

EMA/639652/2016

European Medicines Agency decision

P/0285/2016

of 4 November 2016

on the acceptance of a modification of an agreed paediatric investigation plan for alipogene tiparvovec (Glybera), (EMA-000292-PIP01-08-M03) 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/119/2008 issued on 1 December 2008, and the decision P/0205/2013 issued on 3 September 2013,

Having regard to the application submitted by uniQure biopharma B.V. on 27 June 2016 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 16 September 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 and to the deferral and to the waiver.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral and to the 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

Changes to the agreed paediatric investigation plan for alipogene tiparvovec (Glybera), solution for injection, intramuscular use, including changes to the deferral and to the waiver, 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 uniQure biopharma B.V., Meibergdreef 61, 1105 BA – Amsterdam, The Netherlands.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/450207/2016

London, 16 September 2016

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan and on the granting of a product-specific waiver

EMA-000292-PIP01-08-M03

Scope of the application

Active substance(s):

Alipogene tiparvovec

Invented name:

Glybera

Condition(s):

Treatment of hyperchylomicronaemia

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intramuscular use

Name / corporate name of the PIP applicant:

uniQure biopharma B.V.

Information about the authorised medicinal product:

See Annex II



Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, uniQure biopharma B.V. submitted to the European Medicines Agency on 27 June 2016 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/119/2008 issued on 1 December 2008, and the decision P/0205/2013 issued on 3 September 2013.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral and changes to the waiver to cover the remaining subsets of the paediatric population.

The procedure started on 19 July 2016.

Scope of the modification

The waiver has been extended to cover all subsets of the paediatric population.

Opinion

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 and to the deferral and to amend the scope of the waiver in accordance with Article 11(1)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population, and Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients, as set out in Annex I of this opinion.

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

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

Grounds for the granting of the waiver

1. Waiver

1.1. Condition

Treatment of hyperchylomicronaemia

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- for solution for injection, intramuscular use;
- on the grounds that the specific medicinal product is likely to be unsafe.

The waiver applies to:

- the paediatric population from 2 to less than 18 years of age;
- for solution for injection, intramuscular use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s)

1. Treatment of Hyperchylomicronaemia

Authorised indication(s):

- Glybera is indicated for adult patients diagnosed with familial lipoprotein lipase deficiency (LPLD) and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. The diagnosis of LPLD has to be confirmed by genetic testing. The indication is restricted to patients with detectable levels of LPL protein.

Authorised pharmaceutical form(s)

Solution for injection

Authorised route(s) of administration

Intramuscular use