15 May 2018
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Public summary of opinion on orphan designation

Polatuzumab vedotin for the treatment of diffuse large B-cell lymphoma

On 16 April 2018, orphan designation (EU/3/18/2013) was granted by the European Commission to Roche Registration Limited, United Kingdom, for polatuzumab vedotin for the treatment of diffuse large B-cell lymphoma.

What is diffuse large B-cell lymphoma?

Diffuse large B-cell lymphoma is a type of blood cancer and the most common form of a group of blood cancers known as non-Hodgkin lymphomas.

Diffuse large B-cell lymphoma affects a type of white blood cell called B lymphocytes, or B cells. In patients with this cancer, 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, which is caused by an enlarged lymph node. Patients with diffuse large B-cell lymphoma may also have fever, tiredness, night sweats or weight loss that have no obvious cause.

Although some people with diffuse large B-cell lymphoma can be cured, it remains a serious and life-threatening disease, particularly when the disease is diagnosed late or has come back after treatment.

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

At the time of designation, diffuse large B-cell lymphoma affected approximately 4.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 222,000 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 authorised for the treatment of diffuse large B-cell lymphoma in the EU. The main treatment was chemotherapy (medicines to treat cancer) usually in combination with medicines called monoclonal antibodies and sometimes with radiotherapy (treatment with radiation). Autologous haematopoietic (blood) stem-cell transplantation was also used in patients

[*]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).

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at risk of the disease coming back after treatment. This procedure involves replacing the patient's bone marrow with the patient's own 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 diffuse large B-cell lymphoma because early results suggest that adding polatuzumab vedotin to existing treatments improved their effectiveness in patients whose disease had come back or in whom other treatment had not worked well enough. 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?

Polatuzumab vedotin is made up of a monoclonal antibody combined with a cytotoxic (cell-killing) substance called monomethyl auristatin E (MMAE). The monoclonal antibody is designed to attach to a protein called CD79b that B cells, including cancerous B cells, carry on their surface. When the antibody attaches to CD79b, the B cells absorb the medicine and MMAE is released inside them. MMAE stops microtubules in the B cells from working. Microtubules are structures that cells need to survive and divide. By targeting the B cells and causing them to die, the medicine is expected to reduce symptoms of the disease.

What is the stage of development of this medicine?

The effects of polatuzumab vedotin 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 diffuse large B-cell lymphoma were ongoing.

At the time of submission, polatuzumab vedotin was not authorised anywhere in the EU for diffuse large B-cell lymphoma. Orphan designation of the medicine had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 March 2018 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	Polatuzumab vedotin	Treatment of diffuse large B-cell lymphoma
Bulgarian	Полатузумаб ведотин	Лечение на дифузен В-едроклетъчен лимфом
Croatian	Polatuzumab vedotin	Liječenje difuznog limfoma velikih B-stanica
Czech	Polatuzumab vedotin	Léčba velkobuněčného difuzního B-lymfomu
Danish	Polatuzumab vedotin	Behandling af diffust storcellet B-celle lymfom
Dutch	Polatuzumab vedotin	Behandeling van diffuus grootcellig B-cel-lymfoom
Estonian	Polatuzumabvedotiin	Diffuusse suure β-rakulise lümfoomi ravi
Finnish	Polatutsumabi-vedotiini	Diffuusin suurisoluisen B-solulymfooman hoito
French	Polatuzumab vedotin	Traitement du lymphome diffus à grandes cellules B
German	Polatuzumab vedotin	Behandlung des diffusen großzelligen B-Zell-Lymphoms
Greek	Polatuzumab vedotin	Θεραπεία του διάχυτου μεγαλοκυτταρικού λεμφώματος Β-κυττάρου (DLBCL)
Hungarian	Polatuzumab vedotin	Diffúz nagy B-sejtes lymphoma kezelése
Italian	Polatuzumab vedotin	Terapia del Linfoma non-Hodgkin diffuso a grandi cellule di tipo B (DLBCL)
Latvian	Polatuzumaba vedotīns	Difūzas lielo B šūnu limfomas ārstēšana
Lithuanian	Polatuzumabas vedotinas	Difuzinės stambiųjų B ląstelių limfomos gydymas
Maltese	Polatuzumab vedotin	Kura tal-limfoma taċ-ċelluli tat-tip B kbar mxerrda
Polish	Polatuzumab vedotin	Leczenie rozlanego chłoniaka z dużych limfocytów B
Portuguese	Polatuzumab vedotina	Tratamento do linfoma difuso de grandes células B
Romanian	Polatuzumab vedotin	Tratamentul limfomului difuz cu celule B mari
Slovak	Polatuzumab vedotín	Liečba difúzneho veľkobunkového lymfómu z buniek B
Slovenian	Polatuzumab vedotin	Zdravljenje razširjenega limfoma velikih B celic
Spanish	Polatuzumab vedotin	Tratamiento del linfoma difuso de células B grandes
Swedish	Polatuzumab vedotin	Behandling av diffusa storcelliga B-cells lymfom
Norwegian	Polatuzumab vedotin	Behandling av diffust storcellet B-celle lymfom
Icelandic	Pólatúzúmavedótín	Til meðferðar á dreifðu stórfrumu B frumu eitlakrabbameini

[1] At the time of designation