EMA/600723/2015 Corr

European Medicines Agency decision

P/0209/2015

of 18 September 2015

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant human tripeptidyl peptidase 1 (rhTPP1) (EMEA-001362-PIP01-12-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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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/0260/2013 issued on 29 October 2013,

Having regard to the application submitted by BioMarin International Limited on 27 April 2015 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan and proposing a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 17 July 2015, 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 on the refusal of a deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision on the refusal of 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

Changes to the agreed paediatric investigation plan for recombinant human tripeptidyl peptidase 1 (rhTPP1), concentrate for solution for infusion, intracerebral 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

A deferral for recombinant human tripeptidyl peptidase 1 (rhTPP1), concentrate for solution for infusion, intracerebral use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby refused.

Article 3

This decision is addressed to BioMarin International Limited, Shanbally, Ringaskiddy, County Cork, Ireland

Done at London, 18 September 2015

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

EMA/PDCO/286891/2015
London, 17 July 2015

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

EMEA-001362-PIP01-12-M02

Scope of the application

Active substance(s):

Recombinant human tripeptidyl peptidase 1 (rhTPP1)

Condition(s):

Treatment of Neuronal Ceroid Lipofuscinosis type 2

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intracerebral use

Name/corporate name of the PIP applicant:

BioMarin International Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, BioMarin International Limited submitted to the European Medicines Agency on 27 April 2015 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0260/2013 issued on 29 October 2013.

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

The procedure started on 19 May 2015.

A meeting with the Paediatric Committee took place on 15 July 2015.

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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, by a majority of 23 out of 26 votes:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion;
   - to refuse a deferral as it does not meet the grounds detailed in Article 20(1) of said Regulation.

   The divergent positions are appended to 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 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 annexes 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 Neuronal Ceroid Lipofuscinosis type 2

2.1.1. Indication(s) targeted by the PIP

Treatment of Late Infantile Neuronal Ceroid Lipofuscinosis type 2 (CLN2) Disease

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)

Concentrate for solution for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	2	**Measure 1:** Open-label, dose-escalation trial to evaluate pharmacokinetics, safety, immunogenicity, tolerability and efficacy of multiple doses of recombinant human tripeptidyl peptidase-1 (rhTPP1) compared to historical control in children from birth to 18 years of age with Late-Infantile Neuronal Ceroid Lipofuscinosis type 2 (CLN2) Disease. **Measure 2:** Non interventional natural history study to assess the clinical course of Late Infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease.
Extrapolation, modelling and simulation studies	0	Not applicable.

Other studies	0	Not applicable.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By June 2016
Deferral for one or more measures contained in the paediatric investigation plan:	No