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EMA/COMP/660445/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human pentraxin-2 for the treatment of primary myelofibrosis

On 19 November 2014, orphan designation (EU/3/14/1366) was granted by the European Commission to FGK Representative Service GmbH, Germany, for recombinant human pentraxin-2 for the treatment of primary myelofibrosis.

What is primary myelofibrosis?

Primary myelofibrosis is a disease of unknown cause 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. In this disease, 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 primary myelofibrosis can develop several symptoms, including bone pain, tiredness, weakness, fever and bleeding.

Primary myelofibrosis is a debilitating disease that is long lasting and life threatening because it results in 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, primary myelofibrosis affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 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, ruxolitinib, hydroxycarbamide and busulfan were authorised in the EU for primary 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

*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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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 primary 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 primary 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 primary myelofibrosis were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for primary 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.

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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 primary myelofibrosis
Bulgarian	Рекомбинантен човешки пентраксин-2	Лечение на първична миелофиброза
Croatian	Rekombinantni ljudski pentraksin-2	Liječenje primarne mijelofibroze
Czech	Rekombinantní humánní pentraxin-2	Léčba primární myelofibrózy
Danish	Rekombinant human pentraxin-2	Behandling af primær myelofibrose
Dutch	Recombinant humaan pentraxine-2	Behandeling van primaire myelofibrose
Estonian	Rekombinantne inimese pentraksiin-2	Esmase müelofibroosi ravi
Finnish	Geeniteknisesti tuotettu ihmisen pentraksiini-2	Primaarisen myelofibroosin hoito
French	Pentraxine-2 humaine recombinante	Traitement de la myélofibrose primitive
German	Rekombinantes humanes Pentraxin-2	Behandlung der primären Myelofibrose
Greek	Ανασυνδυασμένη ανθρώπινη πεντραξίνη-2	Θεραπεία της πρωτογενούς μυελοσκλήρυνσης
Hungarian	Rekombináns humán pentraxin-2	Primer mielofibrózis kezelésére
Italian	Pentraxina-2 ricombinante umana	Trattamento della mielofibrosi primitiva
Latvian	Rekombinantais cilvēka pentraksīns 2	Primāras mielofibrozes ārstēšana
Lithuanian	Rekombinantinis žmogaus pentraksinas-2	Pirminės mielofibrozės gydymas
Maltese	Pentraxin-2 rikombinanti uman	Kura tal-mjelofibrożi primarja
Polish	Rekombinowana ludzka pentraksyna 2	Leczenie mielofibrozy pierwotnej
Portuguese	Pentraxina-2 humana recombinante	Tratamento da mielofibrose primária
Romanian	Pentraxina-2 umană recombinantă	Tratamentul mielofibrozei primitive
Slovak	Rekombinantný ľudský pentraxín-2	Liečba primárnej myelofibrózy
Slovenian	Rekombinantni humani pentraksin-2	Zdravljenje primarne mielofibroze
Spanish	Pentraxina-2 humana recombinante	Tratamiento de la mielofibrosis primaria
Swedish	Rekombinant humant pentraxin-2	Behandling av primär myelofibros
Norwegian	Rekombinant humant pentraksin-2	Behandling av primær myelofibrose
Icelandic	Raðbrigða manna pentraxín-2	Meðferð á beinmergsnetjuhersli

[1] At the time of designation