EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Variant of recombinant human fibroblast growth factor 19 for the treatment of primary biliary cirrhosis

On 22 August 2014, orphan designation (EU/3/14/1329) was granted by the European Commission to Diamond BioPharm Ltd, United Kingdom, for variant of recombinant human fibroblast growth factor 19 for the treatment of primary biliary cirrhosis.

What is primary biliary cirrhosis?

Primary biliary cirrhosis is an autoimmune disease in which there is gradual destruction of the small bile ducts in the liver. These ducts transport fluid called bile from the liver where it is produced towards the intestines, where it is used to help digest fats. As a result of the damage to the ducts, bile builds up in the liver causing damage. Early symptoms of the disease include tiredness and itching. The disease is ten times more common in women than in men.

Primary biliary cirrhosis is a long-term debilitating and life-threatening disease because, when the disease progresses, it may lead to liver cirrhosis (scarring of the liver) and liver failure (inability of the liver to work properly), 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 cirrhosis affected approximately 3.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 199,000 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, ursodeoxycholic acid was authorised in most EU countries for the treatment of primary biliary cirrhosis. 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 511,100,000 (Eurostat 2014).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with primary biliary cirrhosis because studies in experimental models show that it may reduce liver damage. 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?

Fibroblast growth factor 19 is a protein that controls the production of bile acid. The medicine contains a protein very similar to fibroblast growth factor 19 and is expected to work in the body in the same way as human fibroblast growth factor 19. When given to the patient, it is expected to decrease the production of bile acid, thereby reducing the symptoms of primary biliary cirrhosis and preventing further damage to the liver.

The protein in this medicine is made by a method known as 'recombinant DNA technology': it is made by bacteria into which a gene (DNA) has been introduced that makes them able to produce it.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for primary biliary cirrhosis. Orphan designation of the medicine 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 10 July 2014 recommending the granting of this designation.

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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:

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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	Variant of recombinant human fibroblast growth factor 19	Treatment of primary biliary cirrhosis
Bulgarian	Вариант на рекомбинантен човешки фибробластен растежен фактор 19	Лечение на първична билиарна цироза
Croatian	Varijanta rekombinantnog ljudskog faktora rasta fibroblasta 19	Liječenje primarne bilijarne ciroze
Czech	Varianta rekombinantního lidského fibroblastového růstového faktoru 19	Léčbě primární biliární cirhózy
Danish	Variant af rekombinant human fibroblastvækstfaktor 19	Behandling af primær biliær cirrose
Dutch	Variant van recombinant humaan fibroblast groeifactor 19	Behandeling van primaire biliaire cirrose
Estonian	Rekombinantse inimese fibroblasti kasvufaktori 19 variant	Primaarse biliaartsirroosi ravi
Finnish	Rekombinantin ihmisen fibroblasti-kasvutekijän 19 variantti	Primaarisen biliaarisen kirroosin hoito
French	Variant du facteur de croissance des fibroblastes 19 humains recombinants	Traitement de la cirrhose biliaire primitive
German	Variante des rekombinanten humanen Fibroblasten-Wachstumsfaktors 19	Behandlung der primären biliären Zirrhose
Greek	Παραλλαγή του ανασυνδυασμένου ανθρώπινου αυξητικού παράγοντα ινοβλαστών-19	Θεραπεία της πρωτοπαθούς χολικής κίρρωσης
Hungarian	Rekombináns humán fibroblaszt növekedési faktor 19 variáns	Primer biliaris cirrhosis kezelése
Italian	Variante del fattore di crescita dei fibroblasti 19 umano ricombinante	Trattamento della cirrosi biliare primitiva
Latvian	Rekombinanta cilvēka fibroblastu augšanas faktora 19 variants	Primāras biliāras cirozes ārstēšana
Lithuanian	Rekombinantinio žmogaus fibroblastų augimo faktoriaus 19 variantas	Pirminės biliarinės cirozės gydymas
Maltese	Varjant tal-fattur ta' tkabbir tal-fibroblasti tat-tip 19 uman rikombinanti	Kura ta' ċirrożi biljari primarja
Polish	Wariant rekombinowanego ludzkiego czynnika wzrostu fibroblastów 19	Leczenie pierwotnej żółciowej marskości wątroby
Portuguese	Variante do fator de crescimento 19 de fibroblastos humano recombinante	Tratamento da cirrose biliar primária
Romanian	Variantă a factorului uman recombinant de creştere fibroblastică 19.	Tratamentul cirozei biliare primitive
Slovak	Variant rekombinantného ľudského fibroblastového rastového faktora 19	Liečba primárnej biliárnej cirhózy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Varianta rekombinantnega človeškega fibroblastnega rastnega dejavnika 19	Zdravljenje primarn2 biliarne ciroze
Spanish	Variante del factor de crecimiento de fibroblastos 19 humano recombinante	Tratamiento de la cirrosis biliar primaria
Swedish	Variant av rekombinant human fibroblasttillväxtfaktor 19	Behandling av primär biliär cirrhos
Norwegian	Variant av rekombinant human fibroblastvekstfaktor 19	Behandling av primær biliær cirrhose
Icelandic	Afbrigði af raðbrigða manna trefjakímfrumuvaxtarþætti 19	Meðferð á frumkominni gallskorpulifur