16 November 2020
EMADOC-628903358-2620

Public summary of opinion on orphan designation

Maralixibat chloride for the treatment of biliary atresia

On 27 July 2020, orphan designation EU/3/20/2304 was granted by the European Commission to Granzer Regulatory Consulting & Services, Germany, for maralixibat chloride for the treatment of biliary atresia.

What is biliary atresia?

Biliary atresia is a condition in which the bile ducts that transport bile from the liver to the intestines are blocked or absent. As a result, the bile – which is used to digest fats – builds up in the liver and causes damage. Symptoms of the disease appear a few weeks after birth.

Biliary atresia is a debilitating and life-threatening disease because of the long-term damage to the liver, including loss of liver tissue and function, and cirrhosis (scarring of the liver).

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

At the time of designation, biliary atresia affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 5,000 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 application for orphan designation, no medicine was authorised for the treatment of patients with biliary atresia. Patients had surgery to restore flow of the bile, or required liver transplantation. They also received antibiotics, medicines to encourage bile flow, vitamin supplements and nutritional support.

How is this medicine expected to work?

The medicine is expected to block certain channels called ASBTs through which bile acids (a major component of bile) leave the intestines to travel in the blood back to the liver. When these channels

*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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

are blocked, the bile acids are instead excreted from the body. In this way, the medicine is expected to reduce the amount of bile acids in the liver and so reduce liver damage.

What is the stage of development of this medicine?

The effects of maralixibat chloride have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with maralixibat chloride in patients with biliary atresia had been started.

At the time of submission, maralixibat chloride was not authorised anywhere in the EU for the treatment of biliary atresia or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 18 June 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	Maralixibat chloride	Treatment of biliary atresia
Bulgarian	Мараликсибатов хлорид	Лечение на атрезия на жлъчните пътища
Croatian	Maraliksibat klorid	Liječenje bilijarne atrezije
Czech	Maralixabat chlorid	Léčba atrézie žlučových cest
Danish	Maralixibatchlorid	Behandling af biliær atresi
Dutch	Maralixibat chloride	Behandeling van biliaire atresie
Estonian	Maraliksibaatkloriid	Sapiteede atreesia ravi
Finnish	Maraliksibaattikloridi	Biliaarisen atresian hoito
French	Chlorure de maralixibat	Traitement de l'atrésie biliaire
German	Maralixibat Chlorid	Behandlung von Gallengangsatresie
Greek	Χλωριούχος μαραλιξιμπάτη	Θεραπεία της ατρησίας των χοληφόρων
Hungarian	Maralixibat-klorid	Epeút-elzáródás kezelése
Italian	Maralixibat cloruro	Trattamento dell’atresia delle vie biliari
Latvian	Maraliksibata hlorīds	Biliārās atrēzijas ārstēšana
Lithuanian	Maraliksibato chloridas	Tulžies latakų atrezijos gydymas
Maltese	Klorur tal-maralissibat	It-trattament tal-atresja biljari
Polish	Chlorek maraliksybatu	Leczenie atrezji układu żółciowego
Portuguese	Cloreto de maralixibato	Tratamento da atrésia das vias biliares
Romanian	Clorură de maralixibat	Tratamentul atreziei biliare
Slovak	Maralixibátchlorid	Liečba biliárnej atrézie
Slovenian	Maraliksibat klorid	Zdravljenje atrezije žolčevodov
Spanish	Cloruro de maralixibat	Tratamiento de la atresia biliar
Swedish	Maralixibat klorid	Behandling av biliär atresi
Norwegian	Maraliksibatklorid	Behandling av biliær atresi
Icelandic	Maralixibatklóríð	Meðhöndlun á gallrásalokun

[1] At the time of designation