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Public summary of opinion on orphan designation

Rilzabrutinib for the treatment of immune thrombocytopenia

On 4 June 2020, orphan designation EU/3/20/2278 was granted by the European Commission to Clinical Network Services (NL) B.V., Netherlands, for rilzabrutinib (also known as PRN1008) 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 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 less than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 156,000 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), Tavlesse (fostamatinib) 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 the medicine might be of significant benefit for patients with immune thrombocytopenia. This is because in early studies patients for whom other treatments had not worked had improvements in platelet levels after treatment with the

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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medicine. 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?

Rilzabrutinib binds to and blocks the activity of an enzyme called Bruton's agammaglobulinemia tyrosine kinase (Btk). This enzyme is involved in activating the immune system, leading to damage to platelets. By blocking Btk, the medicine is expected to reduce platelet damage caused by the immune system and improve symptoms of the condition.

What is the stage of development of this medicine?

The effects of rilzabrutinib have been evaluated in experimental models.

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

At the time of submission, rilzabrutinib was not authorised anywhere in the EU for the treatment of immune thrombocytopenia. Orphan designation of rilzabrutinib 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 23 April 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	Rilzabrutinib	Treatment of immune thrombocytopenia
Bulgarian	Рилзабрутиниб	Лечение на имунна тромбоцитопения
Croatian	Rilzabrutinib	Liječenje imune trombocitopenije
Czech	Rilzabrutinib	Léčba imunitní trombocytopenie
Danish	Rilzabrutinib	Behandling af immun trombocytopeni
Dutch	Rilzabrutinib	Behandeling van immune trombocytopenie
Estonian	Rilzabrutiniib	Immuungeneesiga trombotsütopeenia ravi
Finnish	Riltsabrutinibi	Immuunitrombosytopenian hoito
French	Rilzabrutinib	Traitement de la thrombopénie immunitaire
German	Rilzabrutinib	Behandlung von Immunthrombozytopenie
Greek	Ριλζαμπρουτινίμπη	Θεραπεία της ανοσολογικής θρομβοκυτταροπενίας
Hungarian	Rilzabrutinib	Immun trombocitopénia kezelése
Italian	Rilzabrutinib	Trattamento della trombocitopenia immune
Latvian	Rilzabrutinibs	Imūnās trombocitopēnijas ārstēšana
Lithuanian	Rilzabrutinibas	Imuninės trombocitopenijos gydymas
Maltese	Rilzabrutinib	Kura tat-trombocitopenja immuna
Polish	Rilzabrutinib	Leczenie małopłytkowości immunologicznej
Portuguese	Rilzabrutinib	Tratamento de trombocitopenia imune
Romanian	Rilzabrutinib	Tratamentul trombocitopeniei imune
Slovak	Rilzabrutinib	Liečba imunitnej trombocytopénie
Slovenian	Rilzabrutinib	Zdravljenje imunske trombocitopenije
Spanish	Rilzabrutinib	Tratamiento de la trombocitopenia inmune
Swedish	Rilzabrutinib	Behandling av immuntrombocytopeni
Norwegian	Rilzabrutinib	Behandling av immun trombocytopeni
Icelandic	Rilzabrutinib	Meðferð við ónæmistengdri blóðflagnafæð

[1] At the time of designation