EMA/91887/2018

European Medicines Agency decision

P/0075/2018

of 16 March 2018

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for anifrolumab (EMEA-001435-PIP02-16) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by AstraZeneca AB on 9 September 2016 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 26 January 2018, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for anifrolumab, 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 anifrolumab, 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 anifrolumab, 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 AstraZeneca AB, Södertälje, SE-15185 - Södertälje, Sweden.

EMA/PDCO/765348/2017
London, 26 January 2018

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

EMEA-001435-PIP02-16

Scope of the application

Active substance(s):

Anifrolumab

Condition(s):

Treatment of systemic lupus erythematosus

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intravenous use

Subcutaneous use

Name/corporate name of the PIP applicant:

AstraZeneca AB

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, AstraZeneca AB submitted for agreement to the European Medicines Agency on 9 September 2016 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 18 October 2016.

Supplementary information was provided by the applicant on 3 November 2017. The applicant proposed modifications to the paediatric investigation plan.

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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)(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 systemic lupus erythematosus

The waiver applies to:

- the paediatric population from birth to less than 5 years;
- solution for injection, intravenous use, subcutaneous 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 systemic lupus erythematosus

2.1.1. Indication(s) targeted by the PIP

- Treatment of active, autoantibody-positive patients with systemic lupus erythematosus (SLE) despite receiving standard of care
- Treatment of active, autoantibody-positive patients with lupus nephritis (LN) despite receiving standard of care

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

From 5 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	4	**Study 1:** Observational study to evaluate the magnitude and distribution of the type I IFN gene signature and additional measures of type I IFN activity in children and adolescents from 5 to less than 18 years of age with pSLE or pLN

		Study 2: Placebo controlled, randomised withdrawal study to evaluate pharmacokinetics, efficacy and safety of intravenous anifrolumab in children and adolescents from 5 to less than 18 years of age with pSLE **Study 3:** Open-label, non-comparative, randomised withdrawal study to evaluate pharmacokinetics, pharmacodynamics, efficacy, safety and tolerability of intravenous anifrolumab in children and adolescents from 5 to less than 18 years of age pSLE and moderate to severe LN based on biopsy-proven proliferative nephritis and UPCR ≥1 **Study 4:** Open-label, single-arm trial to evaluate pharmacokinetics, pharmacodynamics and safety of subcutaneous anifrolumab in children and adolescents from 5 to less than 18 years of age with moderate to severely active pSLE
Extrapolation, modelling and simulation studies	4	**Study 5:** Modelling and simulation study to project concentration-time profiles of intravenous anifrolumab and gene signature profiles in paediatric SLE patients (with at most mild LN) **Study 6:** Modelling and simulation study to project concentration-time profiles of subcutaneous anifrolumab and gene signature profiles in paediatric SLE patients (with at most mild LN) **Study 7:** Modelling and simulation study to project concentration-time profiles of intravenous anifrolumab and gene signature profiles in paediatric SLE patients with moderate to severe LN **Study 8:** Extrapolation study to evaluate the use of subcutaneous anifrolumab in children from 5 to less than 18 years of age with pSLE
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:	No
Date of completion of the paediatric investigation plan:	By December 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes