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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Aganirsen for the treatment of central retinal vein occlusion

On 10 June 2014, orphan designation (EU/03/14/1275) was granted by the European Commission to Gene Signal SAS, France, for aganirsen for the treatment of central retinal vein occlusion.

What is central retinal vein occlusion?

Central retinal vein occlusion is blockage of the main vein carrying blood from the retina (the light sensitive membrane at the back of the eye). This means that blood cannot easily drain away from the retina, leading to a build-up of pressure in the retinal vein. As a result, fluid and blood start to leak from this vein, causing swelling and damage to the retina.

Central retinal vein occlusion affects men and women equally and is more common in older people. The disease is long-term debilitating because it causes loss of vision and can lead to blindness.

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

At the time of designation, central retinal vein occlusion affected approximately 2.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 143,000 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, the medicines aflibercept, dexamethasone and ranibizumab were authorised in the EU for the treatment of macular oedema (swelling of the macula) following central retinal vein occlusion. Laser photocoagulation (where a laser is used to seal or destroy abnormal, leaking blood vessels in the retina) was also used.

The sponsor has provided sufficient information to show that aganirsen might be of significant benefit for patients with central retinal vein occlusion because early studies in experimental models show that it might improve the outcome of patients when used in combination with existing treatments. 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 511,100,000 (Eurostat 2014).

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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?

Aganirsen is an 'antisense oligonucleotide', a very short fragment of DNA. It has been designed to attach to the genetic material of cells responsible for producing a protein called IRS-1, blocking its production. IRS-1 has been reported to play an important role in angiogenesis (formation of new blood vessels), particularly in the retina. As central retinal vein occlusion is associated with the abnormal formation of new blood vessels in the retina, by blocking the production of IRS-1, aganirsen is expected to reduce the abnormal growth of the blood vessels in central retinal vein occlusion, thereby improving the symptoms of the condition.

What is the stage of development of this medicine?

The effects of aganirsen have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with aganirsen in patients with central retinal vein occlusion had been started.

At the time of submission, aganirsen was not authorised anywhere in the EU for central retinal vein occlusion or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 April 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	Aganirsen	Treatment of central retinal vein occlusion
Bulgarian	Аганирсен	Лечение на оклузия на централната ретинална вена
Croatian	Aganirsen	Liječenje okluzije centralne retinalne vene
Czech	Aganirsen	Léčba centrální okluze ertinální žíly
Danish	Aganirsen	Behandling af retinal centralvene okklusion
Dutch	Aganirsen	Behandeling van centraal retinale veneuze occlusie
Estonian	Aganirsen	Reetina tsentraalveeni oklusiooni ravi
Finnish	Aganirseeni	Verkkokalvon keskuslaskimon tukoksen hoito
French	Aganirsen	Traitement de l'occlusion de la veine centrale rétinienne
German	Aganirsen	Behandlung des zentralen Venenverschluss der Retina
Greek	Αγκανιρσένη	Θεραπεία της απόφραξης της κεντρικής φλέβας του αμφιβληστροειδούς
Hungarian	Aganirzen	Vena centrális retinae elzáródás kezelése
Italian	Aganirsen	Trattamento dell'occlusione della vena centrale della retina
Latvian	Aganirzēns	Centrālās retinas vēnas oklūzijas ārstēsana
Lithuanian	Aganirsenas	Centrinės tinklainės venos okliuzijos gydymas
Maltese	Aganirsen	Kura ta' sadd tal-vina ċentrali tar-retina
Polish	Aganirsen	Leczenie niedrożności żyły środkowej siatkówki
Portuguese	Aganirsen	Tratamento da oclusão da veia central da retina
Romanian	Aganirsen	Tratamentul ocluziei venei centrale a retinei
Slovak	Aganirsen	Liečba oklúzie centrálnej žily sietnice
Slovenian	Aganirsen	Zdravljenje okluzije centralne retinalne vene
Spanish	Aganirsen	Tratamiento de la oclusion de la vena central de la retina
Swedish	Aganirsen	Behandling av retinal centralvenocklusion
Norwegian	Aganirsen	Behandling av sentral retinal vene-okklusjon
Icelandic	Aganirsen	Meðferð á lokun aðal sjónhimnubláæðar

[1] At the time of designation