12 October 2010
EMA/COMP/454762/2010
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class for the treatment of amyotrophic lateral sclerosis

On 1 October 2010, orphan designation (EU/3/10/797) was granted by the European Commission to Glaxo Group Limited, United Kingdom, for recombinant humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class for the treatment of amyotrophic lateral sclerosis.

What is amyotrophic lateral sclerosis?

Amyotrophic lateral sclerosis (ALS) is a progressive disease of the nervous system, where nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate. This causes the muscles under their control to weaken and waste away, leading to paralysis. The symptoms of ALS vary from patient to patient, depending on which muscles weaken first, and include tripping up, falling over, loss of control of hand and arm movement, difficulty speaking, swallowing and breathing, persistent tiredness, twitching and cramping. ALS usually starts in mid-life. Men are about one-and-a-half times more likely to develop the disease than women.

ALS is a debilitating and life-threatening disease because of the gradual loss of muscle function and its effect on muscles used for breathing.

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

At the time of designation, ALS affected less than 1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 51,000 people, and is below the threshold 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, there was one medicine called riluzole authorised for ALS in the EU.

[*]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. This represents a population of 506,500,000 (Eurostat 2010).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

The sponsor has provided sufficient information to show that the medicine 'recombinant humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class' might be of significant benefit for patients with ALS because it works in a different way to the existing treatment and early studies in experimental models show that it might improve the outcome of patients with this condition. These assumptions 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 a protein called Nogo-A. Nogo-A acts on nerve cells to block the growth of nerve endings. Nogo-A is found in increased amounts in the muscles of patients with ALS, where it plays a role in the loss of muscle function. By attaching to Nogo-A, the medicine is expected to block its activity, helping to delay the onset of the symptoms of the disease.

What is the stage of development of this medicine?

The effects of recombinant humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class 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 ALS were ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for ALS. Orphan designation of the medicine had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 July 2010 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:

Glaxo Group Limited
Glaxo Wellcome House
Berkeley Avenue
Greenford
Middlesex UB6 0NN
United Kingdom
E-mail: neil.2.abbott@gsk.com

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 humanised monoclonal antibody to human Nogo-A protein of the IgG1/kappa class	Treatment of amyotrophic lateral sclerosis
Bulgarian	Рекомбинантно хуманизирано моноклонално антитяло към човешки Nogo-A протеин от IgG1/kappa клас	Лечение на амиотрофична латерална склероза
Czech	Rekombinantní humánní monoklonální protilátka proteinu Nogo-A třídy IgG1/kappa	Léčba amyotrofické laterální sklerózy
Danish	Rekombinant humaniseret monoklonalt antistof til humant Nogo-A-protein af klassen IgG1/kappa	Behandling af amyotrofisk lateralsklerose
Dutch	Recombinant gehumaniseerd monoclonaal antilichaam tegenover humaan Nogo-A proteïne van de IgG1/kappa klasse	Behandeling van amyotrofe lateraalsclerose
Estonian	Rekombinantne humaniseeritud monoklonaalne antikeha inimese IgG1/kappa klassi Nogo-A proteiini vastu	Amüotroofilise lateraalskleroosi ravi
Finnish	Rekombinantti, humanisoitu, monoklonaalinen vasta-aine ihmisen IgG1/kappa-luokan Nogo-A-proteiinia vastaan	Amyotrofisen lateraaliskleroosin hoito
French	Anticorps monoclonal recombinant humanisé de classe IgG1/kappa dirigé contre la protéine humaine Nogo-A	Traitement de la sclérose latérale amyotrophique
German	Rekombinanter humanisierter monoklonaler Antikörper gegen humanes Nogo-A Protein der IgG1/kappa Klasse	Behandlung der amyotrophen Lateralsklerose
Greek	Ανασυνδυασμένο εξανθρωπισμένο μονοκλονικό αντίσωμα για την ανθρώπινη πρωτεΐνη Nogo-A της κατηγορίας IgG1/k	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	rekombináns humanizált monoklonális ellenanyag a human IgG1/kappa osztályhoz tartozó NOGO-A protein ellen	Amyotrophiás lateral sclerosis kezelése
Italian	Anticorpo monoclonale umanizzato ricombinante della classe IgG1/kappa anti proteina umana Nogo-A	Trattamento della sclerosi laterale amiotrofica
Latvian	Rekombinanta humanizēta IgG1/kappa klases monoklonāla antiviela pret cilvēka Nogo-A proteīnu	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Rekombinantinis žmogaus monokloninis antikūnas prieš žmogaus IgG1/kappa klasės Nogo-A baltymą	Šoninės amiotrofinės sklerozės gydymas
Maltese	Antikorp monoklonali umanizzat rikombinanti għall-proteina umana Nogo-A tal-klassi IgG1/kappa	Kura tas-sklerosi laterali amjotrofika
Polish	Rekombinowane, humanizowane przeciwciało monoklonalne przeciw ludzkiemu białku Nogo-A z klasy IgG1/kappa	Leczenie stwardnienia bocznego zanikowego
Portuguese	Anticorpo monoclonal humanizado recombinante anti-proteína humana Nogo-A da classe IgG1/kappa	Tratamento da esclerose lateral amiotrófica

[1] At the time of designation

Romanian	Anticorp monoclonal recombinant umanizat din clasa IgG1/kappa, anti-proteina umană Nogo-A	Tratamentul sclerozei laterale amiotrofice
Slovak	Rekombinantná humanizovaná monoklonálna protilátka proti humánnej bielkovine Nogo-A triedy IgG1/kappa	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Rekombinantno humanizirano monoklonsko protitelo proti humani Nogo-A beljakovini razreda IgG1/kappa	Zdravljenje amiotrofične lateralne skleroze
Spanish	Anticuerpo monoclonal recombinante humanizado de clase IgG1/kappa contra la proteína humana Nogo-A	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Rekombinant humaniserad monoklonal antikropp till humant Nogo-A-protein av IgG1/kappa-klass	Behandling av amyotrofisk lateralskleros
Norwegian	Rekombinant humanisert monoklonalt antistoff mot humant Nogo-A-protein i IgG1/kappa-klassen	Behandling av amyotrofisk lateralsklerose
Icelandic	Raðbrigða mannaaðlagað, einstofna mótefni gegn Nogo-A-mannapróteini af IgG1/kappa-flokki	Meðferð við blandaðri hreyfitaugahrörnun