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Public summary of opinion on orphan designation

Recombinant human placental growth factor for the treatment of pre-eclampsia

On 27 June 2018, orphan designation (EU/3/18/2040) was granted by the European Commission to IQVIA RDS Ireland Limited, Ireland, for recombinant human placental growth factor (also known as AG31) for the treatment of pre-eclampsia.

What is pre-eclampsia?

Pre-eclampsia is a complication of pregnancy whose main features include sustained high blood pressure and abnormally high amounts of protein in the urine.

Pre-eclampsia usually begins after 20 weeks of pregnancy. Pregnant women with the condition may have swelling of the feet, hands and face, severe headache and vision problems, as well as signs of damage to organs such as liver and kidneys.

Pre-eclampsia can lead to a more serious condition called eclampsia, in which the woman has seizures (fits). The exact cause of pre-eclampsia is unknown. The condition normally resolves when the baby is born.

Pre-eclampsia is a life-threatening condition due to its complications, such as seizures, damage to organs such as the kidney and cerebral haemorrhage (bleeding in the brain). Pre-eclampsia also carries risks for the baby who tends to be smaller and have a higher risk of prematurity and death.

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

At the time of designation, pre-eclampsia affected approximately 4.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 233,000 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).

*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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What treatments are available?

At the time of designation, several medicines were used to treat symptoms of pre-eclampsia, including medicines to lower blood pressure and medicines to prevent seizures. However, the only cure for pre-eclampsia is the delivery of the baby.

The sponsor has provided sufficient information from laboratory studies to show that this medicine can lead to an improvement in kidney function, which is currently not addressed by authorised 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?

During pregnancy, a protein called 'placental growth factor' (PlGF) is produced, which plays an important role for the growth of blood vessels in the placenta and for the baby's development. In patients with pre-eclampsia, blood levels of PlGF are lower than normal and the lower they are, the more severe is the condition.

This medicine is a copy of PlGF. Injecting it into the patient's vein is expected to restore blood PlGF levels and thereby improve the symptoms of pre-eclampsia.

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, no clinical trials with the medicine in patients with pre-eclampsia had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for pre-eclampsia. Orphan designation of the medicine had been granted in the United States for the treatment of severe pre-eclampsia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 24 May 2018 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Activeingredient	Indication
English	Recombinant human placental growth factor	Treatment of pre-eclampsia
Bulgarian	Рекомбинантен човешки плацентарен растежен фактор	Лечение на преeклампсия
Croatian	Rekombinantni čimbenik rasta ljudske placente	Liječenje preeklampsije
Czech	Rekombinantní lidský placentární růstový faktor	Léčba preeklampsie
Danish	Rekombinant human placenta vækstfaktor	Behandling af præeklampsi
Dutch	Recombinante menselijke placentale groeifactor	Behandeling van pre-eclampsie
Estonian	Inimese rekombinantne platsentaarne kasvufaktor	Preeklampsia ravi
Finnish	Rekombinantti ihmisen istukan kasvutekijä	Pre-eclampsian hoito
French	Facteur de croissance placentaire humain recombinant	Traitement de la pré-éclampsie
German	Rekombinanter humaner Plazenta-Wachstumsfaktor	Behandlung der Präeklampsie
Greek	Ανασυνδυασμένος παράγοντας ανάπτυξης του ανθρώπινου πλακούντα	θεραπεία της προεκλαμψίας
Hungarian	Rekombináns humán placenta növekedési faktor	Pre-eclampsia kezelése
Italian	Fattore di crescita placentare umano ricombinante	Trattamento della preeclampsia
Latvian	Rekombinants cilvēka placentas augšanas faktors	Preeklampsijas ārstēšana
Lithuanian	Rekombinantinis žmogaus placentos augimo faktorius	Preeklampsijos gydymas
Maltese	Fattur rikombinanti tat-tkabbir tal-plaċenta tal-bniedem	Kura tal-pre-eklampsja
Polish	Rekombinowany ludzki łożyskowy czynnik wzrostu	Leczenie stanu przedrzucawkowego
Portuguese	Fator de crescimento placentário humano recombinante	Tratamento da pré-eclâmpsia
Romanian	Factor de creștere placentară uman recombinant	Tratamentul preeclampsiei
Slovak	Rekombinantný ľudský placentárny rastový faktor	Liečba preeklampsie
Slovenian	Rekombinantni placentarni faktor rasti	Zdravljenje preeklampsije
Spanish	Factor de crecimiento placentario humano recombinante	Tratamiento de la preeclampsia
Swedish	Rekombinant human placentatillväxtfaktor	Behandling av preeklampsi
Norwegian	Rekombinant human morkake vekstfaktor	Behandling av svangerskapsforgiftning
Icelandic	Mannavaxtarþáttur fylgju framleiddur með raðbrigðaerfðatækni	Meðferð meðgöngueitrunar

[1] At the time of designation