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EMA/634183/2017

Public summary of opinion on orphan designation

Seladelpar for the treatment of primary biliary cholangitis

On 16 October 2017, orphan designation (EU/3/17/1930) was granted by the European Commission to Larode Ltd, United Kingdom, for seladelpar 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 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 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, 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 biliary cholangitis affected approximately 3.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 201,000 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 in patients who do not respond or cannot take ursodeoxycholic acid. In advanced cases, the patient may need a liver transplant.

*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 515,700,000 (Eurostat 2017).

The sponsor has provided sufficient information to show that seladelpar might be of significant benefit for patients with primary biliary cholangitis. Preliminary data showed that it may improve the outcome of patients who do not respond well to ursodeoxycholic acid and its effects compare well with those of 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 gamma receptors', which regulate bile flow. By activating PPARs, this medicine is expected to reduce bile flow, thereby reducing damage of liver tissue seen in primary biliary cholangitis.

What is the stage of development of this medicine?

The effects of seladelpar have been evaluated in experimental models.

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

At the time of submission, seladelpar was not authorised anywhere in the EU for primary biliary cholangitis. Orphan designation of seladelpar had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 September 2017 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, 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 languages¹, Norwegian and Icelandic

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

¹ At the time of designation