



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/391460/2014

European Medicines Agency decision

P/0190/2014

of 6 August 2014

on the acceptance of a modification of an agreed paediatric investigation plan for autologous CD34+ Cells Transduced ex-vivo with Retroviral Vector (GIADAI) Containing Human Adenosine Deaminase Gene from cDNA (EMEA-001289-PIP01-12-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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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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0071/2013 issued on 26 March 2013,

Having regard to the application submitted by GlaxoSmithKline Trading Services Limited on 26 March 2014 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 20 June 2014, 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 autologous CD34+ Cells Transduced ex-vivo with Retroviral Vector (GIADAI) Containing Human Adenosine Deaminase Gene from cDNA, 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

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

Done at London, 6 August 2014

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

EMA/PDCO/194051/2014

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

EMA-001289-PIP01-12-M01

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:

GlaxoSmithKline Trading Services Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, GlaxoSmithKline Trading Services Limited submitted to the European Medicines Agency on 26 March 2014 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0071/2013 issued on 26 March 2013.

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

The procedure started on 23 April 2014.

Scope of the modification

Some measures and timelines 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:
 - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

The Icelandic and the Norwegian Paediatric Committee members agree 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 annex and appendix.

London, 20 June 2014

On behalf of the Paediatric Committee
Dr Dirk Mentzer, 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 (PIP)

1. Waiver

Not applicable.

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-related studies	0	
Non-clinical studies	0	
Clinical studies	1	Study 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/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By July 2011
Deferral for one or more measures contained in the paediatric investigation plan:	No