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Public summary of opinion on orphan designation

Tazemetostat for the treatment of follicular lymphoma

On 21 March 2018, orphan designation (EU/3/18/2005) was granted by the European Commission to Quintiles Ireland Limited, Ireland, for tazemetostat for the treatment of follicular lymphoma.

In May 2018, Quintiles Ireland Limited changed name to IQVIA RDS Ireland Limited.

What is follicular lymphoma?

Follicular lymphoma is a cancer of a type of white blood cell called B lymphocytes or B cells. In follicular lymphoma, the B cells multiply quickly and live for too long, so there are too many of them in the lymph nodes. The first sign of the disease is usually a lump in the neck, under the arm or in the groin area, caused by an enlarged lymph node. Patients may also have fever, weight loss, tiredness and night sweats.

Follicular lymphoma is usually diagnosed in people aged over 50 years. It is a long-term debilitating and life-threatening disease due to organ damage and the cancer coming back.

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

At the time of designation, follicular lymphoma affected approximately 3.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 191,000 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, the main treatments for follicular lymphoma available in the EU included chemotherapy (medicines to treat cancer) combined with immunotherapy (medicines that stimulate the body's own immune system to kill the cancer cells). The medicines ibritumomab tiuxetan, idelalisib, interferon alfa-2b, obinutuzumab, pixantrone and rituximab were authorised for the treatment of follicular lymphoma.

*Disclaimer: 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 (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 517,400,000 (Eurostat 2018).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with follicular lymphoma because early results from studies in patients with the condition showed responses to treatment that compared well to those seen with authorised products in other studies. 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?

Tazemetostat blocks the action of a protein called EZH2. Some patients with follicular lymphoma have an altered form of the EZH2 protein, which encourages abnormal growth and development of B cells. By blocking the altered EZH2 protein, tazemetostat is expected to lead to the death of the lymphoma cells and thus stop the spread of the lymphoma.

What is the stage of development of this medicine?

The effects of tazemetostat have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with tazemetostat in patients with follicular lymphoma were ongoing.

At the time of submission, tazemetostat was not authorised anywhere in the EU for follicular lymphoma. Orphan designation of the medicine had been granted in the United States for follicular lymphoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 February 2018 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

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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.

Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Tazemetostat	Treatment of follicular lymphoma
Bulgarian	Таземетостат	Лечение на фоликуларен лимфом
Croatian	Tazemetostat	Liječenje folikularnog limfoma
Czech	Tazemetostat	Léčba folikulárního lymfomu
Danish	Tazemetostat	Behandling af follikulært lymfom
Dutch	Tazemetostat	Behandeling van folliculair lymfoom
Estonian	Tasemetostaat	Folikulaarse lümfoomi ravi
Finnish	Tatsetmetostaatti	Folikulaarisen lymfooman hoito
French	Tazemetostat	Traitement des lymphomes folliculaires
German	Tazemetostat	Behandlung des follikulären Lymphoms
Greek	Ταζεμετοστάτη	θεραπεία του θηλακιδώδους λεμφώματος
Hungarian	Tazemetostat	Follicularis lymphoma kezelése
Italian	Tazemetostat	Trattamento del linfoma follicolare
Latvian	Tazemetostats	Folikulārās limfomas ārstēšana
Lithuanian	Tazemetostatas	Folikulinės limfomos gydymas
Maltese	Tażemetostat	Kura tal-limfoma follikulari
Polish	Tazemetostat	Leczenie chłoniaków grudkowych
Portuguese	Tazemetostate	Tratamento do linfoma folicular
Romanian	Tazemetostat	Tratamentul limfomului folicular
Slovak	Tazemetostat	Liečba folikulárneho lymfómu
Slovenian	Tazemetostat	Zdravljenje folikularnega limfoma
Spanish	Tazemetostat	Tratamiento del linfoma folicular
Swedish	Tazemetostat	Behandling av follikulärt lymfom
Norwegian	Tazemetostat	Behandling av follikulært lymfom
Icelandic	Tazemetóstat	Meðferð á follicular eitilfrumukrabbameini

¹ At the time of designation