



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/552717/2024

European Medicines Agency decision P/0390/2024

of 6 December 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for tulisokibart, (EMEA-003556-PIP01-23) 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 Merck Sharp & Dohme B.V. on 14 December 2023 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 18 October 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 tulisokibart, concentrate for solution for infusion, solution for injection, age-appropriate dosage form for parenteral use, intravenous use, subcutaneous 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 tulisokibart, concentrate for solution for infusion, solution for injection, age-appropriate dosage form for parenteral use, intravenous use, subcutaneous 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 tulisokibart, concentrate for solution for infusion, solution for injection, age-appropriate dosage form for parenteral use, intravenous use, subcutaneous 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 Merck Sharp & Dohme B.V., Waarderweg 39, 2031 BN – Haarlem, The Netherlands.

EMA/PDCO/327825/2024
Amsterdam, 18 October 2024

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

EMA-003556-PIP01-23

Scope of the application

Active substance(s):

Tulisokibart

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of ulcerative colitis

Pharmaceutical form(s):

Concentrate for solution for infusion

Solution for injection

Age-appropriate dosage form for parenteral use

Route(s) of administration:

Intravenous use

Subcutaneous use

Name/corporate name of the PIP applicant:

Merck Sharp & Dohme B.V.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Merck Sharp & Dohme B.V. submitted for agreement to the European Medicines Agency on 14 December 2023 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 22 January 2024.

Supplementary information was provided by the applicant on 4 July 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 12 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 member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan 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 ulcerative colitis

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- concentrate for solution for infusion, solution for injection, age-appropriate dosage form for parenteral use, intravenous use, subcutaneous 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 ulcerative colitis

2.1.1. Indication(s) targeted by the PIP

Treatment of moderately to severely active ulcerative colitis

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

From 2 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion

Solution for injection

Age-appropriate dosage form for parenteral use

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of an age-appropriate formulation of tulisokibart for parenteral use.
Non-clinical studies	Study 2 Prenatal and postnatal development study of intravenous tulisokibart in rabbits with assessment of immune system parameters.

Clinical studies	Study 3 Open-label, uncontrolled study evaluating the efficacy, safety, and PK of tulisokibart in paediatric participants from 2 to less than 18 years of age with moderately to severely active ulcerative colitis.
Modelling and simulation analyses	Study 4 Population pharmacokinetic (PopPK) model to simulate tulisokibart exposures in paediatric participants from 2 to less than 18 years of age to predict the paediatric doses in each cohort to be used in PIP study 3.
Other studies	Not applicable.
Extrapolation plan	Studies 3 and 4 are part of an extrapolation plan covering the paediatric population from 2 years to less than 18 years of age, as agreed by the PDCO.

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 2033
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.