



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/697762/2022

European Medicines Agency decision P/0369/2022

of 9 September 2022

on the acceptance of a modification of an agreed paediatric investigation plan for dafsolimab setaritox/grisnilimab setaritox (EMA-002087-PIP01-16-M01) 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.



European Medicines Agency decision

P/0369/2022

of 9 September 2022

on the acceptance of a modification of an agreed paediatric investigation plan for dafsolimab setaritox/grisnilimab setaritox (EMA-002087-PIP01-16-M01) 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/0053/2020 issued on 29 January 2020,

Having regard to the application submitted by Xenikos BV on 25 April 2022 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 22 July 2022, 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 and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

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 dafsolimab setaritox/grisnilimab setaritox, solution for infusion, intravenous use, including changes to the deferral, 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 Xenikos B.V., Wilhelminasingel 14, 6524 AL – Nijmegen, The Netherlands.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/251490/2022
Amsterdam, 22 July 2022

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMA-002087-PIP01-16-M01

Scope of the application

Active substance(s):

Dafsolimab setaritox/grisnilimab setaritox

Condition(s):

Treatment of acute graft versus host disease

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Xenikos BV

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Xenikos BV submitted to the European Medicines Agency on 25 April 2022 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/0053/2020 issued on 29 January 2020.

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

The procedure started on 23 May 2022.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.



Opinion

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 and to the deferral 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.

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 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 acute graft versus host disease

The waiver applies to:

- the paediatric population from birth to less than 28 days of age;
- solution 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 (aGvHD)

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)

Solution for infusion

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable.
Non-clinical studies	Not applicable.
Clinical studies	Study 1 (deleted in procedure EMEA-002087-PIP01-16-M01) Study 2 Open-label pharmacokinetic, efficacy and safety study in children from 28 days to less than 12 years of age with steroid-refractory acute graft versus host disease. (XEN/TGPED-001) Study 5 (added in procedure EMEA-002087-PIP01-16-M01) Open-label, randomised, multicentre trial, to compare the efficacy and safety of dafsolimab setaritox/grisnilimab setaritox to ruxolitinib in patients from 12 years

	to less than 18 years (and adults) with grade III or IV steroid-refractory acute graft-versus-host disease (SR-aGVHD). (BMT CTN 2002)
Extrapolation, modelling and simulation studies	<p>Study 3</p> <p>Dose-finding modelling and simulation study.</p> <p>Study 4</p> <p>Extrapolation study to analyse existing data on adults and to make inferences to the paediatric population.</p>
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:	No
Date of completion of the paediatric investigation plan:	By July 2028
Deferral for one or more measures contained in the paediatric investigation plan:	Yes