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EPAR summary for the public

Strimvelis

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

This is a summary of the European public assessment report (EPAR) for Strimvelis. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Strimvelis.

For practical information about using Strimvelis, patients should read the package leaflet or contact their doctor or pharmacist.

What is Strimvelis and what is it used for?

Strimvelis is a medicine used to treat severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID). ADA-SCID is a rare inherited condition in which there is a change (mutation) in the gene needed to make an enzyme called adenosine deaminase (ADA). As a result, patients lack the ADA enzyme. Because ADA is essential for maintaining healthy lymphocytes (white blood cells that fight off infections), the immune system of patients with ADA-SCID does not work properly and without effective treatment they rarely survive more than 2 years.

Strimvelis is used in patients with ADA-SCID who cannot be treated by a bone-marrow transplant because they do not have a suitable, matched, related donor.

Strimvelis contains cells derived from the patient's own bone marrow. Some of the cells (called CD34+ cells) have been genetically modified to contain a working gene for ADA. Strimvelis is a type of advanced therapy medicine called a 'gene therapy product'. This type of medicine works by delivering genes into the body.

Because the number of patients with ADA-SCID is low, the disease is considered 'rare', and Strimvelis was designated an 'orphan medicine' (a medicine used in rare diseases) on 26 August 2005.

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How is Strimvelis used?

Strimvelis can only be obtained with a prescription and treatment should only be given in a specialist transplant centre by a doctor who has experience in the treatment of ADA-SCID and the use of this type of medicine.

To prepare Strimvelis, two samples of the patient's bone marrow are collected, one to make Strimvelis and one that is kept as a back-up in case Strimvelis cannot be given or does not work. Strimvelis can only be used to treat the same patient whose bone marrow was used to make the medicine. Strimvelis is given as an infusion (drip) into a vein over about 20 minutes. The dose depends on the bodyweight of the patient.

Before Strimvelis is given, patients receive conditioning (preparatory) treatment with another medicine, busulfan, to get rid of their abnormal bone marrow cells. Patients are also given an antihistamine injection just before treatment to reduce the risk of allergic reactions.

For further information, see the package leaflet.

How does Strimvelis work?

To make Strimvelis, a sample of the patient's bone marrow is collected. Then, CD34+ cells (cells that can make lymphocytes) are extracted from the bone marrow cells. A working gene for ADA is inserted into the CD34+ cells using a type of virus called a retrovirus, which has been altered genetically so that it can carry the ADA gene into cells and does not cause viral disease in humans.

Once given back to the patient into a vein, Strimvelis is transported in the bloodstream to the bone marrow where the CD34+ cells start to grow and make normal lymphocytes that can produce ADA. These lymphocytes improve the patient's ability to fight infection, and so overcome the symptoms of the condition related to the immune system. The effects are expected to last for the patient's lifetime.

What benefits of Strimvelis have been shown in studies?

The benefits of Strimvelis have been shown in one main study involving 12 patients from 6 months to around 6 years old with ADA-SCID. Patients in the study had no appropriate bone marrow donor and alternative treatment had not worked or was not available. All patients were treated with Strimvelis and were still alive 3 years after treatment. The rate of severe infections declined after treatment and continued to decline with longer-term follow-up beyond 3 years.

What are the risks associated with Strimvelis?

The most common side effect with Strimvelis (which may affect up to 1 in 10 people) is pyrexia (fever). Serious side effects with Strimvelis may include effects linked to autoimmunity (when the immune system attacks the body's own cells) such as haemolytic anaemia (low red blood cell counts due to their too rapid breakdown), aplastic anaemia (low blood cell counts due to damaged bone marrow), hepatitis (liver inflammation), thrombocytopenia (low blood platelet count) and Guillain-Barré syndrome (damage to nerves that can result in pain, numbness, muscle weakness and difficulty walking).

Strimvelis must not be used in patients who have leukaemia (cancer of white blood cells) or myelodysplasia (a type of bone marrow disorder) or have had these conditions in the past. It must not be used in patients who have tested positive for human immunodeficiency virus (HIV, the virus that causes AIDS) or some other infections, or in patients who have previously had gene therapy treatment.

For the full list of restrictions and side effects reported with Strimvelis, see the package leaflet.

Why is Strimvelis approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that Strimvelis's benefits are greater than its risks and recommended that it be approved for use in the EU. Strimvelis provides the opportunity of a cure that improves the working of the immune system for patients with ADA-SCID, which is a life-threatening condition. Results from the main study show that Strimvelis is effective at improving survival of ADA-SCID patients. Regarding safety, Strimvelis was relatively well tolerated although data are limited due to the small number of patients studied. Because Strimvelis is produced using a retrovirus, there could be a potential risk of cancer caused by unintended changes in the genetic material, although no such cases have been seen so far. There is also a potential risk of autoimmune disease. However, measures are in place to monitor such events once the medicine is in use by using a registry of patients to study their long-term progress.

What measures are being taken to ensure the safe and effective use of Strimvelis?

A risk management plan has been developed to ensure that Strimvelis is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Strimvelis, including the appropriate precautions to be followed by healthcare professionals and patients.

In addition, the company that makes Strimvelis will provide educational materials for patients and healthcare professionals with information on the medicine, and patients will have to sign a consent form before treatment is started. The company will also maintain a registry of patients treated with Strimvelis and monitor their progress regularly after treatment to study the long-term safety of the medicine.

Other information about Strimvelis

The European Commission granted a marketing authorisation valid throughout the European Union for Strimvelis on 26 May 2016.

The full EPAR for Strimvelis can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Strimvelis, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Strimvelis can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 05-2016.