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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human monoclonal antibody against activin receptor type IIB for the treatment of inclusion body myositis

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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 9 August 2012, orphan designation (EU/3/12/1035) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for recombinant human monoclonal antibody against activin receptor type IIB for the treatment of inclusion body myositis.

What is inclusion body myositis?

Inclusion body myositis is an inflammatory disease of the muscles that leads to progressive muscle weakness and wasting. The exact causes are unknown, but are thought to involve a complex interplay between ageing and genetic and environmental factors. The disease mostly occurs in people aged above 50 years and symptoms primarily affect muscles in the arms and legs, where they can lead to loss of hand function and inability to walk.

Inclusion body myositis is a long-term debilitating and life-threatening condition due to the development of progressive weakness and wasting of the muscles which leads to a complete inability of the patient to live independently.

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

At the time of designation, inclusion body myositis affected approximately 0.05 in 10,000 people in the European Union (EU). This was equivalent to a total of around 3,000 people*, and is below the ceiling

*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 509,000,000 (Eurostat 2012).

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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 orphan designation, no satisfactory treatments were authorised in the EU for patients with inclusion body myositis.

How is this medicine expected to work?

The medicine 'recombinant human monoclonal antibody against activin receptor type IIB' is a monoclonal antibody, a type of protein that has been designed to recognise and attach to a specific structure in the body. It is expected to attach to a receptor on the surface of muscle cells, called activin receptor type IIB, thereby preventing other proteins (including myostatin and activin) from attaching to it. When myostatin and activin normally attach to this receptor, they prevent the growth of muscle mass. Therefore, by blocking the effects of these proteins, this medicine is expected to increase muscle mass and improve muscle function in patients with inclusion body myositis.

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 medicine in patients with inclusion body myositis were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for inclusion body myositis. 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 11 July 2012 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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant human monoclonal antibody against activin receptor type IIB	Treatment of inclusion body myositis
Bulgarian	Рекомбинантно човешко моноклонално антитяло срещу активин тип IIB рецептора	Лечение на спорадичен миозит с включвания
Czech	Rekombinantní lidskámonoklonální protilátka proti aktivinovému receptoru typu IIB	Léčba sporadické myozitidy s inkluzními tělísky
Danish	Rekombinant humant monoklonalt antistof mod aktivinreceptor type IIB	Behandling af sporadisk inklusionslegeme myositis
Dutch	Recombinant humaan monoclonaal antilichaam tegen activinereceptor type IIB	Behandeling van inclusielichaammyositis
Estonian	Aktiviini IIB tüüpi retseptori vastane rekombinantne inimese monoklonaalne antikeha	Inklusioonkeha müosiidi ravi
Finnish	Rekombinantti humaani monoklonaalinen vasta-aine tyypin IIB aktiviini-reseptoria kohtaan	Sporadisen inkluusiokappalemyosiitin hoito
French	Anticorps monoclonal humain recombinant dirigé contre le récepteur de l'activine de type IIB	Traitement de la myosite sporadique à inclusions
German	Rekombinanter humaner monoklonaler Antikörper gegen Activin Rezeptor Typ IIB	Behandlung der Einschlußkörpermyositis
Greek	Ανασυνδυασμένο ανθρώπινο μονοκλωνικό αντίσωμα έναντι του υποδοχέα της ακτιβίνης τύπου IIB	Θεραπεία της σποραδικής μυοσίτιδας εξ εγκλείστων
Hungarian	Aktivin IIB receptor ellenes rekombináns monoklonális antitest	Sporadikus zárványtestes myositis kezelése
Italian	Anticorpo monoclonale umano ricombinante anti recettore dell'attivina di tipo IIB	Trattamento della miosite sporadica da corpi inclusi
Latvian	Rekombinanta monokloniāla cilvēku antiviela pret IIB tipa aktivīna receptoriem	Sporādiska ieslēgto ķermenīšu miozīta ārstēšanai
Lithuanian	Rekombinantinis žmogaus monokloninis antikūnas prieš IIB tipo aktivino receptorių	Inkliuzinių kūnelių miozito gydymas
Maltese	Anti-korp monoklonali uman rikombinanti kontra r-riċettur tal-*activin* tat-tip IIB	Kura tal-mijosite ta' korpi ta' inklużjoni
Polish	Rekombinowane ludzkie przeciwciało monoklonalne przeciw receptorowi typu IIB dla aktywiny	Leczenie wtrętowego zapalenia mięśni

[1] At the time of designation

Language	Active ingredient	Indication
Portuguese	Anticorpo monoclonal recombinante humanizado anti recetor de tipo IIB da ativina	Tratamento da miosite por corpúsculos de inclusão
Romanian	Anticorp monoclonal uman recombinant anti-receptor al activinei tip IIB	Tratamentul miozitei cu corpi de incluzie
Slovak	Rekombinantná humánna monoklonálna protilátka proti receptoru aktivínu typu IIB	Liečba sporadickej myozitídy s inklúznymi telieskami
Slovenian	Rekombinantno humano monoklonsko protitelo proti receptorju tipa IIB za aktivin	Zdravljenje sporadičnega miozitisa z inkluzijskimi telesci
Spanish	Anticuerpo monoclonal recombinante humano contra el receptor de activina tipo IIB	Tratamiento de la miositis por cuerpos de inclusión
Swedish	Rekombinant human monoklonal antikropp mot aktivinreceptor typ IIB	Behandling of inklusionskroppsmyosit
Norwegian	Rekombinant humant monoklonalt antistoff mot aktivinreseptor typ IIB	Behandling av inklusjonslegememyositt
Icelandic	Raðbrigða manna einstofna mótefni gegn aktivín viðtaka gerð IIB	Meðferð innlyksu vöðvabólgu