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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human pentraxin-2 for the treatment of post-essential thrombocythaemia myelofibrosis

On 19 November 2014, orphan designation (EU/3/14/1358) was granted by the European Commission to FGK Representative Service GmbH, Germany, for recombinant human pentraxin-2 for the treatment of post-essential thrombocythaemia myelofibrosis.

What is post-essential thrombocythaemia myelofibrosis?

Myelofibrosis is a disease in which the bone marrow (the spongy tissue inside the large bones) becomes dense and fibrous, and starts producing abnormal immature blood cells that replace the normal blood cells. It can develop following thrombocythaemia (overproduction of platelets, components that help the blood to clot). When the thrombocythaemia is not caused by any known condition, the disease is known as post-essential thrombocythaemia myelofibrosis.

In myelofibrosis, some immature blood cells migrate from the bone marrow to other organs, such as the spleen and liver, where they mature. This causes the organs to become enlarged. Patients with myelofibrosis can develop several symptoms, including bone pain, fever, tiredness, weakness, weight loss, fever and bleeding.

Post-essential thrombocythaemia myelofibrosis is a debilitating disease that is long-lasting and life-threatening because it can lead to severe anaemia (low red blood cell counts) and infections, and can lead to leukaemia (cancer of the white blood cells).

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

At the time of designation, post-essential thrombocythaemia myelofibrosis affected less than 0.15 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 8,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the

*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 511,100,000 (Eurostat 2014).

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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, ruxolitinib, hydroxycarbamide and busulfan were authorised in the EU for myelofibrosis. In addition, medicines were authorised to treat the symptoms, including erythropoietin (a hormone that stimulates the production of red blood cells) to treat anaemia, and surgery to remove the enlarged spleen. In some patients, haematopoietic (blood) stem-cell transplantation was used to treat the disease. This is a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow.

The sponsor has provided sufficient information to show that recombinant human pentraxin-2 might be of significant benefit for patients with post-essential thrombocythaemia myelofibrosis because early studies have shown beneficial effects in patients affected by the condition, including those that were previously given other 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?

This medicine contains pentraxin-2, a protein that activates certain cells of the immune system (the body's natural defences) that are involved in breaking down and removing 'debris' material from the body. Pentraxin-2 is expected to help these immune cells recognise as debris the fibrotic tissue in the bone marrow of patients with post-essential thrombocythaemia myelofibrosis. It is thought to do so by attaching to fibrotic tissue so that it can be recognised by immune cells as debris. Pentraxin-2 also stimulates the development of certain macrophages (a type of immune cells) that promote healing of fibrotic tissue, thereby restoring the function of the bone marrow.

The medicine is made by a method known as 'recombinant DNA technology': it is made by cells into which a gene (DNA) has been introduced that makes them able to produce pentraxin-2.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with <the medicine> in patients with post-essential thrombocythaemia myelofibrosis were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for post-essential thrombocythaemia myelofibrosis. Orphan designation of the medicine had been granted in the United States for myelofibrosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 October 2014 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:

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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	Recombinant human pentraxin-2	Treatment of post-essential thrombocythaemia myelofibrosis
Bulgarian	Рекомбинантен човешки пентраксин-2	Лечение на миелофиброза след есенциална тромбоцитемия
Croatian	Rekombinantni ljudski pentraksin-2	Liječenje mijelofibroze nakon esencijalne trombocitemije
Czech	Rekombinantní humánní pentraxin-2	Léčba post-esenciální trombocytémické myelofibrózy
Danish	Rekombinant human pentraxin-2	Behandling af post essentiel thrombocythæmi myelofibrose
Dutch	Recombinant humaan pentraxine-2	Behandeling van myelofibrosis volgend op essentiële trombocytemie
Estonian	Rekombinantne inimese pentraksiin-2	Postessentsiaalse trombotsüteemia müelofibroosi ravi
Finnish	Geeniteknisesti tuotettu ihmisen pentraksiini-2	Essentiaalisen trombosytemian jälkeisen myelofibroosin hoito
French	Pentraxine-2 humaine recombinante	Traitement de la myélofibrose consécutive à une thrombocytémie essentielle
German	Rekombinantes humanes Pentraxin-2	Behandlung einer Myelofibrose nach essentieller Thrombozythämie
Greek	Ανασυνδυασμένη ανθρώπινη πεντραξίνη-2	Θεραπεία της μυελοϊνωσης από ιδιοπαθή θρομβοκυττάρωση
Hungarian	Rekombináns humán pentraxin-2	Esszenciális thrombocytaemiát követő mielofibrózis kezelésére
Italian	Pentraxina-2 ricombinante umana	Trattamento della mielofibrosi post-trombocitemia essenziale
Latvian	Rekombinantais cilvēka pentraksīns 2	Pēc-esenciālas trombocitēmijas mielofibrozes ārstēšana
Lithuanian	Rekombinantinis žmogaus pentraksinas-2	Mielofibrozės gydymas po esencialinės trombocitemijos
Maltese	Pentraxin-2 rikombinanti uman	Kura tal-mjelofibroži konsegwenti għal trombocitemija essenzjali
Polish	Rekombinowana ludzka pentraksyna 2	Leczenie mielofibrozy wywołanej nadpłytkowością samoistną
Portuguese	Pentraxina-2 humana recombinante	Tratamento da mielofibrose devida a trombocitémia essencial
Romanian	Pentraxina-2 umană recombinantă	Tratamentul mielofibrozei post-trombocitemie esenţială
Slovak	Rekombinantný ľudský pentraxín-2	Liečba myelofibrózy po esenciálnej trombocytémii

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Rekombinantni humani pentraksin-2	Zdravljenje mielofibroze, nastale po esecialni trombocitemiji
Spanish	Pentraxina-2 humana recombinante	Tratamiento de la mielofibrosis secundaria a trombocitemia esencial
Swedish	Rekombinant humant pentraxin-2	Behandling av post-essentiell trombocytemi myelofibros
Norwegian	Rekombinant humant pentraksin-2	Behandling av myelofibrose sekundært til essensiell trombocytemi
Icelandic	Raðbrigða manna pentraxín-2	Meðferð á mýelófíbrósu í kjölfar eðlislægs blóðflagnadreyra