EMA/602330/2022

European Medicines Agency decision

P/0266/2022

of 1 August 2022

on the acceptance of a modification of an agreed paediatric investigation plan for avacopan (Tavneos), (EMEA-002023-PIP01-16-M06) 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/0082/2017 issued on 22 May 2017, decision P/0268/2017 issued on 7 September 2017, decision P/0344/2017 issued on 23 November 2017, decision P/0360/2019 issued on 4 November 2019, decision P/0103/2020 issued on 20 March 2020 and decision P/0181/2021 issued on 10 May 2021,

Having regard to the application submitted by ChemoCentryx Ireland Ltd on 18 February 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 20 May 2022, 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.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[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 avacopan (Tavneos), age-appropriate oral liquid dosage form, age-appropriate oral solid dosage form, capsule, hard, oral use, 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 ChemoCentryx Ireland Ltd, 70 Sir John Rogerson's Quay, Dublin 2, Ireland.

EMA/PDCO/128014/2022
Amsterdam, 20 May 2022

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

EMEA-002023-PIP01-16-M06

Scope of the application

Active substance(s):

Avacopan

Invented name:

Tavneos

Condition(s):

Treatment of anti-neutrophil cytoplasmic auto-antibody (ANCA)-associated vasculitis

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Age-appropriate oral liquid dosage form

Age-appropriate oral solid dosage form

Capsule, hard

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

ChemoCentryx Ireland Ltd

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, ChemoCentryx Ireland Ltd submitted to the European Medicines Agency on 18 February 2022 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0082/2017 issued on 22 May 2017, decision P/0268/2017 issued on 7 September 2017, decision P/0344/2017 issued on 23 November 2017, decision P/0360/2019 issued on 4 November 2019, decision P/0103/2020 issued on 20 March 2020, and decision P/0181/2021 issued on 10 May 2021.

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

The procedure started on 21 March 2022.

Scope of the modification

Some 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 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 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

Not applicable.

2. Paediatric investigation plan

2.1. Condition

Treatment of antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis

2.1.1. Indication(s) targeted by the PIP

Treatment of patients with microscopic polyangiitis (MPA)

Treatment of patients with granulomatosis with polyangiitis (GPA)

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

From birth to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Capsule, hard

Age-appropriate oral solid dosage form

Age-appropriate oral liquid dosage form

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of age-appropriate oral solid dosage form for use in the paediatric population from 2 to less than 12 years of age and of an appropriate dispensing device for granules **Study 2** Development of age-appropriate oral liquid dosage form for use in the paediatric population from birth to less than 12 years of age and of an administration device with suitable graduation for the liquid formulation
Non-clinical studies	**Study 3** 44 week nasogastric / oral toxicity study in cyno monkeys with 6-week recovery **Study 4** Determination of PK parameters of the age-appropriate oral solid dosage form and the age-appropriate oral liquid dosage form in dogs **Study 5** 13-week oral toxicity study in juvenile hamsters with 4-week recovery phase

Clinical studies	**Study 6** Double-blind, double-dummy, randomised, placebo-controlled trial to evaluate safety and efficacy of avacopan as add-on to standard of care compared to prednisone in children from 12 to less than 18 years of age (and adults) with active anti-neutrophil cytoplasmic antibody (ANCA) associated vasculitis **Study 7** Open-label, 3-period, 3-way, crossover, single-dose bioavailability study in adult healthy volunteers to evaluate the PK profile of paediatric formulations **Study 8** Open label, uncontrolled trial to evaluate pharmacokinetics, safety and activity of a single dose of avacopan, followed by multiple doses in children from 6 to less than 12 years of age with active ANCA-associated vasculitis **Study 9** Open label, uncontrolled trial to evaluate pharmacokinetics, safety and activity of a single dose of avacopan, followed by multiple doses in children from birth to less than 6 years of age with active ANCA-associated vasculitis
Extrapolation, modelling and simulation studies	**Study 10** Population PK modelling to support dosing in adolescents from 12 to less than 18 years of age **Study 11** Population PK modelling to support dosing in children from 6 to less than 12 years of age **Study 12** Population PK modelling to support dosing in adolescents from birth to less than 6 years of age **Study 13** Extrapolation study to provide efficacy assumptions in the paediatric population from birth to less than 12 years of age with active ANCA-associated vasculitis based on extrapolation from adult and adolescent patients
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 December 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of anti-neutrophil cytoplasmic auto-antibody (ANCA)-associated vasculitis

Authorised indication(s):

- Tavneos, in combination with a rituximab or cyclophosphamide regimen, is indicated for the treatment of adult patients with severe, active granulomatosis with polyangiitis (GPA) or microscopic polyangiitis (MPA).

Authorised pharmaceutical form(s):

Hard capsule

Authorised route(s) of administration:

Oral administration