EMA/895074/2018

European Medicines Agency decision

P/0043/2019

of 29 January 2019

on the acceptance of a modification of an agreed paediatric investigation plan for bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (RO7239361) (EMEA-001793-PIP01-15-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 decision P/0250/2016 issued on 9 September 2016, the decision P/0084/2017 issued on 16 March 2017 and the decision P/0224/2017 issued on 11 August 2017,

Having regard to the application submitted by Roche Registration GmbH on 5 September 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 14 December 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 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 bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (RO7239361), solution for injection in pre-filled syringe, solution for injection, subcutaneous 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 Roche Registration GmbH, Emil-Barell-Strasse 1, 79639 - Grenzach-Wyhlen, Germany.

EMA/PDCO/664797/2018
London, 14 December 2018

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

EMEA-001793-PIP01-15-M03

Scope of the application

Active substance(s):

Bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (RO7239361)

Condition(s):

Treatment of Duchenne Muscular Dystrophy

Pharmaceutical form(s):

Solution for injection in pre-filled syringe

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Roche Registration GmbH

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Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Roche Registration GmbH submitted to the European Medicines Agency on 5 September 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/0250/2016 issued on 9 September 2016, the decision P/0084/2017 issued on 16 March 2017 and the decision P/0224/2017 issued on 11 August 2017.

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

The procedure started on 16 October 2018.

Scope of the modification

Some measures and timelines 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 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 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;
- solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

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)

Solution for injection

Solution for injection in pre-filled syringe

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of an age-appropriate dosage form.
Non-clinical studies	1	**Study 2** Juvenile toxicity study in rats (DN14071).
Clinical studies	4	**Study 3** Double-blind, randomised, multiple ascending dose study with an open-label phase to assess the safety, tolerability, pharmacokinetics (PK), free-myostatin suppression and immunogenicity of multiple subcutaneous doses of RO7239361 in ambulatory male children from 5 to less than 11 years of age with Duchenne Muscular Dystrophy (WN40226).

		Study 4 Randomised, double blind, placebo-controlled study to assess the efficacy, safety and tolerability of RO7239361 in ambulatory male children from 6 to less than 12 years of age with Duchenne Muscular Dystrophy (WN40227). **Study 5** Randomised, double blind, placebo-controlled study to assess the efficacy, safety and tolerability of RO7239361in non-ambulatory male children from 11 to less than 18 years of age (and adults) with Duchenne Muscular Dystrophy (CN001019). **Study 6** Randomised, double blind, placebo-controlled study to assess the efficacy, safety and tolerability of RO7239361 in male children from 2 to less than 6 years of age with Duchenne Muscular Dystrophy (CN001P46).
Extrapolation, modelling and simulation studies	1	**Study 7** Dose finding modelling and simulation study.
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 April 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes