EMA/113454/2013

European Medicines Agency decision

P/0071/2013

of 26 March 2013

on the agreement of a paediatric investigation plan and on the refusal of a waiver for autologous CD34+ Cells Transduced ex-vivo with Retroviral Vector (GIADAI) Containing Human Adenosine Deaminase Gene from cDNA (EMEA-001289-PIP01-12) 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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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 Glaxo Group Ltd on 10 May 2012 under Article 16(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 8 February 2013, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 13 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 refusal 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 refusing 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 autologous CD34+ Cells Transduced ex-vivo with Retroviral Vector (GIADAI) Containing Human Adenosine Deaminase Gene from cDNA, cell suspension 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 waiver for autologous CD34+ Cells Transduced ex-vivo with Retroviral Vector (GIADAI) Containing Human Adenosine Deaminase Gene from cDNA, cell suspension 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 refused.

Article 3

This decision is addressed to Glaxo Group Ltd, Glaxo Wellcome House, UB6ONN Greenford, United Kingdom.

Done at London, 26 March 2013

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

EMA/PDCO/793313/2012

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

EMEA-001289-PIP01-12

Scope of the application

Active substance(s):

Autologous CD34+ Cells Transduced ex-vivo with Retroviral Vector (GIADAI) Containing Human Adenosine Deaminase Gene from cDNA

Condition(s):

Treatment of severe combined immunodeficiency due to adenosine deaminase deficiency

Pharmaceutical form(s):

Cell suspension for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Glaxo Group Ltd

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Glaxo Group Ltd submitted for agreement to the European Medicines Agency on 10 May 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 19 June 2012.

Supplementary information was provided by the applicant on 16 November 2012. The applicant proposed modifications to the paediatric investigation plan.

A meeting with the Paediatric Committee took place on 6 February 2013.

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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 refuse the granting of a waiver in accordance with Article 13 of said Regulation, for some of the subsets of the paediatric population and the above mentioned condition(s) as it does not meet the grounds detailed in Article 11(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 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.

London, 8 February 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

1.1. Condition: Treatment of severe combined immunodeficiency due to adenosine deaminase deficiency

The request for the waiver applied to:

- the paediatric population from birth to less than 28 days;
- for cell suspension for infusion, intravenous use.

The waiver request does not provide evidence to support the following grounds set out in Article 11(1) of Regulation (EC) No 1901/2006 that:

- the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

Because:

- measures would be justified by the expected therapeutic benefit and clinical trials may be feasible.

The waiver request is therefore refused by the PDCO.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of severe combined immunodeficiency due to adenosine deaminase deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment of severe combined immunodeficiency due to adenosine deaminase deficiency with no HLA-identical family bone marrow donor available.

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	
Non-clinical	0	
Clinical	1	Measure 1: Open-label study, prospective, sequential, single centre to evaluate the safety and efficacy of gene therapy in children from birth to less than 18 years of age diagnosed with Adenosine Deaminase Severe Combined Immunodeficiency (ADA-SCID).

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 June 2011
Deferral for one or more measures contained in the paediatric investigation plan:	No