10 November 2014
EMA/COMP/551233/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human monoclonal IgG1 antibody for fibroblast growth factor 23 for the treatment of X-linked hypophosphataemia

On 15 October 2014, orphan designation (EU/3/14/1351) was granted by the European Commission to NDA Group AB, Sweden, for recombinant human monoclonal IgG1 antibody for fibroblast growth factor 23 for the treatment of X-linked hypophosphataemia.

What is X-linked hypophosphataemia?

X-linked hypophosphataemia is a type of hereditary disorder characterised by low levels of phosphate in the blood (hypophosphatemia). Phosphate is a mineral essential to build bones and teeth and to maintain their strength. Phosphate levels are largely controlled by the kidneys, which eliminate excess phosphate in urine or reabsorb this mineral into the bloodstream when needed.

Patients with X-linked hypophosphataemia have high levels of a protein called fibroblast growth factor 23 (or FGF23). FGF23 signals the kidneys to stop reabsorbing phosphate into the bloodstream. If levels of FGF23 are high, the kidneys stop reabsorbing phosphate which is then eliminated from the body in the urine leading to low levels of phosphate. As a result, the disease causes delays in children's growth, bone pain and bone deformities (a disorder commonly known as 'rickets').

X-linked hypophosphataemia is a long-term debilitating condition due to bone deformities.

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

At the time of designation, X-linked hypophosphataemia affected between 0.002 and 0.04 in 10,000 people in the European Union (EU). This was equivalent to a total of between 100 to 2,000 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).

[*]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 511,100,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

What treatments are available?

At the time of designation, there were no satisfactory methods of treatment for X-linked hypophosphataemia in the EU. Patients with the disease were given phosphate by mouth to try to improve growth, and bone pain.

How is this medicine expected to work?

This medicine is a 'monoclonal antibody', a type of protein designed to recognise and attach to the FGF23 protein. By attaching to the FGF23 protein, the medicine is expected to 'neutralise' its activity leading to the kidneys being able to reabsorb phosphate and restore normal levels of phosphate in the blood. This is expected to improve the symptoms of the disease.

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 the medicine in patients with X-linked hypophosphataemia were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for X-linked hypophosphatemia. Orphan designation of the medicine had been granted in the United States of America for X-linked hypophosphatemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 September 2014 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:

NDA Group AB
Johanneslundsvägen 2
Oxfordhuset
194 81 Upplands Väsby
Sweden
Tel. +46 8 590 778 00
Fax +46 8 590 714 40
E-mail: medinfo@ndareg.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 human monoclonal IgG1 antibody for fibroblast growth factor 23	Treatment of X-linked hypophosphataemia
Bulgarian	Рекомбинантно човешко моноклонално антитяло от клас IgG1 срещу фибробластен растежен фактор 23	Лечение на Х-свързана хипофосфатемия
Croatian	Rekombinantno ljudsko monoklonsko IgG1 protutijelo protiv faktora rasta fibroblasta 23	Liječenje X vezane hipofosfatemije
Czech	Rekombinantní humánní monoklonální IgG1 protilátka k fibroblastovému růstovému faktoru 23	Léčba hypofosfatémie s dominantní dědičností vázanou na X chromozóm
Danish	Rekombinant humant IgG1 monoklonalt antistof mod Fibroblast Growth Factor 23	Behandling af X-bundet hypofosfatæmi
Dutch	Recombinant humaan IgG1 monoklonaal antilichaam voor Fibroblast groeifactor 23	Behandeling van X-linked hypofosfatemie
Estonian	Rekombinantne inimese monoklonaalne IgG1 tüüpi antikeha fibroplastide kasvufaktorile 23	X-liitelise hüpofosfateemia ravi
Finnish	Rekombinantti humaani monoklonaalinen IgG1-vasta-aine fibroblastikasvutekijä-23:lle	X-kromosomaalisen hypofosfatemian hoito
French	Anticorps IgG1 monoclonal humain recombinant dirigé contre le facteur 23 de croissance des fibroblastes	Traitement de l'hypophosphatasie liée au chromosome X
German	Humaner rekombinanter monoklonaler IgG1-Antikörper gegen Fibroblasten-Wachstumsfaktor 23	Behandlung der x-chromosomalen Hypophosphatämie
Greek	Ανασυνδυασμένο ανθρώπινο IgG1 μονοκλωνικό αντίσωμα έναντι του ινοβλαστικού αυξητικού παράγοντα 23	Θεραπεία της φυλοσύνδετης υποφωσφαταιμίας
Hungarian	Rekombináns, humán IgG1 monoklonális, fibroblaszt növekedési faktor 23 elleni antitest	X-kromoszómához kötött hypophosphatemia kezelése
Italian	Anticorpo monoclonale umano ricombinante IgG1 diretto contro il fattore di crescita dei fibroblasti 23	Trattamento dell'ipofosfatemia associata al cromosoma X
Latvian	Rekombinanta cilvēka IgG1 monoklonāla antiviela pret fibroblastu augšanas faktoru 23	Ar X hromosomu saistītas hipofosfatēmijas ārstēšana
Lithuanian	Rekombinantinis žmogaus IgG1 monokloninis antikūnis fibroblastų augimo faktoriui 23	Su X susijusios hipofosfatemijos gydymas
Maltese	Antikorp IgG1 monoklonali uman rikombinanti kontra fattur tat-tkabbir tal-fibroblasti tat-tip 23	Kura tal-ipofosfatemija marbuta mal-kromosoma X
Polish	Rekombinowane ludzkie przeciwciało monoklonalne klasy IgG1 przeciw czynnikowi wzrostu fibroblastów 23	Leczenie hipofosfatemii sprzężonej z chromosomem X
Portuguese	Anticorpo monoclonal IgG1 humano recombinante para o factor de crescimento de fibroblastos 23	Tratamento da hipofosfatémia ligada ao cromossoma X

[1] At the time of designation

Language	Active ingredient	Indication
Romanian	Anticorp monoclonal IgG1 uman recombinant anti-factor de creștere fibroblastic 23	Tratamentul hipofosfatemiei X-lincate
Slovak	Rekombinantná humánna monoklonálna protilátka triedy IgG1 proti fibroblastovému rastovému faktoru-23	Liečba hypofosfatémie viazanej na X-chromozóm
Slovenian	Humano rekombinantno monoklonsko protitelo IgG1 proti fibroblastnemu rastnemu faktorju 23	Zdravljenje na X-vezane hipofosfatemije
Spanish	Anticuerpo monoclonal IgG1 humano recombinante contra el factor de crecimiento de fibroblastos 23	Tratamiento de la hipofosfatemia ligada al cromosoma X
Swedish	Rekombinant human IgG1 monoklonal antikropp mot fibroblast tillväxtfaktor 23	Behandling av X-bunden hypofosfatemi
Norwegian	Rekombinant humant monoklonalt IgG1 antistoff mot fibroblast vekstfaktor 23	Behandling av X-bundet hypofosfatemi
Icelandic	Raðbrigða manna IgG1 einstofna mótefni gegn trefjakímfrumuvaxtarþætti 23	Meðferð við X-tengdum blóðfosfatsskorti