EMA/508748/2013

European Medicines Agency decision

P/0194/2013

of 29 August 2013

on the agreement of a paediatric investigation plan and on the granting of a waiver for idursulfase (EMEA-000294-PIP02-12) 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.

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on the agreement of a paediatric investigation plan and on the granting of a waiver for idursulfase (EMEA-000294-PIP02-12) 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 Shire Human Genetic Therapies AB on 8 October 2012 under Article 15(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 9 August 2013, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 12 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 waiver.

(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 waiver.

[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 idursulfase, solution for injection, intrathecal 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 product-specific waiver for idursulfase, solution for injection, intrathecal 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 Shire Human Genetic Therapies AB, Svärdvägen 11D, 182 33 – Danderyd, Sweden.

Done at London, 29 August 2013

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

EMA/PDCO/330130/2013

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

EMEA-000294-PIP02-12

Scope of the application

Active substance(s):

Idursulfase

Condition(s):

Treatment of Mucopolysaccharidosis II (Hunter syndrome)

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intrathecal use

Name/corporate name of the PIP applicant:

Shire Human Genetic Therapies AB

Basis for opinion

Pursuant to Article 15 of Regulation (EC) No 1901/2006 as amended, Shire Human Genetic Therapies AB submitted for agreement to the European Medicines Agency on 8 October 2012 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 14 November 2012.

Supplementary information was provided by the applicant on 17 May 2013. 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;
   - to grant a waiver for one or more subsets of the paediatric population on its own motion in accordance with Article 12 of said Regulation and concluded in accordance with Article 11(1)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.

   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 and the subset(s) of the paediatric population and condition(s) covered by the waiver 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, 9 August 2013

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

Condition: treatment of Mucopolysaccharidosis II (Hunter syndrome)

The waiver applies to:

- girls from birth to less than 18 years;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan

2.1. Condition: treatment of Mucopolysaccharidosis II (Hunter syndrome)

2.1.1. Indication(s) targeted by the PIP

Treatment of MPS II with cognitive impairment.

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

2.1.4. Measures

Area	Number of measures	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	2	Measure 1: Open-label, assessor-blind, randomised, multi-centre, single dose, two-arm, controlled trial to evaluate, pharmacokinetics/ pharmacodynamic, safety, immunogenicity and efficacy of intrathecal (IT) administration of idursulfase as add-on to intravenous administration of idursulfase in boys from birth to 18 with MPS II with early cognitive impairment. Measure 2: Open-label, extension study to evaluate, the safety, pharmacokinetics, activity, immunogenicity of intrathecal administration of idursulfase as add-on to intravenous administration of idursulfase in boys from 3 years to less than 18 with MPS II with cognitive impairment.

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 December 2015
Deferral for one or more measures contained in the paediatric investigation plan:	No