EMA/107868/2016

European Medicines Agency decision

P/0083/2016

of 18 March 2016

on the agreement of a paediatric investigation plan for autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human Wiskott Aldrich Syndrome (WAS) cDNA sequence (EMEA-001792-PIP01-15) 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 application submitted by GlaxoSmithKline Trading Services Limited on 8 May 2015 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 29 January 2016, 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 autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human Wiskott Aldrich Syndrome (WAS) cDNA sequence, dispersion 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

This decision is addressed to GlaxoSmithKline Trading Services Limited, Currabinny, County Cork, 999937 – Carrigaline, Ireland.

Done at London, 18 March 2016

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

EMA/PDCO/473012/2015
London, 29 January 2016

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

EMEA-001792-PIP01-15

Scope of the application

Active substance(s):

Autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human Wiskott Aldrich Syndrome (WAS) cDNA sequence

Condition(s):

Treatment of Wiskott Aldrich Syndrome

Pharmaceutical form(s):

Dispersion for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

GlaxoSmithKline Trading Services Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, GlaxoSmithKline Trading Services Limited submitted for agreement to the European Medicines Agency on 8 May 2015 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 16 June 2015.

Supplementary information was provided by the applicant on 5 November 2015. The applicant proposed modifications to the paediatric investigation plan.

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 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 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

Not applicable.

2. Paediatric investigation plan

2.1. Condition:

Treatment of Wiskott Aldrich Syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of Wiskott Aldrich 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)

Dispersion 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	1	Open-label, non-randomised, single dose trial to evaluate safety and activity of autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with lentiviral vector that encodes for the human Wiskott Aldrich Syndrome (WAS) cDNA sequence in children from birth to less than 18 years of age with WAS.
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/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By May 2016
Deferral for one or more measures contained in the paediatric investigation plan:	No