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Public summary of opinion on orphan designation

Sutimlimab for the treatment of immune thrombocytopenia

On 28 February 2020, orphan designation EU/3/20/2258 was granted by the European Commission to Celerion Austria GmbH, Austria, for sutimlimab for the treatment of immune thrombocytopenia. The sponsorship was transferred to Genzyme Europe B.V., the Netherlands, in April 2020.

What is immune thrombocytopenia?

Immune thrombocytopenia is a condition in which the immune system (the body's natural defences) attacks the platelets, components in the blood that help it to clot. As a result, blood levels of platelets are low (thrombocytopenia) resulting in spontaneous bleeding and bruising. Although not all patients experience bleeding, severe bleeding can occur in some patients.

Immune thrombocytopenia is a debilitating and life-threatening condition because of the risk of severe bleeding, especially in the brain.

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

At the time of designation, immune thrombocytopenia affected approximately 2.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 125,000 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, authorised treatments included human immunoglobulin, Nplate (romiplostim), Revolade (eltrombopag) and certain corticosteroids. Some patients required surgery (splenectomy) to remove the spleen, an organ involved in the filtering of blood which includes the removal of old platelets from the body.

The sponsor has provided sufficient information to show that sutimlimab might be of significant benefit for patients with immune thrombocytopenia. Early studies show that the medicine could lead to significant improvements in platelet levels in patients whose condition had not responded to at least

*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 519,200,000 (Eurostat 2020).

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two authorised treatments. 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?

In patients with immune thrombocytopenia, components of the immune system called IgG antibodies are faulty and attack platelets.

This medicine is a monoclonal antibody (a type of protein) that has been designed to attach to and block a protein called C1 which is involved in setting off an immune response. By blocking C1, the medicine is expected to reduce the damage to platelets, increase their levels and thus improve symptoms of the condition.

What is the stage of development of this medicine?

The effects of sutimlimab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with sutimlimab in patients with immune thrombocytopenia were ongoing.

At the time of submission, sutimlimab was not authorised anywhere in the EU for the treatment of immune thrombocytopenia. It was designated as an orphan medicinal product in the United States for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 22 January 2020, 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

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website.

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	Sutimlimab	Treatment of immune thrombocytopenia
Bulgarian	Сутимлимав	Лечение на имунна тромбоцитопения
Croatian	Sutimlimab	Liječenje imune trombocitopenije
Czech	Sutimlimab	Léčba imunitní trombocytopenie
Danish	Sutimlimab	Behandling af immun trombocytopeni
Dutch	Sutimlimab	Behandeling van immune trombocytopenie
Estonian	Sutimlimab	Immuungeneesiga trombotsütopeenia ravi
Finnish	Sutimlimab	Immuunitrombosytopenian hoito
French	Sutimlimab	Traitement de la thrombopénie immunitaire
German	Sutimlimab	Behandlung von Immunthrombozytopenie
Greek	Σουτιμλιμάμπη	Θεραπεία της ανοσολογικής θρομβοκυτταροπενίας
Hungarian	Sutimlimab	Immun trombocitopénia kezelése
Italian	Sutimlimab	Trattamento della trombocitopenia immune
Latvian	Sutimlimabs	Imūnās trombocitopēnijas ārstēšana
Lithuanian	Sutimlimabas	Imuninės trombocitopenijos gydymas
Maltese	Sutimlimab	Kura tat-trombożitopenja immuna
Polish	Sutimlimab	Leczenie małopłytkowości immunologicznej
Portuguese	Sutimlimab	Tratamento de trombocitopenia imune
Romanian	Sutimlimab	Tratamentul trombocitopeniei imune
Slovak	Sutimlimab	Liečba imunitnej trombocytopénie
Slovenian	Sutimlimab	Zdravljenje imunske trombocitopenije
Spanish	Sutimlimab	Tratamiento de la trombocitopenia inmune
Swedish	Sutimlimab	Behandling av immuntrombocytopeni
Norwegian	Sutimlimab	Behandling av immun trombocytopeni
Icelandic	Sutimlimab	Meðferð ónæmistegdrii blóðflagnafæð

[1] At the time of designation