6 May 2015
EMA/COMP/132952/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human monoclonal antibody binding to vascular adhesion protein-1 for the treatment of primary sclerosing cholangitis

On 19 March 2015, orphan designation (EU/3/15/1461) was granted by the European Commission to Biotie Therapies Corp, Finland, for recombinant human monoclonal antibody binding to vascular adhesion protein-1 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).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with primary sclerosing cholangitis because in early studies the medicine has been shown to reduce liver scarring. 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 a monoclonal antibody (a type of protein) that has been designed to recognise and attach to another protein called vascular adhesion protein-1 (VAP-1). VAP-1 plays a key role in the development of the inflammation that leads to liver damage and scarring in patients with primary sclerosing cholangitis. By attaching to VAP-1 and blocking its action, the medicine is expected to reduce scarring in the liver, thereby helping to reduce the symptoms and the progression of the disease.

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, no clinical trials with the medicine in patients with primary sclerosing cholangitis had been started.

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 February 2015 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	Recombinant human monoclonal antibody binding to vascular adhesion protein-1	Treatment of primary sclerosing cholangitis
Bulgarian	Рекомбинантно човешко моноклонално антитяло към съдов адхезионен протеин 1	Лечение на първичен склерозиращ холангит
Croatian	Rekombinantno ljudsko monoklonsko protutijelo koje se veže na vaskularni adhezijski protein-1	Liječenje primarnog sklerozirajućeg kolangitisa
Czech	Rekombinantní lidská monoklonální protilátka vázající se na vaskulární adhezivní protein-1	Léčba primární sklerotizující cholangoitidy
Danish	Rekombinant humant monoklonalt antistof der binder til vasculær adhesionsprotein-1	Behandling af primær skleroserende cholangitis
Dutch	Recombinant human monoclonal antilichaambinding aan vasculair adhesie protëine-1	Behandeling van primaire scleroserende cholangitis
Estonian	Rekombinantne inimese monoklonaalne antikeha, mis seob vaskulaarse adhessioonivalguga 1	Primaarse skleroseeriva kolangiidi ravi
Finnish	Ihmisen rekombinantti monoklonaalinen vasta-aine, joka sitoutuu verisuonen kiinnittymisproteiini-1:een	Primaarisen sklerosoivan kolangiitin hoito
French	Anticorps monoclonal recombinant humain se liant à la protéine 1 d'adhésion vasculaire	Traitement de la cholangite sclérosante primitive
German	Rekombinanter menschlicher monoklonaler Antikörper gegen Vaskuläres Adhäsionsprotein-1	Behandlung der primär sklerosierenden Cholangitis
Greek	Ανυνδυασμένο ανθρώπινο μονοκλωνικό αντίσωμα που προσδένεται στην αγγειακή πρωτεΐνη προσκόλλησης -1	Θεραπεία της πρωτοπαθούς σκληρυντικής χολαγγειίτιδας
Hungarian	Vaszkuláris adhéziós protein-1-hez kötött rekombináns humán monoklonális antitest	Primer sclerotizáló cholangitis kezelése
Italian	Anticorpo monoclonale recombinante umano ligante la proteina 1 d'adesione vascolare	Trattamento della colangite sclerosante primitiva
Latvian	Rekombinanta cilvēka monoklonālā antiviela, kas sasaistās ar vaskulārā adhēzijas proteīnu 1	Primārā sklerozējošā holangīta ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Rekombinantinis žmogaus monokloninis antikūnas, susietas su kraujagysliniu adhezijos baltymu-1	Pirminio sklerozuojančio cholangito gydymas
Maltese	Antikorp monoklonali uman rikombinanti li jintrabat mal-proteina 1 ta' adeżjoni vaskulari	Kura tal-kolanġite sklerosanti primarja
Polish	Rekombinowane ludzkie przeciwciało monoklonalne wiążące naczyniowe białko adhezyjne-1	Leczenie pierwotnego stwardniającego zapalenia dróg żółciowych
Portuguese	Anticorpo monoclonal humano recombinante que se liga à proteína de adesão vascular-1	Tratamento da colangite esclerosante primária
Romanian	Anticorp monoclonal uman recombinant ce se cuplează cu proteina 1 de adeziune vasculară	Tratamentul colangitei sclerozante primare
Slovak	Rekombinatná ľudská monoklonálna protilátka viažuca sa na vaskulárny adhézny protein-1	Liečba primárnej sklerotizujúcej cholangitídy
Slovenian	Rekombinantno humano monoklonsko protitelo proti žilnemu adhezijskemu proteinu-1	Zdravljenje primarnega sklerozirajočega holangitisa
Spanish	Anticuerpo monoclonal humano que lia a la proteina vascular de adhesion 1	Tratamiento de colangitis esclerosante primaria
Swedish	Rekombinant human monoclonal antikropp mot vaskulärt adhesionsprotein-1.	Behandling av primär skleroserande kolangit
Norwegian	Rekombinant humant monoklonalt antistoff som binder seg til vaskulær adhesjonsprotein-1	Behandling av primær skleroserende cholangitt
Icelandic	Raðbrigða manna einstofna mótefni sem binst æða viðloðunar próteini-1	Meðferð við frumkominni herslisgallrásarbólgu