EMA/585489/2016

European Medicines Agency decision

P/0260/2016

of 5 October 2016

on the acceptance of a modification of an agreed paediatric investigation plan for autologous CD34+ cells transduced with lentiviral vector containing the human Wiskott Aldrich Syndrom Protein gene (EMEA-000786-PIP01-09-M02) 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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Contact EMA www.ema.europa.eu/contact

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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 European Medicines Agency's decision P/0266/2013 issued on 30 October 2013 and the decision P/0137/2014 issued on 11 June 2014,

Having regard to the application submitted by Genethon on 30 May 2016 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan and proposing a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 19 August 2016, in accordance with Article 22 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 acceptance of changes to the agreed paediatric investigation plan and on the refusal of a deferral.

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

(3) It is therefore appropriate to adopt a decision on the refusal of 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

Changes to the agreed paediatric investigation plan for autologous CD34+ cells transduced with lentiviral vector containing the human Wiskott Aldrich Syndrom Protein gene, cell suspension for infusion, intravenous 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

A deferral for autologous CD34+ cells transduced with lentiviral vector containing the human Wiskott Aldrich Syndrom Protein gene, cell suspension for infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby refused.

Article 3

This decision is addressed to Genethon, 1 bis rue de l'Internationale, 91002 - Evry Cedex, France.

EMA/PDCO/389729/2016
London, 19 August 2016

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

EMEA-000786-PIP01-09-M02

Scope of the application

Active substance(s):

Autologous CD34+ cells transduced with lentiviral vector containing the human Wiskott Aldrich Syndrom Protein gene

Condition(s):

Treatment of Wiskott-Aldrich syndrome

Pharmaceutical form(s):

Cell suspension for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Genethon

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Genethon submitted to the European Medicines Agency on 30 May 2016 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0266/2013 issued on 30 October 2013 and the decision P/0137/2014 issued on 11 June 2014.

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

The procedure started on 21 June 2016.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5510
Send a question via our website www.ema.europa.eu/contact

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

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;
   - to refuse a deferral as it does not meet the grounds detailed in Article 20(1) 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 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 annexes 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)

Cell suspension for infusion.

2.1.4. Measures

Area	Number of measures	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	2	Measure 1) GTG002-07 Open-label, single arm study of haematopoietic stem cell gene therapy in the treatment of the Wiskott-Aldrich Syndrome Measure 2) GTG003.08 Open-label, single arm study of haematopoetic stem cell gene therapy in the treatment of the Wiscott-Aldrich-Syndrome

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By July 2016
Deferral for one or more measures contained in the paediatric investigation plan:	No