EMA/587131/2013

European Medicines Agency decision

P/0260/2013

of 29 October 2013

on the agreement of a paediatric investigation plan for recombinant human tripeptidyl peptidase-1 (EMEA-001362-PIP01-12) 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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

European Medicines Agency decision

P/0260/2013

of 29 October 2013

on the agreement of a paediatric investigation plan for recombinant human tripeptidyl peptidase-1 (EMEA-001362-PIP01-12) 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 application submitted by BioMarin Europe Limited on 3 September 2012 under Article 16(1) of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 September 2013, 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 recombinant human tripeptidyl peptidase-1, concentrate for solution for infusion, intraventricular 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 BioMarin Europe Limited, 164 Shaftesbury Avenue, WC2H 8HL – London, United Kingdom

Done at London, 29 October 2013

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

EMA/PDCO/389828/2013

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

EMEA-001362-PIP01-12

Scope of the application

Active substance(s):

Recombinant human tripeptidyl peptidase-1

Condition(s):

Treatment of Neuronal Ceroid Lipofuscinosis type 2

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intraventricular use

Name/corporate name of the PIP applicant:

BioMarin Europe Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, BioMarin Europe Limited submitted for agreement to the European Medicines Agency on 3 September 2012 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 10 October 2012.

Supplementary information was provided by the applicant on 21 June 2013. The applicant proposed modifications to the paediatric investigation plan.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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 Icelandic and the Norwegian Paediatric Committee members agree 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, 13 September 2013

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 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	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	3	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: Open-label, multi-centre, trial to evaluate, long term safety, tolerability, immunogenicity and efficacy 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 3: Non interventional natural history study to assess the clinical course of Late Infantile Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease.

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 July 2018
Deferral for one or more measures contained in the paediatric investigation plan:	No