29 January 2016
EMA/COMP/791407/2015
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 sclerosing cholangitis

On 14 December 2015, orphan designation (EU/3/15/1584) was granted by the European Commission to Diamond BioPharm Limited, United Kingdom, for variant of recombinant human fibroblast growth factor 19 for the treatment of primary sclerosing cholangitis.

What is primary sclerosing cholangitis?

Primary sclerosing 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 men.

Primary sclerosing 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 sclerosing cholangitis affected approximately 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 82,000 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 sclerosing cholangitis. 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 512,900,000 (Eurostat 2015).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with primary sclerosing cholangitis because early studies in experimental models show that it might reduce damage to the bile ducts. 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 acids. This medicine contains a protein very similar to fibroblast growth factor 19 and is expected to work in the body in a similar way to human fibroblast growth factor 19. When given to the patient, the medicine is expected to decrease the production of bile acids, thereby preventing further damage to the liver and reducing the symptoms of primary sclerosing cholangitis.

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 this medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with the medicine in patients with primary sclerosing cholangitis was planned.

At the time of submission, the medicine was not authorised anywhere in the EU for primary sclerosing cholangitis or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 November 2015 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Variant of recombinant human fibroblast growth factor 19	Treatment of primary sclerosing cholangitis
Bulgarian	Вариант на рекомбинантен човешки фибробластен растежен фактор 19	Лечение на първичен склерозиращ холангит
Croatian	Varijanta rekombinantnog ljudskog faktora rasta fibroblasta 19	Liječenje primarnog sklerozirajućeg kolangitisa
Czech	Varianta rekombinantního lidského fibroblastového růstového faktoru 19	Léčba primární sklerotizující cholangoitidy
Danish	Variant af rekombinant human fibroblastvækstfaktor 19	Behandling af primær skleroserende cholangitis
Dutch	Variant van recombinant humaan fibroblast groeifactor 19	Behandeling van primaire scleroserende cholangitis
Estonian	Rekombinantse inimese fibroblasti kasvufaktori 19 variant	Primaarse skleroseeriva kolangiidi ravi
Finnish	Rekombinantin ihmisen fibroblasti-kasvutekijän 19 variantti	Primaarisen sklerosoivan kolangiitin hoito
French	Variant du facteur de croissance des fibroblastes 19 humains recombinants	Traitement de la cholangite sclérosante primitive
German	Variante des rekombinanten humanen Fibroblasten-Wachstumsfaktors 19	Behandlung der primär sklerosierenden Cholangitis
Greek	Παραλλαγή του ανασυνδυασμένου ανθρώπινου αυξητικού παράγοντα ινοβλαστών-19	Θεραπεία της πρωτοπαθούς σκληρυντικής χολαγγειίτιδας
Hungarian	Rekombináns humán fibroblaszt növekedési faktor 19 variáns	Primer sclerotizáló cholangitis kezelése
Italian	Variante del fattore di crescita dei fibroblasti 19 umano ricombinante	Trattamento della colangite sclerosante primitiva
Latvian	Rekombinanta cilvēka fibroblastu augšanas faktora 19 variants	Primārā sklerozējošā holangīta ārstēšana
Lithuanian	Rekombinantinio žmogaus fibroblastų augimo faktoriaus 19 variantas	Pirminio sklerozuojančio cholangito gydymas
Maltese	Varjant tal-fattur ta' tkabbir tal-fibroblasti tat-tip 19 uman rikombinanti	Kura tal-kolanġite sklerosanti primarja
Polish	Wariant rekombinowanego ludzkiego czynnika wzrostu fibroblastów 19	Leczenie pierwotnego stwardniającego zapalenia dróg żółciowych
Portuguese	Variante do fator de crescimento 19 de fibroblastos humano recombinante	Tratamento da colangite esclerosante primária
Romanian	Variantă a factorului uman recombinant de creştere fibroblastică 19.	Tratamentul colangitei sclerozante primare
Slovak	Variant rekombinantného ľudského fibroblastového rastového faktora 19	Liečba primárnej sklerotizujúcej cholangitídy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Varianta rekombinantnega človeškega fibroblastnega rastnega dejavnika 19	Zdravljenje primarnega sklerozirajočega holangitisa
Spanish	Variante del factor de crecimiento de fibroblastos 19 humano recombinante	Tratamiento de colangitis esclerosante primaria
Swedish	Variant av rekombinant human fibroblasttillväxtfaktor 19	Behandling av primär skleroserande kolangit
Norwegian	Variant av rekombinant human fibroblastvekstfaktor 19	Behandling av primær skleroserende cholangitt
Icelandic	Afbrigði af raðbrigða manna trefjakímfrumuvaxtarþætti 19	Meðferð við frumkominni herslisgallrásarbólgu