



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Teclistamab for the treatment of multiple myeloma

On 19 October 2020, orphan designation EU/3/20/2331 was granted by the European Commission to Janssen-Cilag International N.V, Belgium, for teclistamab for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cell called plasma cells. Plasma cells originate from the bone marrow, the spongy tissue inside the large bones. 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 the 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).

What treatments are available?

At the time of designation, several medicines were already 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 immune system, the body's natural defences. Patients received a stem-cell transplant if they were considered suitable for it. In stem-cell transplantation the patient's bone marrow is replaced with stem cells to form new bone marrow that produces healthy blood cells.

*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).



The sponsor has provided sufficient information to show that the medicine might be of significant benefit to patients with multiple myeloma because early studies showed improvements in patients whose cancer had come back or did not respond to treatment with authorised medicines. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

This medicine is a monoclonal antibody, a type of protein that has been designed to attach to two targets at the same time:

- a protein called BCMA, which is present on the surface of the multiple myeloma cells;
- a protein called CD3, which is present on T cells, cells of the immune system responsible for destroying abnormal cells.

By attaching to BCMA and CD3 at the same time, the medicine is expected to activate the T cells to kill the multiple myeloma cells.

What is the stage of development of this medicine?

The effects of teclistamab have been evaluated in experimental models.

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

At the time of submission, teclistamab was not authorised anywhere in the EU for the treatment of multiple myeloma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 10 September 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.

Withdrawn