29 March 2012
EMA/COMP/139305/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lentiviral vector expressing the truncated form of human tyrosine hydroxylase gene, human aromatic L amino-acid decarboxylase gene, human GTP-cyclohydrolase 1 gene for the treatment of 'off'-periods in adult patients with advanced Parkinson's disease who are not responding adequately to L-DOPA treatment

On 9 September 2010, the Committee for Orphan Medicinal Products (COMP) adopted a negative opinion on the orphan designation application for 'lentiviral vector expressing the truncated form of human tyrosine hydroxylase gene, human aromatic L amino-acid decarboxylase gene, human GTP-cyclohydrolase 1 gene' for the treatment of 'off'-periods in adult patients with advanced Parkinson's disease who are not responding adequately to L-DOPA treatment. A negative decision was issued by the European Commission on 31 May 2011.

The sponsor applied for orphan designation on the basis of the seriousness and the rarity of the condition, as well as an assumption of potential benefit over currently available methods of treatment.

The negative opinion was based on the following reasons:

- The COMP considered that the condition applied for ('off'-periods in adult patients with advanced Parkinson's disease who are not responding adequately to L-DOPA treatment) is not a distinct, recognisable medical entity but rather a stage of a broader medical condition, namely Parkinson's disease, which is not rare.
- In addition, the Committee considered that the condition applied for is not a valid subset for orphan designation, because it could not be excluded that the medicinal product could be used in milder forms of Parkinson's disease as opposed to the proposed restricted subset.
- Since the Committee was of the opinion that there was no distinct subset of patients within Parkinson's disease in which the product would be used, the prevalence of the broader medical condition (Parkinson's disease) should have been taken into account. This is more than 5 in 10,000 people in the European Union and therefore above the ceiling for orphan designation.

Requests for designation as an orphan medicinal product are made for investigational products. Absence of orphan designation does not preclude the development of this product, including its use in

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clinical trials. A marketing authorisation can still be obtained if quality, safety and efficacy are demonstrated.

For more information:

Sponsor's contact details:

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.