8 March 2018
EMA/4237/2018

Public summary of opinion on orphan designation

Itacitinib for treatment of graft-versus-host disease

On 17 January 2018, orphan designation (EU/3/17/1964) was granted by the European Commission to Incyte Biosciences UK Ltd, United Kingdom, for itacitinib for the treatment of graft-versus-host disease.

What is graft-versus-host disease?

Graft-versus-host disease is a complication that can occur in patients who have had a transplant. In this disease, the transplanted cells recognise the patient's body as 'foreign' and attack the patient's organs, such as the stomach, gut, skin and liver, leading to organ damage. The disease may occur shortly after transplantation or later on, in which case a wider range of organs can be involved.

Graft-versus-host disease is a serious and life-threatening disease with a high mortality rate.

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

At the time of designation, graft-versus-host disease affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 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 in the European Union (EU) for the treatment of graft-versus-host disease, such as ciclosporin and corticosteroids. Treatment aimed to reduce the activity of transplanted cells involved in graft-versus-host disease, thereby reducing their ability to attack the patient's organs.

The sponsor has provided sufficient information to show that itacitinib might be of significant benefit for patients with graft-versus-host disease, with early studies showing an improved outcome when it is given together with corticosteroids. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

*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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How is this medicine expected to work?

Itacitinib blocks an enzyme known as JAK1, which plays an important role in the inflammation and tissue damage that occur in graft-versus-host disease. By blocking this enzyme, the medicine is expected to help reduce the tissue damage and thereby relieve symptoms of the condition.

What is the stage of development of this medicine?

The effects of itacitinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with itacitinib in patients with graft-versus-host disease were ongoing.

At the time of submission, itacitinib was not authorised anywhere in the EU for graft-versus-host disease. Orphan designation 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 December 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	Itacitinib	Treatment of graft-versus-host disease
Bulgarian	Итацитиниб	Лечение на болестта на присадката срещу приемателя
Croatian	Itacitinib	Liječenje reakcije presatka protiv primatelja
Czech	Itacitinib	Léčba reakce štěpu proti hostiteli
Danish	Itacitinib	Behandling af graft versus host reaktion
Dutch	Itacitinib	Behandeling van graft versus host ziekte
Estonian	Itatsitiniib	Graft versus host haiguse ravi
Finnish	Itasitinibi	Käänteishyljintäreaktion hoito
French	Itacitinib	Traitement de la réaction du greffon contre l'hôte
German	Itacitinib	Behandlung der Graft-versus-Host-Reaktion
Greek	Ιτασιτινίμπη	Θεραπεία της αντίδρασης του μοσχεύματος
Hungarian	Itacitinib	Graft-versus-host betegség kezelése
Italian	Itacitinib	Trattamento della reazione del trapianto contro l'ospite
Latvian	Itacitinibs	Saimnieka-transplantāta slimības ārstēšana
Lithuanian	Itacitinibas	Transplantato atmetimo ligos gydymas
Maltese	Itacitinib	Kura tal-marda tat-tessut għat-trapjant kontra dak li jirċievih
Polish	Itacytynib	Leczenie choroby przeszczep przeciw gospodarzowi
Portuguese	Itacitinib	Tratamento da reacção do enxerto contra o hospedeiro
Romanian	Itacitinib	Tratamentul reacţiei grefei contra gazdei
Slovak	Itacitinib	Liečba reakcie štepu proti hostiteľovi
Slovenian	Itacitinib	Zdravljenje bolezni presadka proti gostitelju
Spanish	Itacitinib	Tratamiento de la enfermedad de injerto contra huésped
Swedish	Itacitinib	Behandling av graft-värd host reaktion
Norwegian	Itacitinib	Behandling av graft-versus-host -reaksjon
Icelandic	Ítacítíníb	Til meðferðar á hýsilssótt

[1] At the time of designation