13 September 2019
EMADOC-628903358-1134

Public summary of opinion on orphan designation

Elafibranor for the treatment of primary biliary cholangitis

On 25 July 2019, orphan designation EU/3/19/2182 was granted by the European Commission to Genfit, France, for elafibranor for the treatment of primary biliary cholangitis.

What is primary biliary cholangitis?

Primary biliary 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 to the intestines, where it helps to digest fats. Because of the damage to the ducts, bile acids, essential components of bile, build up in the liver causing 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 women.

Primary biliary cholangitis is a long-term debilitating and life-threatening disease because it can 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 biliary cholangitis affected less than 3.9 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 202,000 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, obeticholic acid and ursodeoxycholic acid were authorised in the EU for the treatment of primary biliary cholangitis, with obeticholic acid being used if ursodeoxycholic acid does not work well enough or the patient cannot take it. In advanced cases, the patient may need liver transplantation.

The sponsor has provided sufficient information to show that elafibranor might be of significant benefit for patients with primary biliary cholangitis. Early studies showed that it may work in patients whose condition does not improve with other treatment and the medicine's effects were greater than those

*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 518,400,000 (Eurostat 2019).

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

© European Medicines Agency, 2019. Reproduction is authorised provided the source is acknowledged.

with obeticholic acid. 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 is expected to work by attaching to and activating receptors (targets) called 'PPAR receptors', which control the levels of bile acid. By activating PPARs, this medicine is expected to reduce the levels of bile acid, thereby reducing damage of liver tissue that occurs in primary biliary cholangitis.

What is the stage of development of this medicine?

The effects of elafibranor have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with elafibranor in patients with primary biliary cholangitis were ongoing.

At the time of submission, elafibranor was not authorised anywhere in the EU for the treatment of primary biliary cholangitis 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 20 June 2019, 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

Sponsor's contact details:

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	Elafibranor	Treatment of primary biliary cholangitis
Bulgarian	Елафибранор	Лечение на първичен билиарен холангит
Croatian	Elafibranor	Liječenje primarnog bilijarnog kolangitisa
Czech	Elafibranor	Léčba primární biliární cholangoitidy
Danish	Elafibranor	Behandling af primær biliær cholangitis
Dutch	Elafibranor	Behandeling van primaire biliaire cholangitis
Estonian	Elafibranoor	Primaarse biliaarse kolangiidi ravi
Finnish	Elafibranori	Primaarin biliaarisen kolangiitin hoito
French	Elafibranor	Traitement de la Cholangite Biliaire Primitive
German	Elafibranor	Behandlung der primären biliären Cholangitis
Greek	Ελαφιμπρανόρη	Θεραπεία της πρωτοπαθούς χολικής χολλαγειίτιδας
Hungarian	Elafibranor	Primer biliáris cholangitis kezelése
Italian	Elafibranor	Trattamento della colangite biliare primaria
Latvian	Elafibranors	Primāra biliārā holangīta ārstēšana
Lithuanian	Elafibranoras	Pirminio biliarinio cholangito gydymas
Maltese	Elafibranor	Kura ta' kolanġite biljari primarja
Polish	Elafibranor	Leczenie pierwotnej marskości żółciowej
Portuguese	Elafibranor	Tratamento da colangite biliar primária
Romanian	Elafibranor	Tratamentul colangitei biliare primare
Slovak	Elafibranor	Liečba primárnej biliárnej cholangitídy
Slovenian	Elafibranor	Zdravljenje primarnega biliarnega holangitisa
Spanish	Elafibranor	Tratamiento de colangitis destructiva crónica no supurativa
Swedish	Elafibranor	Behandling av primär biliär cholangit
Norwegian	Elafibranor	Behandling av primær biliær kolangitt
Icelandic	Elafíbranor	Meðferð við frumkomnum gallvega kolangitis

[1] At the time of designation