EMA/541695/2017

European Medicines Agency decision

P/0268/2017

of 7 September 2017

on the acceptance of a modification of an agreed paediatric investigation plan for avacopan (EMEA-002023-PIP01-16-M01) 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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on the acceptance of a modification of an agreed paediatric investigation plan for avacopan (EMEA-002023-PIP01-16-M01) 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[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 European Medicines Agency's decision P/0082/2017 issued on 22 May 2017,

Having regard to the application submitted by ChemoCentryx, Ltd. on 26 June 2017 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 18 August 2017, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for avacopan, capsule, hard, age-appropriate oral solid dosage form, age-appropriate oral liquid dosage form, oral 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 ChemoCentryx, Ltd., 12 New Fetter Lane, EC4A 1JP - London, United Kingdom.

EMA/PDCO/419913/2017
London, 18 August 2017

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

EMEA-002023-PIP01-16-M01

Scope of the application

Active substance(s):

Avacopan

Condition(s):

Treatment of antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis

Pharmaceutical form(s):

Capsule, hard

Age-appropriate oral solid dosage form

Age-appropriate oral liquid dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

ChemoCentryx, Ltd.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, ChemoCentryx, Ltd. submitted to the European Medicines Agency on 26 June 2017 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.

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

The procedure started on 18 July 2017.

Scope of the modification

Some measures or timelines of the Paediatric Investigation Plan have been modified. A deferral for a measure has been added.

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 Norwegian Paediatric Committee member 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 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

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	Number of measures	Description
Quality-related studies	2	**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	3	**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	4	**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	4	**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	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 December 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes