EMADOC-1700519818-1976753
Corr[1]

European Medicines Agency decision

EMA/PE/0000228081

of 19 March 2025

on the acceptance of a modification of an agreed paediatric investigation plan for voclosporin (Lupkynis) 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.

[1] Corrigendum 16 May 2025 – invented name amended

European Medicines Agency decision

EMA/PE/0000228081

of 19 March 2025

on the acceptance of a modification of an agreed paediatric investigation plan for voclosporin (Lupkynis) 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 726/2004[2],

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[3],

Having regard to the European Medicines Agency's decision P/0152/2019 issued on 17 April 2019,

Having regard to the application submitted by Otsuka Pharmaceutical Netherlands B.V. on 21 October 2024 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 January 2025, 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.

[2] OJ L 378, 27.12.2006, p.1, as amended.
[3] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for voclosporin (Lupkynis), 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 Otsuka Pharmaceutical Netherlands B.V., Herikerbergweg 292, 1101 CT Amsterdam Noord-Holland, Netherlands.

EMADOC-1700519818-1775648 Corr[1]
Amsterdam, 31 January 2025

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

EMA/PE/0000228081

Scope of the application

Active substance(s):

Voclosporin

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of systemic lupus erythematosus

Pharmaceutical form(s):

Capsule, soft

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Otsuka Pharmaceutical Netherlands B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Otsuka Pharmaceutical Netherlands B.V. submitted to the European Medicines Agency on 21 October 2024 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/0152/2019 issued on 17 April 2019.

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

The procedure started on 25 November 2024.

[1] 26 February 2025

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 (SLE)

The waiver applies to:

- the paediatric population from birth to less than 5 years of age;
- capsule, soft, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Treatment of systemic lupus erythematosus (SLE)

2.1.1. Indication(s) targeted by the PIP

Treatment of lupus nephritis in paediatric patients with systemic lupus erythematosus

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)

Capsule, soft

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1** Four group and three period, adaptive design, double-blind controlled versus placebo study of pharmacokinetic, efficacy and safety of voclosporin in lupus nephritis (LN) in addition to standard therapy, in paediatric patients from 5 years to less than 18 years of age with systemic lupus erythematosus (SLE); AURORA PEDS 01 (AURinia vOclosporin Renal Assessments PEDiatricS). **Study 2** Study deleted in EMA/PE/0000228081

1. Waiver

1.1. Condition:

Treatment of systemic lupus erythematosus (SLE)

The waiver applies to:

- the paediatric population from birth to less than 5 years of age;
- capsule, soft, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Treatment of systemic lupus erythematosus (SLE)

2.1.1. Indication(s) targeted by the PIP

Treatment of lupus nephritis in paediatric patients with systemic lupus erythematosus

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)

Capsule, soft

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
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Clinical studies	**Study 1** Four group and three period, adaptive design, double-blind controlled versus placebo study of pharmacokinetic, efficacy and safety of voclosporin in lupus nephritis (LN) in addition to standard therapy, in paediatric patients from 5 years to less than 18 years of age with systemic lupus erythematosus (SLE); AURORA PEDS 01 (AURinia vOclosporin Renal Assessments PEDiatricS). **Study 2** Study deleted in EMA/PE/0000228081

	Study 4 Study added in EMA/PE/0000228081 Prospective, 12-month, open-label, long-term extension safety and activity study of voclosporin in addition to background standard of care with mycophenolate mofetil (MMF) and oral corticosteroids in paediatric participants from 5 years to less than 18 years of age with lupus nephritis (LN) (AUR-VCS-2020-04 / VOCAL-EXT).
Extrapolation, modelling and simulation studies	**Study 3** Analysis of existing in house data on voclosporin on lupus nephritis in paediatric patients to confirm whether observed paediatric data are consistent with extrapolated efficacy predictions from adults.
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:	Yes
Date of completion of the paediatric investigation plan:	By Feb 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:

Condition(s) and authorised indication(s)

1. Treatment of Lupus Nephritis

Authorised indication(s):

- Lupkynis is indicated in combination with mycophenolate mofetil for the treatment of adult patients with active class III, IV or V (including mixed class III/V and IV/V) lupus nephritis (LN).
  - Invented name(s): Lupkynis

Authorised pharmaceutical form(s): soft capsule

Authorised route(s) of administration: oral use

Authorised via centralised procedure