27 March 2017
EMA/72259/2017

Public summary of opinion on orphan designation

Recombinant human club cell 10 KDa protein for the treatment of bronchiolitis obliterans syndrome

On 27 February 2017, orphan designation (EU/3/17/1842) was granted by the European Commission to EUDRAC Limited, United Kingdom, for recombinant human club cell 10 KDa protein for the treatment of bronchiolitis obliterans syndrome.

What is bronchiolitis obliterans syndrome?

Bronchiolitis obliterans syndrome is the most common complication affecting patients who have had a lung transplant. Rarely, it may also occur in patients who receive a bone marrow transplant. Bronchiolitis obliterans syndrome occurs when cells of the immune system (the body's natural defences) recognise the patient's lungs as 'foreign' and attack them. This leads to extensive scarring (fibrosis) that obstructs and damages the lungs, leading eventually to death.

Bronchiolitis obliterans syndrome is a debilitating and life-threatening disease due to the progressive damage to the lungs.

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

At the time of designation, bronchiolitis obliterans syndrome affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 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 European Union (EU) for the treatment of bronchiolitis obliterans syndrome. Patients received medicines such as ciclosporin and corticosteroids. Treatment aimed to reduce the activity of immune cells, thereby reducing their ability to attack the patient's lungs.

*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 515,700,000 (Eurostat 2017).

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

How is this medicine expected to work?

The medicine is made of a protein called 'club cell 10 KDa protein' (CC10) which is normally produced by cells in the lungs and which is lacking in patients with bronchiolitis obliterans syndrome. CC10 plays a crucial role in repairing lung tissue following injury by blocking several inflammatory substances. In particular, it reduces the number of neutrophils (a type of immune cell) in the lungs and blocks the production of cytokines (messenger molecules of the immune system) which are involved in the inflammatory process. By increasing CC10 levels, the medicine is expected to help repair lung tissue and reduce the symptoms of bronchiolitis obliterans syndrome.

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 bronchiolitis obliterans syndrome were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for bronchiolitis obliterans syndrome or designated as an orphan medicinal product elsewhere for this condition. The medicine had been granted orphan designation in the EU for bronchopulmonary dysplasia (a lung disease affecting premature babies).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 January 2017 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	Recombinant human club cell 10 KDa protein	Treatment of bronchiolitis obliterans syndrome
Bulgarian	Рекомбинантен човешки протеин 10 KDa от бронхиоларни екзокринни клетки	Лечение на синдром на облитериращ бронхиолит
Croatian	Rekombinantni ljudski protein od 10 kDa iz egzokrinih stanica bronhiola	Liječenje sindroma obliterirajućeg bronhiolitisa
Czech	Rekombinantní lidský protein bronchiolárních exokrinních buněk o hmotnosti 10 KDa	Léčba bronchiolitis obliterans syndromu
Danish	Rekombinant human club-celle 10 kDa protein	Behandling af bronchiolitis obliterans syndrom
Dutch	Recombinant humaan club celeiwit van 10 kDa	Behandeling van bronchiolitis obliterans syndroom
Estonian	Inimese club-rakue rekombinantne 10 KDa valk	Oblitereeriva bronhioliidi sündroomi ravi
Finnish	Ihmisen club-solun 10 KDa:n rekombinanttiproteiini	Obliteroiva bronkioliitti-oireyhtymän hoito
French	Protéine humaine recombinante de cellule club de 10 KDa	Traitement de la bronchiolite oblitérante
German	Rekombinantes menschliches 10-kDa Clubzell-Protein	Behandlung von Bronchiolitis obliterans Syndrom
Greek	Ανασυνδυασμένη ανθρώπινη πρωτεΐνη κυττάρου club 10 kDa	Θεραπεία του συνδρόμου αποφρακτικής βρογχιολίτιδας
Hungarian	10 kDA molekulatömegű rekombináns humán club-sejt-fehérje	Bronchiolitis obliterans szindróma kezelése
Italian	Proteina 10 KDa della cellula club umana ricombinante	Trattamento della bronchiolite obliterante
Latvian	Rekombinanta cilvēka bronhiolu sekrētšunu 10 KDa olbaltumviela	Obliterējoša bronhiolīta sindroma ārstēšana
Lithuanian	Rekombinantinis žmogaus bronchiolių egzokrininių ląstelių 10 KDa baltymas	Obliteruojančio bronchiolito sindromo gydymas
Maltese	Proteina 10 KDa ta' ċelloli club umani rikombinanti	Kura tas-sindromu ta' bronkite obliterans
Polish	Rekombinowane ludzkie białko z komórekoskrzelikowych o ciężarze 10 kD	Leczenie zespołu zarostowego zapalenia oskrzelików
Portuguese	Proteína humana recombinante de célula club de 10 Kda	Tratamento da síndrome de bronquiolite obliterante
Romanian	Proteină umană recombinantăde 10 kDa din celule bronhiolare exocrine	Tratamentul bronşiolitei obliterante

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Rekombinantný ľudský proteín clubových buniek o veľkosti 10 kDa	Liečba syndrómu bronchiolotis obliterans
Slovenian	Rekombinantni humani protein alveolarnih celic tipa II, z molekulsko maso 10 KDa	Zdravljenje obliteracijskega bronhiolitisa
Spanish	Proteína recombinante de célula exocrina bronquiolar humana de 10 KDa	Tratamiento de la Bronquiolitis Obliterante
Swedish	Rekombinant mänsklig claracell 10 KDa protein	Behandling av bronkiolitis obliterans-syndrom
Norwegian	Rekombinant humant Clara-celle 10 KDa-protein	Behandling av bronkiolitis obliterans syndrom
Icelandic	Raðbrigða manna klúbb fruma 10 KDa prótein	Til meðferðar við heilkenni stíflumyndandi berkjubólgu