EMA/224404/2017

European Medicines Agency decision

P/0082/2017

of 22 May 2017

on the agreement of a paediatric investigation plan and on the granting of a deferral for avacopan (EMEA-002023-PIP01-16) 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 ChemoCentryx, Ltd. on 5 July 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 24 March 2017, in accordance with Article 18 of Regulation (EC) No 1901/2006 and Article 21 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.

(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.

[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 avacopan, capsule, hard, age-appropriate oral solid dosage form, age-appropriate oral liquid dosage form, oral 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 avacopan, capsule, hard, age-appropriate oral solid dosage form, age-appropriate oral liquid dosage form, oral 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

This decision is addressed to ChemoCentryx, Ltd., 12 New Fetter Lane, EC4A 1JP - London, United Kingdom.

EMA/PDCO/8416/2017
London, 24 March 2017

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

EMEA-002023-PIP01-16

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 16(1) of Regulation (EC) No 1901/2006 as amended, ChemoCentryx, Ltd. submitted for agreement to the European Medicines Agency on 5 July 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 16 August 2016.

Supplementary information was provided by the applicant on 28 December 2016. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

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 18 of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation.

   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 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