EMA/666036/2017

European Medicines Agency decision

P/0327/2017

of 31 October 2017

on the acceptance of a modification of an agreed paediatric investigation plan for domagrozumab (EMEA-001763-PIP01-15-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 domagrozumab (EMEA-001763-PIP01-15-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/0108/2016 issued on 15 April 2016,

Having regard to the application submitted by Pfizer Limited on 22 June 2017 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 15 September 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.

(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 domagrozumab, powder for solution for infusion, 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 Pfizer Limited, Ramsgate Road, CT13 9NJ - Sandwich, United Kingdom.

EMA/PDCO/422268/2017
London, 15 September 2017

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

EMEA-001763-PIP01-15-M01

Scope of the application

Active substance(s):

Domagrozumab

Condition(s):

Treatment of Duchenne Muscular Dystrophy

Pharmaceutical form(s):

Powder for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Pfizer Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Pfizer Limited submitted to the European Medicines Agency on 22 June 2017 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/0108/2016 issued on 15 April 2016.

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

The procedure started on 18 July 2017.

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 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 Duchenne Muscular Dystrophy

The waiver applies to:

- the paediatric population from birth to less than 2 years;
- powder for solution for infusion, intravenous use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition

Treatment of Duchenne Muscular Dystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of Duchenne Muscular Dystrophy

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 for solution for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	2	**Study 1** Dose range-finding juvenile toxicity study **Study 2** Definitive juvenile toxicity study

Area	Number of measures	Description
Clinical studies	4	**Study 3** Randomized, double-blind, placebo-controlled, multiple ascending dose study to evaluate the safety, efficacy, PK and PD of Domagrozumab, a humanised monoclonal antibody against myostatin, in ambulatory boys from 6 to less than 16 years with Duchenne Muscular Dystrophy **Study 4** Open-label study to evaluate the long-term safety and maintenance of effect of Domagrozumab in ambulatory boys with Duchenne Muscular Dystrophy who completed study 3 **Study 5** Randomized, double-blind, placebo-controlled study to evaluate the safety and efficacy of Domagrozumab in non-ambulatory boys from 10 to less than 18 years with Duchenne Muscular Dystrophy **Study 6** Open-label study compared to matched historical control to evaluate the safety, pharmacokinetics and efficacy of Domagrozumab in ambulatory boys from 2 to less than 6 years with Duchenne Muscular Dystrophy
Extrapolation, modelling and simulation studies	1	**Study 7** Modelling and simulation study, to determine the dose for the age subset 2 to less than 6 years of age
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 June 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes