EMA/247811/2011

European Medicines Agency decision

P/90/2011

of 8 April 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral for recombinant human N-acetylgalactosamine-6-sulfatase (EMEA-000973-PIP01-10) 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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An agency of the European Union

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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 BioMarin Europe Limited on 8 April 2010 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation, and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 18 February 2011, 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 N-acetylgalactosamine-6-sulfatase, concentrate for solution for 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 human N-acetylgalactosamine-6-sulfatase, concentrate for solution for 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 BioMarin Europe Limited, 164 Shaftesbury Avenue, London, WC2H 8HL.

Done at London, 8 April 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director
(Signature on file)

EMA/PDCO/9330/2011

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

EMEA-000973-PIP01-10

Scope of the application

Active substance(s):

Recombinant human N-acetylgalactosamine-6-sulfatase

Condition(s):

Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome)

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous 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 8 April 2010 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 20 May 2010.

Supplementary information was provided by the applicant on 6 December 2010 and on 7 February 2011. 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 Icelandic and the Norwegian Paediatric Committee member(s) 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(es) and appendix.

London, 18 February 2011

On behalf of the Paediatric Committee
Dr Daniel Brasseur, 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 IVA (Morquio A Syndrome)

2.1.1. Indication(s) targeted by the PIP

Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome)

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. Studies

Area	Number of studies	Description
Quality	None	Not applicable.
Non-clinical	5	Study 1: Embryo-foetal development in rabbits(dose range finding) Study 2: Combined fertility and embryo-foetal developmental study in rats Study 3: Embryo-foetal development in rabbits Study 4: Peri- and post-natal developmental study in rats Study 5: Repeat dose toxicity and toxicokinetics study in monkey
Clinical	4	Study 6: Open-label, multicentre, dose-escalation safety study in children from 5 to less than 18 years Study 7: Open-label, multicentre, extension study in adults and children from 5 years of age Study 8: Double-blind, randomised, multicentre, 3-arm, placebo-controlled, efficacy and safety study in adults and children from 5 years of age Study 9: Open-label, multicentre, safety and efficacy study in children under 5 years of age

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By December 2014
Deferral for one or more studies contained in the paediatric investigation plan:	Yes