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Public summary of opinion on orphan designation

Stiripentol for the treatment of primary hyperoxaluria

On 26 June 2020, orphan designation EU/3/20/2290 number was granted by the European Commission to Biocodex S.A.S., France, for stiripentol for the treatment of primary hyperoxaluria.

What is primary hyperoxaluria?

Primary hyperoxaluria is an inherited disease in which patients suffer from recurring kidney and bladder stones which lead to pain, blood in the urine and frequent urinary tract infections. The disease is caused by the lack of certain enzymes produced by the liver that are needed to breakdown a substance called glyoxalate in the body. Instead of being converted into the amino acid glycine, glyoxalate is converted into excess oxalate. This can form calcium oxalate deposits, which cause kidney and bladder stones and may damage the kidneys and other organs.

Primary hyperoxaluria is long-term debilitating and life threatening because of the high rate of kidney failure seen in patients with the condition.

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

At the time of designation, primary hyperoxaluria 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 designation, no satisfactory methods were authorised in the EU for treating primary hyperoxaluria. Different treatments were used to prevent the accumulation of calcium oxalate such as dietary changes, drinking plenty of fluids and taking vitamin B6. Kidney and liver transplantation have been possible options in patients with kidney failure.

*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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How is this medicine expected to work?

This medicine is expected to block the action of an enzyme in the liver, LDH-5, that is involved in transforming glyoxalate into oxalate. By blocking the action of this enzyme, it is expected to reduce the amount of oxalate in the body and thereby limit the damage caused by oxalate deposits.

What is the stage of development of this medicine?

The effects of stiripentol have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with stiripentol in patients with primary hyperoxaluria were ongoing.

At the time of submission, stiripentol was authorised in the EU for a type of epilepsy called severe myoclonic epilepsy in infancy.

At the time of submission, stiripentol was not authorised anywhere in the EU for the treatment of primary hyperoxaluria 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 May 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	Stiripentol	Treatment of primary hyperoxaluria
Bulgarian	Стирипентол	Лечение на първична хипероксалурия
Croatian	Stiripentola	Liječenje primarne hiperoksalurije
Czech	Stiripentol	Léčba primární hyperoxalurie
Danish	Stiripentol	Behandling af primær hyperoxaluri
Dutch	Stiripentol	Behandeling van primaire hyperoxalurie
Estonian	Stiripentoli	Esmase hüperoksaluuria ravi
Finnish	Stiripentoli	Primaarisen hyperoksalurian hoito
French	Stiripentol	Traitement de l'hyperoxalurie primaire
German	Stiripentol	Behandlung der primären Hyperoxalurie
Greek	Στιριπεντόλη	Θεραπεία της πρωτοπαθούς υπεροξαλουρίας
Hungarian	Stiripentol	Primer hiperoxaluria kezelésére
Italian	Stiripentolo	Trattamento delll'iperossaluria primaria
Latvian	Stiripentols	Primāras hiperoksalūrijas ārstēšana
Lithuanian	Stiripentolis	Pirminės hiperoksalurijos gydymas
Maltese	Stiripentol	Kura ta' iperoxalurja primarja
Polish	Styrypentol	Leczenie pierwotnej hiperoksalurii
Portuguese	Estiripentol	Tratamento da hiperoxalúria primária
Romanian	Stiripentol	Tratamentul hiperoxaluriei primare
Slovak	Stiripentol	Liečba primárnej hyperoxalúrie typu 1
Slovenian	Stiripentol	Zdravljenje primarne hiperoksalurije
Spanish	Stiripentol	Tratamiento de la hiperoxaluria primaria
Swedish	Stiripentol	Behandling av primär hyperoxaluri
Norwegian	Stiripentol	Behandling av primær hyperoksaluri
Icelandic	Stiripentol	Meðferð við fyrsta stigs sólarhringsútskilnaði

[1] At the time of designation