EMA/299615/2014

European Medicines Agency decision

P/0151/2014

of 13 June 2014

on the agreement of a paediatric investigation plan and on the granting of a deferral for recombinant human beta-glucuronidase (rhGUS, UX003) (EMEA-001540-PIP01-13) 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 Ultragenyx Pharmaceutical Inc. on 30 August 2013 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 25 April 2014, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 of said Regulation,

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 and on the granting of a deferral.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a 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

A paediatric investigation plan for recombinant human beta-glucuronidase (rhGUS, UX003), solution for injection/infusion, intravenous 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

A deferral for recombinant human beta-glucuronidase (rhGUS, UX003), solution for injection/infusion, intravenous 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 granted.

Article 3

This decision is addressed to Ultragenyx Pharmaceutical Inc., 60 Leveroni Court, 94949 - Novato, California, USA.

Done at London, 13 June 2014

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/590871/2013

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

EMEA-001540-PIP01-13

Scope of the application

Active substance(s):

Recombinant human beta-glucuronidase (rhGUS, UX003)

Condition(s):

Treatment of Mucopolysaccharidosis type VII

Pharmaceutical form(s):

Solution for injection/infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Ultragenyx Pharmaceutical Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Ultragenyx Pharmaceutical Inc. submitted for agreement to the European Medicines Agency on 30 August 2013 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 10 October 2013.

Supplementary information was provided by the applicant on 31 January 2014. The applicant proposed modifications to the paediatric investigation plan.

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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;
   - to grant a deferral in accordance with Article 21 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.

London, 25 April 2014

On behalf of the Paediatric Committee
Dr Dirk Mentzer, Chairman
(Signature on file)

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

1. Waiver

Not applicable

2. Paediatric Investigation Plan

2.1. Condition:

Treatment of Mucopolysaccharidosis type VII

2.1.1. Indication(s) targeted by the PIP

Treatment of Mucopolysaccharidosis type VII (MPS VII)

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)

Solution for injection/infusion

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	1	Study 1: Chronic toxicity study in juvenile cynomolgus monkeys during 26 weeks of dosing.
Clinical studies	4	Study 2: Open-label trial to evaluate the safety, activity and dose of UX003 in children with confirmed diagnosis of mucopolysaccharidosis type VII from 5 to less than 18 years of age (and adults up to 30 years of age) Study 3: Randomized, double-blind, single cross-over from placebo, blind start study to assess the efficacy and safety of UX003 in children with confirmed diagnosis of mucopolysaccharidosis type VII from 5 to less than 18 years of age (and adults up to 30 years of age) Study 4: Open-label study to assess the safety and activity of UX003 in children with confirmed diagnosis of mucopolysaccharidosis type VII from birth to less than 5 years of age Study 5: Open-label, multi-centre study to assess the long-term safety and efficacy of UX003 in children with confirmed diagnosis of mucopolysaccharidosis type VII from 5 to less than 18 years of age (and adults up to 30 years of age)

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By December 2019
Deferral for one or more measures contained in the paediatric investigation plan:	Yes