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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant derivative of C3 transferase for the treatment of traumatic spinal cord injury

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Rev.1: sponsor's change of address	4 February 2014
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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 5 September 2008, orphan designation (EU/3/08/563) was granted by the European Commission to Triskel EU Services, United Kingdom, for recombinant derivative of C3 transferase for the treatment of traumatic spinal cord injury.

The sponsorship was transferred to Vertex Pharmaceuticals (U.K.) Limited, United Kingdom, in January 2015.

What is traumatic spinal cord injury?

Traumatic spinal cord injury is damage to the spinal cord caused by an accident, such as a blow to the back. Injury to the spinal cord can damage and kill the nerve cells that run through the cord and that branch out from it. This can stop the flow of nerve impulses between the brain and the body, resulting in the loss of feeling, paralysis and even death, depending upon the severity of the injury and where it is located.

Traumatic spinal cord injury is life-threatening and chronically debilitating because it can cause paralysis of the arms and legs and reduce life expectancy.



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

At the time of designation, traumatic spinal cord injury affected approximately 4.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 211,000 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 submission of the application for orphan drug designation, methylprednisolone (a steroid) was authorised for the treatment of spinal cord injury in some countries in the Community. Methylprednisolone reduces inflammation and pressure on the spinal cord that can happen after it is damaged. Patients with spinal cord injury can also have surgery to reduce the pressure on the spine, but the role of surgery is controversial.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that recombinant derivative of C3 transferase might be of potential significant benefit for the treatment of traumatic spinal cord injury mainly because it has a new mechanism of action. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Recombinant derivative of C3 transferase is expected to work by inactivating a group of proteins called 'Rho proteins', which are believed to play a key role in preventing nerve cells from regrowing after they have been damaged. By blocking the activity of Rho proteins, this medicine may allow the nerve cells to repair themselves and regrow their damaged axons (the long processes of nerve cells along which nerve impulses pass). There is also some evidence that the blocking Rho proteins may also prevent the death of damaged nerve cells. Together, these effects may restore the flow of nerve impulses along the spinal cord in patients with traumatic spinal cord injury.

What is the stage of development of this medicine?

The effects of recombinant derivative of C3 transferase have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with traumatic spinal cord injury were ongoing.

Recombinant derivative of C3 transferase was not authorised anywhere worldwide for the treatment of traumatic spinal cord injury, at the time of submission.

Orphan designation for recombinant derivative of C3 transferase had been granted in the United States of America for the treatment of acute spinal cord injury.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 June 2008 recommending the granting of this designation.

*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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 502,800,000 (Eurostat 2008).

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant derivative of C3 transferase	Treatment of traumatic spinal cord injury
Bulgarian	Рекомбинантен дериват на C3 трансфераза	Лечение на гръбначно-мозъчна травма
Croatian	Rekombinantni derivat C3 transferaze	Liječenje traumatskih ozljeda kralježnične moždine
Czech	Rekombinantní derivát C3 transferázy	Léčba míšního traumatu
Danish	Rekombinant derivat af C3 transferase	Behandling af traumatisk rygmarvslæsion
Dutch	Recombinant C3 transferasederivaat	Behandeling van traumatisch ruggenmergletsel
Estonian	Rekombinantne C3 transferraasi derivatiiv	Traumaatilise seljaaju kahjustuse ravi
Finnish	Rekombinantti C3 transferaasin johdannainen	Selkäydinvamman hoito
French	Dérivé recombinant de C3-transférase	Traitement du traumatisme de la moëlle épinière
German	Rekombinante C3-Transferase	Behandlung von Rückenmarktrauma
Greek	Ανασυνδιασμένο παράγωγο της τρανσφεράσης C3	Θεραπεία τραύματος της σπονδυλικής στήλης
Hungarian	Rekombináns C3-transzferáz származék	Traumás gerincvelő sérülés kezelése
Italian	Derivato ricombinante della C3 transferasi	Trattamento del trauma acuto della colonna vertebrale
Latvian	C3 transferāzes rekombinants derivāts	Muguras smadzeņu traumatiska ievainojuma ārstēšana
Lithuanian	Rekombinantinis C3 transferazės derivatas	Nugaros smegenų trauminio pažeidimo gydymas
Maltese	Derivattiv rikombinanti ta' C3 transferase	Kura ta' korriment trawmatiku tan-nerv qawwi li jgħaddi minn ġos-sinla
Polish	Rekombinowana pochodna transferazy C3	Leczenie pourazowego uszkodzenia rdzenia kręgowego
Portuguese	Derivado recombinante da C3 transferase	Tratamento da lesão traumática da medula espinal
Romanian	Derivat recombinant de C3 transferază	Tratamentul leziunilor traumatice ale măduvei spinării
Slovak	Rekombinantný derivát C3 transferázy	Liečba traumatického poškodenia miechy
Slovenian	Rekombinantni derivat C3 transferaze	Zdravljenje travmatske poškodbe hrbtenjače
Spanish	Derivado recombinante de la C3 transferasa	Tratamiento de las lesiones espinales medulares traumáticas

¹ At the time of transfer of sponsorship

Language	Active ingredient	Indication
Swedish	Rekombinant derivativ av C3 transferas	Behandling av traumatisk ryggmärgsskada
Norwegian	Rekombinant derivat av C3 transferase	Behandling av traumatisk ryggmargsskade
Icelandic	Raðbrigða afleiða C3 transferasa	Meðferð mænuskaða vegna slyss