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Public summary of opinion on orphan designation

Efgartigimod alfa for the treatment of immune thrombocytopenia

On 16 December 2019, orphan designation EU/3/19/2230 was granted by the European Commission to Argenx B.V.B.A., Belgium, for efgartigimod alfa for the treatment of immune thrombocytopenia.

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 1.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 93,000 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 removal of platelets from the body.

The sponsor has provided sufficient information to show that efgartigimod alfa 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 authorised

*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 518,400,000 (Eurostat 2019).

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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. IgG antibodies are long-lasting because they are recycled and kept in the body after attaching to a protein in cells called FcRn.

This medicine blocks FcRn and prevents IgG antibodies from attaching in this way. This allows the damaging IgGs to be broken down and removed from the body much more quickly, which is expected to increase the levels of platelets in the blood and thus improve symptoms of the condition.

What is the stage of development of this medicine?

The effects of efgartigimod alfa have been evaluated in experimental models.

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

At the time of submission, efgartigimod alfa was not authorised anywhere in the EU for immune thrombocytopenia. Orphan designation had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 7 November 2019, 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	Efgartigimod alfa	Treatment of immune thrombocytopenia
Bulgarian	Ефгартигимод алфа	Лечение на имунна тромбоцитопения
Croatian	Efgartigimod alfa	Lječenje imune trombocitopenije
Czech	Efgartigimod alfa	Léčba imunitní trombocytopenie
Danish	Efgartigimod alfa	Behandling af immun trombocytopeni
Dutch	Efgartigimod alfa	Behandeling van immune trombocytopenie
Estonian	Alfaefgartigimood	Immuungeneesiga trombotsütopeenia ravi
Finnish	Efgartigimodi alfa	Immuunitrombosytopenian hoito
French	Efgartigimod alfa	Traitement de la thrombopénie immunitaire
German	Efgartigimod alfa	Behandlung von Immunthrombozytopenie
Greek	Εφγαρτιγιμόδη άλφα	Θεραπεία της ανοσολογικής θρομβοκυτταροπενίας
Hungarian	Efgartigimod alfa	Immun trombocitopénia kezelése
Italian	Efgartigimod alfa	Trattamento della trombocitopenia immune
Latvian	Alfa efgartigimods	Imūnās trombocitopēnijas ārstēšana
Lithuanian	Efgartigimodas alfa	Imuninės trombocitopenijos gydymas
Maltese	Efgartigimod alfa	Kura tat-trombożitopenja immuna
Polish	Efgartigimod alfa	Leczenie małopłytkowości immunologicznej
Portuguese	Efgartigimod alfa	Tratamento de trombocitopenia imune
Romanian	Efgartigimod alfa	Tratamentul trombocitopeniei imune
Slovak	Efgartigimod alfa	Liečba imunitnej trombocytopénie
Slovenian	Efgartigimod alfa	Zdravljenje imunske trombocitopenije
Spanish	Efgartigimod alfa	Tratamiento de la trombocitopenia inmune
Swedish	Efgartigimod alfa	Behandling av immuntrombocytopeni
Norwegian	Efgartigimod alfa	Behandling av immun trombocytopeni
Icelandic	Efgartigimód alfa	Meðferð ónæmistegdrii blóðflagnafæð

[1] At the time of designation