



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/627044/2015

European Medicines Agency decision

P/0226/2015

of 2 October 2015

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for begelomab (EMEA-001744-PIP01-14) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency²,

Having regard to the application submitted by Adienne S.r.l. S.U. on 16 February 2015 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 11 September 2015, in accordance with Article 18 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.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for begelomab, concentrate for solution for infusion, intravenous 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 begelomab, concentrate for solution for infusion, intravenous 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 begelomab, concentrate for solution for infusion, intravenous 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 ADIENNE S.r.l. S.U, Via Galileo Galilei, 19, 20867 - Caponago (MB), Italy.

Done at London, 2 October 2015

For the European Medicines Agency
Jordi Llinares Garcia
Head of Division (ad interim)
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/438911/2015
London, 11 September 2015

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

EMA-001744-PIP01-14

Scope of the application

Active substance(s):

Begelomab

Condition(s):

Treatment of acute graft-versus-host disease (aGvHD)

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. S.U.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Adienne S.r.l. S.U. submitted for agreement to the European Medicines Agency on 16 February 2015 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 24 March 2015.

Supplementary information was provided by the applicant on 22 June 2015. The applicant proposed modifications to the paediatric investigation plan.

A meeting with the Paediatric Committee took place on 9 September 2015.

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 18 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)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.

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 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 (aGvHD)

The waiver applies to:

- newborn infants (from birth to less than 28 days);
- 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 steroid-resistant grade II-IV acute graft-versus-host disease (aGvHD) after allogeneic haematopoietic progenitor cell transplantation (HPCT) or donor lymphocyte infusion (DLI)

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	Number of measures	Description
Quality-related studies	1	Study 1: Development of a concentrate for solution for infusion.
Non-clinical studies	0	Not applicable.
Clinical studies	2	Study 2: Open-label, single arm dose-finding trial to evaluate pharmacokinetics, pharmacodynamics and safety of begelomab in children from 4 weeks to less than 18 years of age with steroid-resistant grade II-IV acute graft-versus-host disease (aGvHD) after allogeneic haematopoietic progenitor cell transplantation (HPCT) or donor lymphocyte infusion (DLI). Study 3: Open-label, randomised, active-controlled trial to

		evaluate pharmacokinetics, safety and efficacy of begelomab compared to study site standard-of-care in children from from 4 weeks to less than 18 years of age with steroid-resistant grade II-IV aGvHD after HPCT or DLI.
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

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 March 2021
Deferral for one or more measures contained in the paediatric investigation plan:	Yes