EMA/170358/2023

European Medicines Agency decision

P/0149/2023

of 21 April 2023

on the acceptance of a modification of an agreed paediatric investigation plan for anifrolumab (Saphnelo), (EMEA-001435-PIP02-16-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/0075/2018 issued on 16 March 2018 and the decision P/0239/2020 issued on 16 June 2020,

Having regard to the application submitted by AstraZeneca AB on 16 December 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 31 March 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 anifrolumab (Saphnelo), concentrate for solution for infusion, solution for injection, intravenous use, subcutaneous 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 AstraZeneca AB, Södertälje, SE-151 85 – Södertälje, Sweden.

EMA/PDCO/6568/2023
Amsterdam, 31 March 2023

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

EMEA-001435-PIP02-16-M02

Scope of the application

Active substance(s):

Anifrolumab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of systemic lupus erythematosus

Pharmaceutical form(s):

Concentrate for solution for infusion

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 22 of Regulation (EC) No 1901/2006 as amended, AstraZeneca AB submitted to the European Medicines Agency on 16 December 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/0075/2018 issued on 16 March 2018 and the decision P/0239/2020 issued on 16 June 2020.

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

The procedure started on 30 January 2023.

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 systemic lupus erythematosus

The waiver applies to:

- the paediatric population from birth to less than 5 years of age;
- 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 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for injection

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1 (OBS 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 *This study was deleted as a result of procedure EMEA-001435-PIP02-16-M02.* **Study 3 (pSLE + LN IV study #2)** 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 (pSLE SC study #3)** 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 **Study 9 (pSLE IV study #1)** Double-blind, placebo-controlled, randomised trial to evaluate pharmacokinetics, efficacy and safety of intravenous anifrolumab in children and adolescents from 5 years to less than 18 years of age with pSLE *This study was added as a result of procedure EMEA-001435-PIP02-16-M02.*
Extrapolation, modelling and simulation studies	**Study 5 (pSLE IV MS Study #1, pSLE)** 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 (pSLE SC MS Study #2 pSLE)** 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 (pLN IV MS Study #3 pLN)** 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 (Anifrolumab EXP-1 pSLE - SC route of administration)** Extrapolation study to evaluate the use of subcutaneous anifrolumab in children from 5 to less than 18 years of age with pSLE
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 September 2028
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:

Condition(s) and authorised indication(s)

1. Treatment of Systemic Lupus Erythematosus

Authorised indication(s):

- Saphnelo is indicated as an add-on therapy for the treatment of adult patients with moderate to severe, active autoantibody-positive systemic lupus erythematosus (SLE), despite standard therapy.

  Treatment of moderate to severe, active autoantibody-positive systemic lupus erythematosus (SLE), despite standard therapy in adult patients, in association with drug Saphnelo

  - Invented name(s): Saphnelo
  - Authorised pharmaceutical form(s): Concentrate for solution for infusion
  - Authorised route(s) of administration: Intravenous infusion
  - Authorised via centralised procedure