



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant protein derived from the saliva of the *Ornithodoros moubata* tick for the treatment of Guillain-Barré syndrome

On 27 June 2016, orphan designation (EU/3/16/1687) was granted by the European Commission to Akari Therapeutics, Plc, United Kingdom, for recombinant protein derived from the saliva of the *Ornithodoros moubata* tick (also known as coversin or rEV576) for the treatment of Guillain-Barré syndrome.

What is Guillain-Barré syndrome?

Guillain-Barré syndrome is a condition of the nervous system that usually develops after a minor infection, such as a cold, in previously healthy individuals. It occurs when the antibodies produced by the immune system (the body's natural defence system) to target the infection turn against the nerve cells and attack them, leading to muscle weakness or paralysis. The weakness usually starts in the legs, then progresses over several days to affect the arms and facial muscles, leading to drooling, difficulty swallowing and breathing.

Guillain-Barré syndrome is a long-term debilitating and life-threatening condition due to breathing difficulties, nerve damage in arms and legs and organ dysfunction.

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

At the time of designation, Guillain-Barré syndrome affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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, the main treatments for Guillain-Barré syndrome were aimed at relieving the symptoms and reducing the duration of the disease. They consisted of immunoglobulins and

^{*}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 513,700,000 (Eurostat 2016).



plasma exchange, a procedure used to remove antibodies that are attacking the nerves by replacing the patient's plasma (the liquid part of the blood) with plasma from a donor.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with Guillain-Barré syndrome, because early laboratory data show a reduction of symptoms with the medicine, which may improve recovery. 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 consists of a protein obtained from a blood-feeding tick, *Ornithodoros moubata*, which the tick uses to suppress the immune response of its host so as to go undetected. The protein works by blocking part of the immune system known as the complement system, which consists of a series of proteins that can enhance the actions of antibodies and immune cells. When given to patients with Guillain-Barré syndrome, the protein is expected to block the patient's complement system, thereby weakening the immune system and reducing the inflammation and nerve damage linked to attacks by immune cells.

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, a clinical trial with the medicine in patients with Guillain-Barré syndrome was planned.

At the time of submission, the medicine was not authorised anywhere in the EU for Guillain-Barré syndrome 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 19 May 2016 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant protein derived from the saliva of the <i>Ornithodoros moubata</i> tick	Treatment of Guillain-Barré syndrome
Bulgarian	Рекомбинантен протеин, извлечен от слюнката на кърлеж <i>Ornithodoros moubata</i>	Лечение на синдрома на Гилен-Баре
Croatian	Rekombinantni protein dobiven iz sline krpelja <i>Ornithodoros moubata</i>	Liječenje sindroma Guillain-Barré
Czech	Rekombinantní protein odvozený ze slin klíštěte <i>Ornithodoros moubata</i>	Léčba Guillainova-Barrého syndromu
Danish	Rekombinant protein afledt af saliva fra flåten <i>Ornithodoros moubata</i>	Behandling af Guillain-Barré syndrom
Dutch	Recombinant eiwit afgeleid van het speeksel van de <i>Ornithodoros moubata</i> -teek	Behandeling van het Guillain-Barré syndroom
Estonian	Puugi <i>Ornithodoros moubata</i> süljest saadud rekombinantne valk	Guillaini-Barré sündroomi ravi
Finnish	<i>Ornithodoros moubata</i> -punkin syljestä peräisin oleva rekombinanttiproteiini	Guillain-Barrén oireyhtymän hoito
French	Protéine recombinante dérivée de la salive de tique <i>Ornithodoros moubata</i>	Traitement du syndrome de Guillain-Barré
German	Rekombinantes Protein, gewonnen aus dem Speichel der Zecke <i>Ornithodoros moubata</i>	Behandlung des Guillain-Barré-Syndroms
Greek	Ανασυνδυασμένη πρωτεΐνη προερχόμενη από σίελο του ακάρεως <i>Ornithodoros moubata</i>	Αγωγή για το σύνδρομο Guillain-Barré
Hungarian	<i>Ornithodoros moubata</i> kullancs nyálából nyert rekombináns fehérje	Guillain-Barré szindróma kezelése
Italian	Proteina ricombinante derivata dalla saliva della zecca <i>Ornithodoros moubata</i>	Trattamento della sindrome di Guillain-Barré
Latvian	Rekombinants proteīns, kas iegūts no <i>Ornithodoros moubata</i> ērču siekalām	Gijēna Barē (Guillain-Barré) sindroma ārstēšana
Lithuanian	Rekombinantinis baltymas, išgautas iš erkės <i>Ornithodoros moubata</i> seilių	Guillain-Barre sindromo gydymui
Maltese	Proteina rikombinanti mnissla mill-bżieq tal-qurdiena <i>Ornithodoros moubata</i>	Kura tas-sindrome Guillain-Barré
Polish	Rekombinowane białko wyprowadzone ze śliny kleszcza <i>Ornithodoros moubata</i>	Leczenie zespołu Guillain-Barré
Portuguese	Proteína recombinante derivada da saliva da carraça <i>Ornithodoros moubata</i>	Tratamento do síndrome de Guillain-Barré
Romanian	Proteină recombinantă obținută din saliva căpușei <i>Ornithodoros moubata</i>	Tratamentul sindromului Guillain-Barré-Strohl
Slovak	Rekombinantný proteín odvodený zo slín kliešťa <i>Ornithodoros moubata</i>	Liečba syndrómu Guillain-Barré

¹ At the time of designation

Language	Active ingredient	Indication
Slovenian	Rekombinantni protein, pridobljen iz sline klopa <i>Ornithodoros moubata</i>	Zdravljenje Guillain-Barréjevega sindroma
Spanish	Proteína recombinante derivada de la saliva de la garrapata <i>Ornithodoros moubata</i>	Tratamiento del síndrome Guillain-Barré
Swedish	Rekombinant protein framställt från saliven hos <i>Ornithodoros moubata</i> -fästing	Behandling av Guillain-Barrés syndrom
Norwegian	Rekombinant protein fremstilt fra spytt fra flåtten <i>Ornithodoros moubata</i>	Behandling av Guillain-Barrés syndrom
Icelandic	Raðbrigða prótein unnið úr munnvatni blódmítilsins <i>Ornithodoros moubata</i>	Meðferð við Guillain-Barré heilkenni