EMA/104860/2016 Corr

European Medicines Agency decision

P/0079/2016

of 18 March 2016

on the agreement of a paediatric investigation plan for arimoclomol (citrate) (EMEA-001748-PIP01-15) 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 Orphazyme ApS on 16 March 2015 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 29 January 2016, in accordance with Article 18 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 agreement of a paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision agreeing a 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

A paediatric investigation plan for arimoclomol (citrate), capsule, hard, 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

This decision is addressed to Orphazyme ApS, Ole Maaløes Vej 3, 2200 - Copenhagen, Denmark.

Done at London, 18 March 2016

For the European Medicines Agency
Zaïde Frias
Head of Division
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/747765/2015 Corr
London, 29 January 2016

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

EMEA-001748-PIP01-15

Scope of the application

Active substance(s):

Arimoclomol (citrate)

Condition(s):

Treatment of Niemann-Pick disease, type C

Pharmaceutical form(s):

Capsule, hard

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Orphazyme ApS

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Orphazyme ApS submitted for agreement to the European Medicines Agency on 16 March 2015 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 21 April 2015.

Supplementary information was provided by the applicant on 9 November 2015. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

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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.

   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 Niemann-Pick disease, type C

2.1.1. Indication(s) targeted by the PIP

Treatment of Niemann-Pick disease, type C

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

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	3	**Study 1** In-use stability of drug product in beverages **Study 2** In-use stability of drug product dispersed in food **Study 3** Dose recovery by administration through feeding tube of drug product dissolved in water
Non-clinical studies	3	**Study 4** Juvenile toxicity study **Study 5** Reprotoxicity (enhanced) pre- and postnatal developmental study **Study 6** Juvenile toxicity study
Clinical studies	1	**Study 7** Randomised, double-blind, placebo-controlled study to evaluate safety and efficacy of arimoclomol, in addition to best standard of care, in patients diagnosed with Niemann-Pick disease type C

Extrapolation, modelling and simulation studies	1	**Study 8** Modelling and simulation study optimisation of study 7
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 July 2018
Deferral for one or more measures contained in the paediatric investigation plan:	No