EMA/74601/2023

European Medicines Agency decision

P/0078/2023

of 10 March 2023

on the acceptance of a modification of an agreed paediatric investigation plan for begelomab (EMEA-001744-PIP01-14-M02) 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 European Medicines Agency's decision P/0226/2015 issued on 2 October 2015,

Having regard to the application submitted by Adienne S.r.l SU on 13 October 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 20 January 2023, 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 begelomab, concentrate for 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 ADIENNE S.r.l SU, Via Galileo Galilei, 19, 20867 - Caponago (MB), Italy.

EMA/PDCO/853668/2022
Amsterdam, 20 January 2023

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

EMEA-001744-PIP01-14-M02

Scope of the application

Active substance(s):

Begelomab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of acute graft-versus-host disease

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Adienne S.r.l SU

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Adienne S.r.l SU submitted to the European Medicines Agency on 13 October 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/0226/2015 issued on 2 October 2015.

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

The procedure started on 21 November 2022.

Scope of the modification

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

Opinion

1. 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.

2. 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 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 acute graft-versus-host disease (aGvHD)

The waiver applies to:

- newborn infants (from birth to less than 28 days of age);
- concentrate for 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 acute Graft-versus-Host Disease (aGvHD) in patients who have undergone allogeneic haematopoietic progenitor cell transplantation (HPCT)

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

From 4 weeks to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	**Study 1:** Development of a concentrate for solution for infusion.
Non-clinical studies	Not applicable.
Clinical studies	**Study 2** (*deleted during EMEA-001744-PIP01-14-M02*) **Study 3** (*deleted during EMEA-001744-PIP01-14-M02*) **Study 4** (*added during EMEA-001744-PIP01-14-M02*) Open-label, dose-escalation study in sequential cohorts to evaluate pharmacokinetics, pharmacodynamics and safety of begelomab as an initial treatment in in adolescents from 12 years to less than 18 years of age (and adults) with acute Graft-versus-Host Disease (aGvHD) in combination with steroid therapy. (ADN014)

	Study 5 (*added during EMEA-001744-PIP01-14-M02*) Double blind, randomised, placebo-controlled trial to evaluate pharmacokinetics, safety and efficacy of begelomab in combination with standard steroid therapy compared to placebo plus standard steroid therapy in children from 4 weeks to less than 18 years of age (and adults) with acute Graft-versus-Host Disease (aGvHD) (ADN017)
Modelling and simulation studies	**Study 6** (*added during EMEA-001744-PIP01-14-M02*) Modelling and simulation study to support the use of begelomab in patients from 4 weeks of age to less than 18 years of age with aGvHD.
Other studies	Not applicable.
Extrapolation plan	Studies 4, 5 and 6 are part of an extrapolation plan covering the paediatric population from 4 weeks to less than 12 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:	Yes
Date of completion of the paediatric investigation plan:	By December 2029
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.