10 February 2021
EMADOC-628903358-3041

Public summary of opinion on orphan designation

Tipifarnib for the treatment of peripheral T-cell lymphoma

On 19 October 2020, orphan designation EU/3/20/2333 was granted by the European Commission to TMC Pharma (EU) Limited, Ireland, for tipifarnib for the treatment of peripheral T-cell lymphoma.

What is peripheral T-cell lymphoma?

Peripheral T-cell lymphoma is a cancer of the lymphatic system, a network of vessels that transport fluid from tissues through the lymph nodes and into the bloodstream. In peripheral T-cell lymphoma there is uncontrolled growth of T lymphocytes (T cells), a type of white blood cell found in the lymphatic system. Peripheral T-cell lymphomas include types that mainly occur in the lymph nodes (primary nodal) and types that occur mainly outside the lymph nodes (primary extranodal).

Symptoms of the disease vary according to the type of lymphoma, but the first sign may be a lump in the neck, under the arm or in the groin, which is caused by an enlarged lymph node. The lymphoma may also affect other organs in the body such as the bone marrow, liver, gastrointestinal tract and the skin.

Peripheral T-cell lymphoma is a long-term debilitating and life-threatening condition because in most cases the disease does not respond well to therapy, usually comes back within one year and is associated with early death.

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

At the time of designation, peripheral T-cell lymphoma affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 52,000 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, there were no specific treatments for peripheral T-cell lymphoma, and the disease was treated in the same way as the non-Hodgkin's lymphomas, for which several medicines

*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 Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

were authorised in the EU. The main treatment was chemotherapy (medicines to treat cancer), sometimes in combination with radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that this medicine might be of benefit for patients with peripheral T-cell lymphoma because early studies showed that the medicine can be of benefit in patients whose disease had not improved with previous treatments or had come back after treatment. 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?

Tipifarnib blocks the enzyme farnesyl transferase, but the way it works in T cell lymphoma is not fully understood. It is thought to reduce the production of a substance called CXCL12 by certain cells in the bone marrow. CXCL12 is involved in directing T cells to the tumour sites in the body. By reducing CXCL12, the medicine is expected to slow down growth of the cancer.

What is the stage of development of this medicine?

The effects of tipifarnib 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 peripheral T-cell lymphoma were ongoing.

At the time of submission, tipifarnib was not authorised anywhere in the EU for the treatment of peripheral T-cell lymphoma. Orphan designation of tipifarnib had been granted in the EU for acute myeloid leukaemia, and in the United States for acute myeloid leukaemia, multiple myeloma and T-cell lymphoma.

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.