment for the infection

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)

Hard capsule

Age-appropriate formulation

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of an age-appropriate formulation for children from 1 year to less than 18 years of age
Non-clinical studies	Not applicable
Clinical studies	**Study 2 (VE303-003 - RESTORATiVE303)** Randomised, double-blind, placebo-controlled study to evaluate the efficacy, safety, and microbiota changes following administration of VE303 or placebo for 14 days in preventing *Clostridioides difficile* infection recurrence following completion of a course of standard-of-care

	antibiotics in participants from 12 years to less than 18 years (and adults) with recurrent *Clostridioides difficile* infection and those with primary infection who are at high risk for recurrence **Study 3 (VE303-004)** Randomised, double-blind, placebo-controlled study to evaluate the efficacy, safety, and microbiota changes following administration of VE303 in preventing recurrence of *Clostridioides difficile* infection following completion of a course of standard-of-care antibiotics in participants from 1 year to less than 18 years of age with *Clostridioides difficile* infection
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 June 2032
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.