

EMA/194423/2022

European Medicines Agency decision P/0127/2022

of 13 April 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for abelacimab (EMEA-003017-PIP01-21) 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 Anthos Therapeutics, Inc. on 19 April 2021 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 25 February 2022, 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 abelacimab, concentrate for solution for injection, 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 abelacimab, concentrate for solution for injection, 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 abelacimab, concentrate for solution for injection, 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 Anthos Therapeutics, Inc., 55 Cambridge Parkway, Suite 103, 02142 – Cambridge, United States.

EMA/PDCO/641390/2021
Amsterdam, 25 February 2022

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

EMA-003017-PIP01-21

Scope of the application

Active substance(s):

Abelacimab

Condition(s):

Treatment of thromboembolic events

Prevention of thromboembolic events

Pharmaceutical form(s):

Concentrate for solution for injection

Route(s) of administration:

Intravenous use

Subcutaneous use

Name/corporate name of the PIP applicant:

Anthos Therapeutics, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Anthos Therapeutics, Inc. submitted for agreement to the European Medicines Agency on 19 April 2021 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 25 May 2021.

Supplementary information was provided by the applicant on 27 October 2021. The applicant proposed modifications to the paediatric investigation plan and waiver.

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)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population, and 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 Norwegian Paediatric Committee member 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 conditions 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 annex 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 thromboembolic events

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- concentrate for solution for injection, intravenous use, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as the needs are already covered;

1.2. Condition:

Prevention of thromboembolic events

The waiver applies to:

- the paediatric population from birth to less than 28 days of age;
- concentrate for solution for injection, intravenous use, subcutaneous use;
- on the grounds that the specific medicinal product is likely to be ineffective;

2. Paediatric investigation plan

2.1. Condition:

Prevention of thromboembolic events

2.1.1. Indication(s) targeted by the PIP

Prevention of thromboembolic events in paediatric patients with malignancies and high risk for thrombosis

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

From 28 days of age to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Concentrate for solution for injection

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Compatibility study
Non-clinical studies	Study 2 Enhanced pre and postnatal development study
Clinical studies	Study 3 Open-label, single arm, single dose, three cohort study to evaluate pharmacokinetics (PK), pharmacodynamics (PD), safety, activity, immunogenicity and to contribute to modelling of the PK/PD of abelacimab in children from 28 days to less than 18 years of age with malignancies and high risk for thrombosis. Study 4 Open-label, randomised, multiple dose, active controlled trial to evaluate safety, efficacy and immunogenicity of abelacimab against standard of care in children from 28 days to less than 18 years of age with malignancies and high risk for thrombosis.
Extrapolation, modelling and simulation studies	Study 5 Modelling and simulation study to define and confirm the dose of abelacimab in children from 28 days to less than 18 years of age with malignancies and high risk for thrombosis.
Other studies	Study 6 Ex-vivo contact surface study. Study 7 In vitro concentration – response study.
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:	No
Date of completion of the paediatric investigation plan:	By December 2034
Deferral for one or more measures contained in the paediatric investigation plan:	Yes