



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/k class for the treatment of spinal cord injury

On 19 January 2009, orphan designation (EU/3/08/605) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/k class for the treatment of spinal cord injury.

What is acute spinal cord injury?

The spinal cord can be injured through accidents, such as damage to the back, or by internal causes such as tumours or bleeding within the spine putting pressure on the spinal cord. Injury to the spinal cord can damage the nerves 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.

The complications include paralysis of the legs with or without paralysis of the arms, breathing problems, blood clots in the veins and the lungs, and repeated infections of the lungs, the airways, the kidneys and the urinary tract (the structures that carry urine). Spinal cord injury is life-threatening and chronically debilitating.

What is the estimated number of patients affected by the acute spinal cord injury*?

At the time of designation spinal cord injury affected approximately 2.2 and 4.2 in 10,000 people in the European Union (EU)*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around between 12,000 to 22,000 people.

What treatments are available?

Surgical intervention is often performed to decrease the pressure over the spine (decompression), but its role is controversial. Methylprednisolone sodium succinate is approved in many European countries for the treatment of spinal cord injury.

The sponsor has provided sufficient information to show that recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/k class for the treatment of acute spinal cord injury might be of significant benefit for the patients because its different mechanism of action is expected to improve the overall outcome in patients. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 502,282,000 (Eurostat 2008).

How is this medicine expected to work?

The product is expected to work by inactivating Nogo-A, which is believed to play role in inhibiting nerve cells from regrowing after they have been damaged. By blocking the activity of Nogo-A, 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), and thus improve the symptoms of spinal cord injury.

What is the stage of development of this medicine?

The effects of the recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/k class have been evaluated in experimental models.

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

At the time of submission, the recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/k class was not authorised anywhere in the world for acute spinal cord injury or designated as 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 5 November 2008 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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information:

Sponsor's contact details:

Novartis Europharm Limited

Wimblehurst Road

Horsham

West Sussex RH12 5AB

United Kingdom

Telephone: +44 1403 323542

Telefax: +44 1403 323259

E-mail: carol.beauchamp@novartis.com

Patients' associations contact points:

Associazione Luca Coscioni

via di Torre Argentina

76 - 00186 Roma

Italy

Telephone: +39 06 68 97 91

Telefax: +39 06 68 80 53 96

Danish Spinal Cord Injuries Association

Hans Knudsens Plads 1 A

DK 2100 København Ø

Denmark

Telephone: +45 39 29 35 55

Telefax: +45 39 29 39 48

E-mail: info@ryk.dk

I.W.A. – Irish Wheelchair Association

National headquarters

Áras Chúchulainn

Blackheath Drive

Clontarf

Dublin 3

Ireland

Telephone: +353 01 8186 400

Telefax: +33 01 8333 873

E-mail: info@iwa.ie

**Translations of the active ingredient and indication in all official EU languages,
Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/kappa class	Treatment of spinal cord injury
Bulgarian	Рекомбинантно човешко моноклонално антитяло на човешки Nogo-A протеин от IgG4/κ клас	Лечение на гръбначно-мозъчни травми
Czech	Rekombinantní lidská monoklonální protilátka k lidskému Nogo-A proteinu třídy IgG4/kappa	Léčba míšního traumatu
Danish	Rekombinant humant monoklonalt antistof mod humant Nogo-A protein fra IgG4/kappa-klassen	Behandling af rygmarsvulæsion
Dutch	Recombinant humaan monoklonaal antilichaam tegen humaan Nogo-A eiwit van de IgG4/kappa klasse	Behandeling van ruggenmergletsel
Estonian	Inimese IgG4/kappa klassi kuuluva Nogo-A valgu rekombineeritud monoklonaalne antikeha	Seljaaju vigastuse ravi
Finnish	Yhdistelmä-DNA-tekniikalla tuotettu ihmisen monoklonaalinen vasta-aine ihmisen IgG4/kappa-luokan Nogo-A –proteiinille	Selkäydinvamman hoito
French	Anticorps monoclonal humain recombinant anti- protéine humaine Nogo-A de la classe des IgG4/kappa	Traitement des lésions de la moëlle épinière
German	Rekombinanter humaner monoklonaler Antikörper gegen humanes Nogo-A Protein der IgG4/kappa Klasse	Behandlung der Rückenmarkverletzung
Greek	Ανασυνδυασμένο ανθρώπινο μονοκλωνικό αντίσωμα έναντι της ανθρώπινης πρωτεΐνης Nogo-A της τάξης IgG4/κ	Θεραπεία τραύματος της σπονδυλικής στήλης
Hungarian	Az IgG4/kappa osztályba tartozó humán Nogo-A fehérje elleni rekombináns humán monoclonális antitest	Gerincvelő sérülés kezelése
Italian	Anticorpo ricombinante umano monoclonale anti proteina Nogo-A, appartenente alla classe delle Immunoglobuline IgG4/kappa	Trattamento delle lesioni del midollo spinale
Latvian	Cilvēka rekombinantās IgG4/kappa klases monoklonālās antivielas pret Nogo-A proteīnu	Mugurkaula traumū ārstēšana
Lithuanian	Rekombinantinis žmogaus monokloninis antikūnas prieš žmogaus IgG4/kappa klasės NOGO-A baltymą	Nugaros smegenų sužalojimo gydymas
Maltese	Anti-korp monoklonali uman rikombinanti għall-proteina umana Nogo-A tal-klassi IgG4/κ	Kura ta' korriment tan-nerv qawwi li jgħaddi minn ġos-sinla
Polish	Rekombinowane ludzkie monoklonalne przeciwciało przeciwko ludzkiemu białku Nogo-A z klasy IgG4/κ	Leczenie uszkodzenia rdzenia kręgowego
Portuguese	Anticorpo monoclonal humano recombinante anti-proteína humana Nogo-A	Tratamento da lesão da medula espinal

	da classe IgG4/kappa	
Romanian	Anticorp monoclonal uman recombinant din clasa IgG4/kappa anti proteină umană Nogo-A	Tratamentul leziunilor măduvei spinării
Slovak	Rekombinatná humánna monoklonálna protilátka proti humánnej bielkovine Nogo-A triedy IgG4/kappa	Liečba poškodenia miechy
Slovenian	rekombinantno humano monoklonsko protitelo iz podrazreda IgG4/kappa proti humanemu proteinu Nogo-A	Zdravljenje poškodbe hrbtenjače
Spanish	Anticuerpo monoclonal humano recombinante de clase IgG4/kappa contra la proteína humana Nogo-A	Tratamiento de las lesiones de la médula espinal
Swedish	Rekombinant human monoklonal antikropp från humant Nogo A-protein av IgG4/κ-klass	Behandling av ryggmärgsskada
Norwegian	Rekombinant humant monoklonalt antistoff klasse IgG4/kappa rettet mot humant Nogo-A protein	Behandling av ryggmargsskade
Icelandic	Einstofna raðbrigða mannamótefni gegn Nogo-A mannapróteini í IgG4/kappa flokki	Meðferð mænuskaða vegna slyss