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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human anti-interferon gamma monoclonal antibody for the treatment of haemophagocytic lymphohistiocytosis

On 9 June 2010, orphan designation (EU/3/10/749) was granted by the European Commission to NovImmune B.V., the Netherlands, for recombinant human anti-interferon gamma monoclonal antibody for the treatment of haemophagocytic lymphohistiocytosis.

What is haemophagocytic lymphohistiocytosis?

Haemophagocytic lymphohistiocytosis (HLH) is a disease in which the body produces too many histiocytes and lymphocytes (cells of the immune system), which accumulate in the body's tissues and organs, including the liver, spleen, bone marrow, brain and skin. This causes the symptoms of the disease, such as fever, an enlarged liver and spleen, skin rash, jaundice (yellowing of the skin and eyes) and pancytopenia (low blood cell counts).

HLH is a debilitating and life-threatening disease that is associated with poor long-term survival.

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

At the time of designation, HLH affected less than 0.1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 5,000 people, and is below the threshold 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, no satisfactory methods were authorised in the EU for the treatment of HLH. Patients were treated with immunosuppressant medicines (medicines that reduce the activity of the immune system) and chemotherapy (medicines to treat cancer). In some cases, bone marrow transplantation has cured the disease. This is a complex procedure where the bone marrow of the patient is destroyed and replaced with healthy bone marrow from a matched donor.

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,500,000 (Eurostat 2010).

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How is this medicine expected to work?

Recombinant human anti-interferon gamma monoclonal antibody is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a substance called interferon gamma. Patients with HLH have high levels of interferon gamma, which is thought to be responsible for the accumulation of the immune cells causing the disease. By attaching to interferon gamma, this medicine is expected to block its activity, improving the symptoms of HLH.

What is the stage of development of this medicine?

The effects of recombinant human anti-interferon gamma monoclonal antibody have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with recombinant human anti-interferon gamma monoclonal antibody in patients with HLH had been started.

At the time of submission, recombinant human anti-interferon gamma monoclonal antibody was not authorised anywhere in the EU for HLH or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 March 2010 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

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Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Recombinant human anti-interferon gamma monoclonal antibody	Treatment of haemophagocytic lymphohistiocytosis
Bulgarian	Рекомбинантно човешко моноклонално антитяло срещу интерферон гама	Лечение на хемофагоцитна лимфохистиоцитоза.
Czech	Rekombinantní monoklonální protilátka proti lidskému interferonu gama	Léčba hemofagocytové lymfohistiocytózy
Danish	Recombinant humant monoklonalt anti-interferon gamma antistof	Behandling af hæmofagocytisk lymfohistiocytose
Dutch	Recombinant humaan anti-interferon-gamma monoclonaal antilichaam	Behandeling van hemofagocytaire lymfohistiocytose
Estonian	Rekombinantne inimese anti-interferoon gamma monoklonaalne antikeha	Hemofagotsütaarse lümfohistiotsütoosi ravi
Finnish	Rekombinanttitekniikalla tehty ihmisen monoklonaalinen anti-gammainterferonivasta-aine	Hemofagosyyttisen lymfohistiosytoosin hoito
French	Anticorps monoclonal humain recombinant dirigé contre l'interféron gamma	Traitement de la lymphohistiocytose hémophagocytaire
German	Rekombinanter humaner monoklonaler Antikörper gegen Interferon gamma	Behandlung der hämophagozytäre lymphohistiozytose
Greek	Ανασυνδυασμένο ανθρώπινο μονοκλωνικό αντίσωμα έναντι της ιντερφερόνης γάμμα	Θεραπεία της αιμοφαγοκυτταρικής λεμφοϋστιοκυττάρωσης.
Hungarian	Rekombináns humán anti-interferon gamma monoklonális antitest	Haemophagocytás lymphohistiocytosis kezelése
Italian	Anticorpo monoclonale umano ricombinante anti-interferon gamma	Trattamento della linfoistiocitosi emofagocitica
Latvian	Rekombinēta cilvēka monoklonāla antiviela pret gamma interferonu	Hemofagocītiskas limfohistiocitozes ārstēšana
Lithuanian	Rekombinantinis žmogaus anti – interferono gama monokloninis antikūnas	Hemofagocitinės limfohistiocitozės gydymas
Maltese	Antikorp monoklonali uman rikombinanti kontra l-interferon gamma	Kura ta' limfoistjoċitosi emofagoċitika
Polish	Rekombinowane ludzkie przeciwciało monoklonalne przeciwko interferonowi gamma	Leczenie lymfohistiocytozy z erytrofagocytozą (HLH)
Portuguese	Anticorpo monoclonal recombinante humano anti- interferão gama	Tratamento de linfohistiocitose hemofagocítica
Romanian	Anticorp monoclonal uman recombinant anti-interferon gama	Tratamentul limfohistiocitozei hemofagocitice
Slovak	Rekombinantná ľudská monoklonálna protilátka proti interferónu gama	Liečba hemofagocytárnej lymfohistiocytózy

[1] At the time of designation

Language	Active Ingredient	Indication
Slovenian	Rekombinantno humano monoklonsko protitelo proti interferonu gama	Zdravljenje hemofagocitne limfohistiocitoze
Spanish	Anticuerpo monoclonal humano recombinante anti interferón gamma	Tratamiento de la linfohistiocitosis hemofagocítica
Swedish	Rekombinant human monoklonal antikropp mot interferon-gamma	Behandling av hemofagocyterande lymfohistiocytos
Norwegian	Rekombinant humant monoklonalt antistoff mot interferon gamma	Behandling av hemofagocytisk lymfohistiocytose
Icelandic	Raðbrigða anti-interferón gamma einstofna mótefni	Meðferð við eitil- og traffrumnageri með rauðkornaáti