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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Tadekinig alfa for the treatment of haemophagocytic lymphohistiocytosis

On 14 October 2016, orphan designation (EU/3/16/1763) was granted by the European Commission to Coté Orphan Consulting UK Limited, United Kingdom, for tadekinig alfa for the treatment of haemophagocytic lymphohistiocytosis.

What is haemophagocytic lymphohistiocytosis?

Haemophagocytic lymphohistiocytosis (HLH) is a disease in which the immune system (the body's natural defences) produces too many immune cells, which build up in tissues and organs, including the liver, spleen, bone marrow, brain and skin. This causes inflammation and damage and can lead to an enlarged liver and spleen with fever, rash, jaundice (yellowing of the skin and eyes), seizures (fits) and pancytopenia (low blood cell counts). HLH can be hereditary or develop after infections or cancers of the blood.

HLH is a severe and life-threatening disease mainly because of damage to the brain and pancytopenia. Most patients with the disease die within a few years.

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

At the time of designation, HLH affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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, 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, haematopoietic stem cell transplantation was used. This is a procedure where the patient's bone marrow is replaced by stem cells from a donor to form new bone marrow that produces healthy blood cells.

*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 513,700,000 (Eurostat 2016).

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How is this medicine expected to work?

The medicine is a copy of a natural occurring substance called 'interleukin-18-binding protein' (IL-18BP). IL-18BP blocks the activity of interleukin-18 (IL-18) which activates the immune system. Patients with HLH have increased levels of IL-18 and not sufficient IL-18BP to control immune activation. Giving the medicine to patients with HLH is expected to reduce the activity of IL-18 and control the symptoms 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, no clinical trials with the medicine in patients with HLH had started.

At the time of submission, the medicine 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 8 September 2016 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	Tadekinig alfa	Treatment of haemophagocytic lymphohistiocytosis
Bulgarian	Тадекиниг алфа	Лечение на хемофагоцитна лимфохистиоцитоза
Croatian	Tadekinig alfa	Liječenje hemofagocitne limfohistiocitoze
Czech	Tadekinig alfa	Léčba hemofagocytující lymfohistiocytózy
Danish	Tadekinig alfa	Behandling af hæmofagocytisk lymfohistiocytose
Dutch	Tadekinig alfa	Behandeling van hemofagocytaire lymfohistiocytose
Estonian	Tadekinig alfa	Hemofagotsütaarse lümfohistiotsütoosi ravi
Finnish	Tadekinigi alfa	Hemofagosyyttisen lymfohistiosytoosin hoito
French	Tadekinig alfa	Traitement de la lymphohistiocytose hémophagocytaire
German	Tadekinig alfa	Behandlung von hämophagozytischer Lymphohistiozytose
Greek	Ταδεκινίγη άλφα	Θεραπεία της αιμοφαγοκυτταρικής λεμφοϊστιοκυττάρωσης
Hungarian	Tadekinig-alfa	Hemofagocitikus limfohistiocitózis kezelésére
Italian	Tadekinig alfa	Trattamento della linfoistiocitosi emofagocitica
Latvian	Tadekinigs alfa	Hemofagocītiskas limfohistiocitozes ārstēšana
Lithuanian	Tadekinigas alfa	Hemofagocitinės limfohistiocitozės gydymas
Maltese	Tadekinig alfa	Kura ta' limfo-istjoċitosi emofagoċitika
Polish	Tadekinig alfa	Leczenie limfohistiocytozy hemofagocytarnej
Portuguese	Tadekinig alfa	Tratamento da linfohistiocitose hemofagocítica
Romanian	Tadekinig alfa	Tratamentul limfohistiocitozei hemofagocitare
Slovak	Tadekinig alfa	Liečba hemofagocytujúcej lymfohistiocytózy
Slovenian	Tadekinig alfa	Zdravljenje hemofagocitne limfohistiocitoze
Spanish	Tadekinig alfa	Tratamiento de la linfohistiocitosis hemofagocítica
Swedish	Tadekinig-alfa	Behandling av hemofagocyterande lymfohistiocytos
Norwegian	Tadekinig alfa	Behandling av hemofagocytisk lymfohistiocytose
Icelandic	Tadekínig alfa	Meðferð við eitil- og traffrumnageri með rauðkornaáti

[1] At the time of designation