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Public summary of opinion on orphan designation

Humanised IgG1 monoclonal antibody against human eotaxin-2 for the treatment of primary sclerosing cholangitis

On 21 August 2020, orphan designation EU/3/20/2314 was granted by the European Commission to Granzer Regulatory Consulting & Services, Germany, for humanised IgG1 monoclonal antibody against human eotaxin-2 (also known as CM-101) for the treatment of primary sclerosing cholangitis.

What is primary sclerosing cholangitis?

Primary sclerosing cholangitis is a disease in which there is long-term damage to the small bile ducts in the liver. These ducts transport fluid called bile from the liver towards the intestines, where it is used to help digest fats. Because of the damage to the ducts, bile acids, essential components of bile, build up in the liver causing inflammation and damage to liver tissue and leading to liver cirrhosis (scarring of the liver). Early symptoms of the disease include tiredness and itching. The disease is more common in middle-aged men.

Primary sclerosing cholangitis is a long-term debilitating and life-threatening disease because, when the disease progresses, it may lead to liver cirrhosis and liver failure, and may increase the risk of liver cancer.

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

At the time of designation, primary sclerosing cholangitis affected approximately 2.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 130,000 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, ursodeoxycholic acid was authorised in some EU countries for the treatment of primary sclerosing cholangitis. In advanced cases, the patient may need a liver transplant.

*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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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with primary sclerosing cholangitis because it works in a different way to existing treatments and laboratory results suggest that unlike them it could reduce inflammation and scarring of the liver.

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?

The medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a protein called eotaxin-2 (also called chemokine CCL24) which is involved in the inflammation process and the formation of fibrous tissue. By blocking eotaxin-2, this medicine is expected to reduce inflammation and scarring of the liver, thereby relieving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of Humanised IgG1 monoclonal antibody against human eotaxin-2 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 primary sclerosing cholangitis had started.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of primary sclerosing cholangitis. Orphan designation of the medicine had been granted in the United Sates for the condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 16 July 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

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	Humanised IgG1 monoclonal antibody against human eotaxin-2	Treatment of primary sclerosing cholangitis
Bulgarian	Хуманизирано IgG1 моноклонално антитяло срещу човешки еотаксин-2	Лечение на първичен склерозиращ холангит
Croatian	Humanizirano IgG1 monoklonsko protutijelo protiv ljudskog eotaksina-2	Liječenje primarnog sklerozirajućeg kolangitisa
Czech	Humanizovaná IgG1 monoklonální protilátka ki lidskému eotaxinu-2	Léčba primární sklerotizující cholangoitidy
Danish	Humaniseret IgG1 monoklonalt antistof mod humant eotaxin-2	Behandling af primær skleroserende cholangitis
Dutch	Gehumaniseerd IgG1 monoklonaal antilichaam tegen humaan eotaxine-2	Behandeling van primaire scleroserende cholangitis
Estonian	Humaniseeritud IgG1 monoklonaalne antikeha inimese eotaksiini 2 vastu	Primaarse skleroseeriva kolangiidi ravi
Finnish	Humanisoitu IgG1 monoklonaalinen vasta-aine ihmisen eotaksiini 2:a vastaan	Primaarisen sklerosoivan kolangiitin hoito
French	Anticorps monoclonal humanisé IgG1 dirigé contre l'éotaxine-2 humaine	Traitement de la cholangite sclérosante primitive
German	Humanisierter monoklonaler IgG1-Antikörper gegen humanes Eotaxin-2	Behandlung der primär sklerosierenden Cholangitis
Greek	Ανθρωποποιημένο IgG1 μονοκλωνικό αντίσωμα έναντι της ανθρώπινης εοταξίνης-2	Θεραπεία της πρωτοπαθούς σκληρυντικής χολαγγειίτιδας
Hungarian	Humán eotaxin 2 ellenes humanizált IgG1 monoklonális antitest	Primer sclerotizáló cholangitis kezelése
Italian	Anticorpo monoclonale IgG1 umanizzato diretto contro l'eotassina-2 umana	Trattamento della colangite sclerosante primitiva
Latvian	Humanizēta IgG1 monoklonāla antiviela pret cilvēka eotaksīnu-2	Primārā sklerozējošā holangīta ārstēšana
Lithuanian	Žmogaus IgG1 monokloninis antikūnas prieš žmogaus eotaksiną-2	Pirminio sklerozuojančio cholangito gydymas
Maltese	Antikorp monoklonali IgG1 umanizzat kontra eotaxin-2 uman	Kura tal-kolanġite sklerosanti primarja

[1] At the time of designation

Language	Active ingredient	Indication
Polish	Humanizowane przeciwciało monoklonalne IgG1 przeciw ludzkiej eotaksynie-2	Leczenie pierwotnego stwardniającego zapalenia dróg żółciowych
Portuguese	Anticorpo monoclonal humanizado IgG1 contra eotaxina-2	Tratamento da colangite esclerosante primária
Romanian	Anticorp monoclonal umanizat IgG1 împotriva eotaxinei-2 umane	Tratamentul colangitei sclerozante primare
Slovak	Humanizovaná IgG1 monoklonálna protilátka proti ľudskému eotaxínu-2	Liečba primárnej sklerotizujúcej cholangitídy
Slovenian	Humanizirano monoklonsko protitelo IgG1 proti humanemu eotaksinu-2	Zdravljenje primarnega sklerozirajočega holangitisa
Spanish	Anticuerpo monoclonal IgG1 humanizado contra eotaxina-2 humana	Tratamiento de colangitis esclerosante primaria
Swedish	Humaniserad IgG1 monoklonal antikropp mot humant eotaxin-2	Behandling av primär skleroserande kolangit
Norwegian	Humanisert IgG1 monoklonalt antistoff mot human eotaksin-2	Behandling av primær skleroserende cholangitt
Icelandic	Mannaðlagað IgG1 einstofna mótefni gegn manna eotaxíni-2	Meðferð við frumkominni herslisgallrásarbólgu