19 July 2012
EMA/CHMP/472167/2012
Committee for Medicinal Products for Human Use (CHMP)

Summary of positive opinion[1] for Glybera

Further to a request from the European Commission in the framework of the Standing Committee procedure

International nonproprietary name (INN): Alipogene tiparvovec

Following a request from the European Commission, the Committee for Medicinal Products for Human Use (CHMP) re-evaluated the medicinal product Glybera in a restricted patient population with severe or multiple pancreatitis attacks. On 19 July 2012, the CHMP adopted a positive opinion[2] recommending the granting of a marketing authorisation under exceptional circumstances[3] for Glybera, 3 x 10^{12} gc/ml, solution for injection, for treatment of patients diagnosed with lipoprotein lipase deficiency and suffering of severe or multiple pancreatitis attacks. Glybera was designated as an orphan medicinal product on 8 March 2004. The applicant for this medicinal product is uniQure biopharma B.V.

The active substance of Glybera, alipogene tiparvovec, is a lipid modifying agent (ATC Code C10 AX10). Glybera is a gene therapy medicinal product constructed with an adeno-associated viral vector carrying the gene for lipoprotein lipase protein, which is expressed in muscle after administration.

The benefits with Glybera are its ability to allow expression of the lipoprotein lipase protein in deficient patients, suffering from severe or multiple pancreatitis attacks. Glybera must be used in conjunction with appropriate diet. The most common side effects are pain in extremities reported in about one third of patients. A pharmacovigilance plan for Glybera, as for all medicinal products, will be implemented as part of the marketing authorisation.

The approved indication is: 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 (see section 4.4)."

It is proposed for Glybera to be prescribed and administered under the supervision of a physician with expertise in treating patients with lipoprotein lipase deficiency and in gene therapy administration, in full consultation with the patient.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued within 67 days from adoption of the opinion.
[2] Applicants may request a re-examination of any CHMP opinion, provided they notify the Agency in writing of their intention to request a re-examination within 15 days of receipt of the opinion.
[3] Marketing authorisation under exceptional circumstances refers to the fact that in exceptional circumstances an authorisation may be granted subject to certain specific obligations, to be reviewed annually.

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Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and will be available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

The CHMP, on the basis of quality, safety and efficacy data submitted, considers that there is a favourable benefit-to-risk balance for Glybera in the restricted indication and therefore recommends the granting of the marketing authorisation under exceptional circumstances[4].

[4] Marketing authorisation under exceptional circumstances refers to the fact that in exceptional circumstances an authorisation may be granted subject to certain specific obligations, to be reviewed annually.