EMA/215078/2019

European Medicines Agency decision

P/0146/2019

of 17 April 2019

on the acceptance of a modification of an agreed paediatric investigation plan for roxadustat (EMEA-001557-PIP01-13-M03) 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 European Medicines Agency's decision P/0020/2015 issued on 30 January 2015, the decision P/0275/2017 issued on 4 October 2017and the decision P/0233/2018 issued on 15 August 2018,

Having regard to the application submitted by Astellas Pharma Europe B.V. on 20 November 2018 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 1 March 2019, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for roxadustat, film-coated tablet, 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 Astellas Pharma Europe B.V., Sylviusweg 62, 2333 BE - Leiden, The Netherlands.

EMA/PDCO/836369/2018
London, 1 March 2019

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

EMEA-001557-PIP01-13-M03

Scope of the application

Active substance(s):

Roxadustat

Condition(s):

Treatment of anaemia due to chronic disorders

Pharmaceutical form(s):

Film-coated tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Astellas Pharma Europe B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Astellas Pharma Europe B.V. submitted to the European Medicines Agency on 20 November 2018 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/0020/2015 issued on 30 January 2015, the decision P/0275/2017 issued on 4 October 2017and the decision P/0233/2018 issued on 15 August 2018.

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

The procedure started on 3 January 2019.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

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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 Norwegian Paediatric Committee member 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 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

1.1. Condition

Treatment of anaemia due to chronic disorders

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- for film-coated tablet for oral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition

Treatment of anaemia due to chronic disorders

2.1.1. Indication(s) targeted by the PIP

Treatment of anaemia associated with chronic kidney disease (CKD)

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

From 6 months to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of lower strength of the film-coated tablet not containing azo dyes
Non-clinical studies	1	**Study 2** Definitive juvenile toxicity study. (ASP1517-PED-NON-CLIN-01)

Clinical studies	3	**Study 3** Open label, randomised, 3-way cross-over study to evaluate the relative bioavailability, PK, food effect and palatability of the paediatric formulation versus adult tablet. (ASP1517-PED-CLIN-01) **Study 4** Open-label, randomised, age-group adjusted starting dose, active-controlled trial to evaluate PK/PD, safety and efficacy of roxadustat compared to recombinant human erythropoietin or its analogues in ESA-naïve children from 6 months to less than 18 years of age with anaemia due to chronic kidney disease stages 3, 4 and 5. (ASP1517-PED-CLIN-03) **Study 5** Open-label, randomised, age-group adjusted starting dose, active-controlled trial to evaluate PK/PD, safety and efficacy of roxadustat compared to recombinant human erythropoietin or its analogues in children from 6 months to less than 18 years of age with anaemia due to chronic kidney disease stages 3, 4 and 5 on stable ESA treatment. (ASP1517-PED-CLIN-04)
Extrapolation, modelling and simulation studies	0	Not applicable
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 July 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes