EMA/584742/2018

European Medicines Agency decision

P/0294/2018

of 12 September 2018

on the acceptance of a modification of an agreed paediatric investigation plan for caplacizumab (EMEA-001157-PIP01-11-M02) 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 caplacizumab (EMEA-001157-PIP01-11-M02) 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/0060/2012 issued on 26 March 2012 and the decision P/0189/2016 issued on 15 July 2016,

Having regard to the application submitted by Ablynx NV on 4 May 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 27 July 2018, 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 caplacizumab, powder and solvent for solution for injection, subcutaneous use, intravenous 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 Ablynx NV, Technologiepark 21, 9052 – Zwijnaarde, Belgium.

EMA/PDCO/298076/2018 Corr
London, 27 July 2018

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

EMEA-001157-PIP01-11-M02

Scope of the application

Active substance(s):

Caplacizumab

Condition(s):

Treatment of thrombotic thrombocytopenic purpura

Pharmaceutical form(s):

Powder and solvent for solution for injection

Route(s) of administration:

Subcutaneous use

Intravenous use

Name/corporate name of the PIP applicant:

Ablynx NV

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Ablynx NV submitted to the European Medicines Agency on 4 May 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/0060/2012 issued on 26 March 2012 and the decision P/0189/2016 issued on 15 July 2016.

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

The procedure started on 29 May 2018.

Scope of the modification

Some measures 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 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 annexand 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

Treatment of thrombotic thrombocytopenic purpura

The waiver applies to:

- infants and children from birth to less than 2 years of age;
- for powder and solvent for solution for injection, subcutaneous and intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan

2.1. Condition

Treatment of thrombotic thrombocytopenic purpura

2.1.1. Indication(s) targeted by the PIP

Treatment of acquired thrombotic thrombocytopenic purpura

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

From 2 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder and solvent for solution for injection

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	0	**Study 2 (ALX-0681-2.1/10)**: *This study was deleted in procedure EMEA-00157-PIP01-11-M01* **Study 3 (ALX-0681-C301)**: *This study was deleted in procedure EMEA-00157-PIP01-11-M02*
Extrapolation, modelling and simulation studies	1	**Study 1 (ALX-0681-MS-01)** Modelling/Simulation (M/S) study using a semi-mechanistic population PK/PD model with parallel linear clearance of the free caplacizumab and non-linear clearance of the caplacizumab-vWF (von Willebrand Factor) complex.

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:	No
Date of completion of the paediatric investigation plan:	By May 2018
Deferral for one or more measures contained in the paediatric investigation plan:	Yes