10 May 2021
EMADOC-628903358-3724

Public summary of opinion on orphan designation

Autologous CD4+ and CD8+ T cells genetically modified with a lentiviral vector encoding a B-cell maturation antigen-specific chimeric antigen receptor for the treatment of multiple myeloma

On 9 December 2020, orphan designation EU/3/20/2366 was granted by the European Commission to Celgene Europe B.V., Netherlands, for autologous CD4+ and CD8+ T cells genetically modified with a lentiviral vector encoding a B-cell maturation antigen-specific chimeric antigen receptor (also known as JCARH125) for the treatment of multiple myeloma.

Please note that this product was withdrawn from the Union Register of orphan medicinal products in March 2021 on request of the Sponsor.

What is multiple myeloma?

Multiple myeloma (also called plasma cell myeloma) is a cancer of a type of white blood cell called plasma cells. Plasma cells are produced in the bone marrow, the spongy tissue inside the large bones in the body. In multiple myeloma, the division of plasma cells becomes uncontrolled, resulting in abnormal, immature plasma cells multiplying and filling up the bone marrow. This interferes with production of normal white blood cells, red blood cells and platelets (components that help the blood to clot), leading to complications such as anaemia (low red blood cell counts), bone pain and fractures, raised blood calcium levels and kidney disease.

Multiple myeloma is a debilitating and life-threatening disease particularly because it disrupts the normal functioning of the bone marrow, damages the bones and causes kidney failure.

What is the estimated number of patients affected by the condition?

At the time of designation, multiple myeloma affected approximately 4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 208,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

*For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question via www.ema.europa.eu/contacts **Telephone** +31(0)88 781 6000 An agency of the European Union

What treatments are available?

At the time of designation, several medicines were authorised for multiple myeloma in the EU. The main treatment for multiple myeloma was chemotherapy (medicines to treat cancer) usually combined with corticosteroids to reduce the activity of the body's immune (defence) system. After chemotherapy patients received a stem-cell transplant if they were considered suitable for it. Stem-cell transplantation is a procedure where the patient's bone marrow is replaced with stem cells to form new bone marrow that produces healthy blood cells.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with multiple myeloma. This is because early results have shown that the medicine was beneficial in patients whose cancer had come back or not responded after extensive treatment; this compares favourably to other therapies used in this group of patients.

How is this medicine expected to work?

The medicine is made up of the patient's own T cells (a type of white blood cell) that have been modified genetically in the laboratory so that they make a protein called chimeric antigen receptor (CAR). This protein is designed to attach to a target called B-cell maturation antigen (BCMA), which is found on plasma cells. When the modified cells, called CAR-T cells, are given to the patient, they are expected to attach to BCMA on the plasma cells and kill the abnormal plasma cells, thereby helping to clear the cancer from the body.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with multiple myeloma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of multiple myeloma. Orphan designation of the medicine had been granted in the United States for multiple myeloma.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 5 November 2020, recommending the granting of this designation.

_____

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Contact details of the current sponsor for this orphan designation can be found on EMA website.

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.