EMA/34943/2017

European Medicines Agency decision

P/0008/2017

of 31 January 2017

on the acceptance of a modification of an agreed paediatric investigation plan for idursulfase (EMEA-000294-PIP02-12-M01) 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 acceptance of a modification of an agreed paediatric investigation plan for idursulfase (EMEA-000294-PIP02-12-M01) 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 European Medicines Agency's decision P/0194/2013 issued on 29 August 2013,

Having regard to the application submitted by Shire Human Genetic Therapies AB on 26 September 2016 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 16 December 2016, 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.

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

Changes to the agreed paediatric investigation plan for idursulfase, solution for injection, intrathecal 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

This decision is addressed to Shire Human Genetic Therapies AB, Vasagatan 7, 111 20 – Stockholm, Sweden.

EMA/PDCO/654011/2016
London, 16 December 2016

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

EMEA-000294-PIP02-12-M01

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 22 of Regulation (EC) No 1901/2006 as amended, Shire Human Genetic Therapies AB submitted to the European Medicines Agency on 26 September 2016 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0194/2013 issued on 29 August 2013.

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

The procedure started on 18 October 2016.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

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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:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

   The measures and timelines of the 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.

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

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

The waiver applies to:

- girls from birth to less than 18 years;
- solution for injection, intrathecal use;
- 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

Boys 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-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	2	**Study 1** Open-label, assessor-blind, randomised, multi-centre, 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.

Area	Number of measures	Description
		Study 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/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By November 2017
Deferral for one or more measures contained in the paediatric investigation plan:	No