5 January 2018
EMA/COMP/632529/2017

Public summary of opinion on orphan designation

Glucopyranosyl lipid A stable emulsion for the treatment of follicular lymphoma

On 16 October 2017, orphan designation (EU/3/17/1924) was granted by the European Commission to Immune Design Ltd, United Kingdom, for glucopyranosyl lipid A stable emulsion (also known as G100) for the treatment of follicular lymphoma.

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 3.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 186,000 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 and rituximab were specifically 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 515,700,000 (Eurostat 2017).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with follicular lymphoma, with laboratory studies showing improved outcomes with this medicine, including when combined with radiotherapy. 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?

The medicine is expected to work by stimulating the patient's immune (defence) system, so that it targets and destroys the cancer cells. When injected directly into a tumour, this medicine is expected to attach to and activate a protein called Toll-like receptor 4, which in turn stimulates immune cells to attack cancer cells and thereby slow progression of the disease.

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 follicular lymphoma were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 September 2017 recommending the granting of this designation.

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Glucopyranosyl lipid A	Treatment of follicular lymphoma
Bulgarian	Глюкопиранозил липид А	Лечение на фоликуларен лимфом
Croatian	Glukopiranozil lipid A	Liječenje folikularnog limfoma
Czech	Glukopyranosylu A	Léčba folikulárního lymfomu
Danish	Glucopyranosyl lipid A	Behandling af follikulært lymfom
Dutch	Glucopyranosyl lipide A	Behandeling van folliculair lymfoom
Estonian	Glükopüranosüül lipiid A	Follikulaarse lümfoomi ravi
Finnish	Glukopyranosyyli lipidi A:n	Follikulaarisen lymfooman hoito
French	Lipide A glucopyranosylique	Traitement des lymphomes folliculaires
German	Glucopyranosyl Lipid A	Behandlung des follikulären Lymphoms
Greek	Γλυκοπυρανοσυλο λιπίδιο Α	θεραπεία του θηλακιώδους λεμφώματος
Hungarian	Glükopiranozil-lipid-A	Follicularis lymphoma kezelése
Italian	Glucopiranosile lipide A	Trattamento del linfoma follicolare
Latvian	Glikopiranozila A lipīds	Folikulārās limfomas ārstēšana
Lithuanian	Gliukopiranozilo lipido A	Folikulinės limfomos gydymas
Maltese	Glucopyranosyl lipid A	Kura tal-limfoma follikulari
Polish	Adjuwant glukopiranozylolipidowy	Leczenie chłoniaków grudkowych
Portuguese	Lípidos A glicopiranosil	Tratamento do linfoma folicular
Romanian	Glucopiranozil lipid A	Tratamentul limfomului folicular
Slovak	Glukopyranozyl lipid A	Liečba folikulárneho lymfómu
Slovenian	Glukopiranozil lipid A	Zdravljenje folikularnega limfoma
Spanish	Glucopiranosil lípido A	Tratamiento del linfoma folicular
Swedish	Glukopyranosyl lipid A	Behandling av follikulärt lymfom
Norwegian	Glukopyranosyl lipid A	Behandling av follikulært lymfom
Icelandic	Glúkópýranósýl lípíð-A	Meðferð á follicular eitilfrumukrabbameini

[1] At the time of designation