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Public summary of opinion on orphan designation

Rozanolixizumab for the treatment of immune thrombocytopenia

On 11 January 2019, orphan designation (EU/3/18/2131) was granted by the European Commission to UCB Biopharma S.P.R.L., Belgium, for rozanolixizumab 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?

At the time of designation, immune thrombocytopenia affected approximately 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 135,000 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 normal 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 rozanolixizumab 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 is difficult to treat. This

*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 517,400,000 (Eurostat 2018).

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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 damage platelets. Rozanolixizumab is expected to remove the faulty IgG antibodies from the body by blocking a protein called FcRn on the surface of cells, which keeps these antibodies in the body for longer. By blocking FcRn, the medicine 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 rozanolixizumab have been evaluated in experimental models.

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

At the time of submission, rozanolixizumab was not authorised anywhere in the EU for 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 of 16 December 1999, the COMP adopted a positive opinion on 6 December 2018 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 the 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 orphan condition in all official EU languages[1], Norwegian and Icelandic

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

[1] At the time of designation