11 May 2021
EMADOC-628903358-4018

Public summary of opinion on orphan designation

Setanaxib for the treatment of primary biliary cholangitis

On 6 January 2021, orphan designation EU/3/20/2387 was granted by the European Commission to GenKyoTex S.A, France, for setanaxib for the treatment of primary biliary cholangitis.

What is primary biliary cholangitis?

Primary biliary cholangitis is an autoimmune disease in which there is long-term damage to the small bile ducts in the liver. 'Autoimmune' means that it is caused by the body's immune (defence) system attacking its own tissues. The reason why the immune system acts in this way is not known. These ducts transport 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 approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 104,000 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.

*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 biliary cholangitis because preliminary data show it can reduce symptoms when one of the two authorised treatments alone did not work well enough. The medicine may also be more effective than current treatments at reducing certain symptoms such as tiredness. 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 expected to work by blocking the action of enzymes called NOX1 and NOX4. They are involved in the production of harmful oxygen-based substances which are thought to cause liver damage in patients with primary biliary cholangitis. By blocking the actions of NOX1 and NOX4, the medicine is expected to reduce harmful substances in the liver, thereby reducing the symptoms of the disease.

What is the stage of development of this medicine?

The effects of setanaxib have been evaluated in experimental models.

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

At the time of submission, setanaxib was not authorised anywhere in the EU for the treatment of primary biliary cholangitis. Orphan designation of setanaxib had been granted in the EU and the United States for the treatment of systemic sclerosis and for the treatment of idiopathic pulmonary fibrosis.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 3 December 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.