1 April 2016
EMA/CHMP/160482/2016
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Strimvelis

autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence

On 1 April 2016, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Strimvelis, intended for the treatment of severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID).

Strimvelis was designated as an orphan medicinal product on 26 August 2005. As Strimvelis is an advanced therapy medicinal product, the CHMP positive opinion is based on an assessment by the Committee for Advanced Therapies. The applicant for this medicinal product is GlaxoSmithKline Trading Services.

Strimvelis will be available as dispersion for infusion (1-10 million cells/ml). The active substance of Strimvelis is autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with a retroviral vector that encodes for the human ADA cDNA sequence. After infusion, CD34+ cells engraft in the bone marrow where they repopulate the haematopoetic system with cells, a proportion of which expresses the pharmacologically active levels of the ADA enzyme. Following engraftment in the patient, the effects of the product are expected to be life-long. Patients are expected to be enrolled in a post-treatment registry and will be followed-up long term.

In a pivotal clinical study in patients treated with Strimvelis the survival rate was 100%, with an average follow-up period of 7 years. ADA-SCID is usually fatal in the first 1-2 years of life if left untreated. There was also evidence of immune reconstitution with an increase in CD3+ T cells as well as T cell subsets and some evidence of thymopoiesis as well as of peripheral T cell function. The most common side effects are pyrexia, increased hepatic enzyme levels, autoimmune reactions, such as anaemia, neutropenia, and autoimmune haemolytic anaemia, aplastic anaemia and thrombocytopenia.

The full indication is: "Strimvelis is indicated for the treatment of patients with severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID), for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available". It is proposed that Strimvelis be

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

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administered in a specialist transplant centre, by a physician with previous experience in the treatment and management of patients with ADA-SCID and in the use of autologous CD34+ *ex vivo* gene therapy products.

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 made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.