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EMA/COMP/698747/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Interferon alfa-n3 for the treatment of Middle East respiratory syndrome

On 11 November 2015, orphan designation (EU/3/15/1568) was granted by the European Commission to NV Hemispherx BioPharma Europe, Belgium, for interferon alfa-n3 for the treatment of Middle East respiratory syndrome.

What is Middle East respiratory syndrome?

Middle East respiratory syndrome is an infection caused by a virus called Middle East respiratory syndrome coronavirus. It usually affects the lungs and airways with fever, cough and shortness of breath. Patients may also get diarrhoea, vomiting and muscle pain.

Middle East respiratory syndrome is a severe and life-threatening illness because it can cause respiratory failure that requires mechanical ventilation and support in an intensive-care unit. Some patients may develop organ failure, especially of the kidneys, or septic shock (life-threatening drop in blood pressure).

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

At the time of designation, Middle East respiratory syndrome affected less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 500 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?

No satisfactory methods for treating Middle East respiratory syndrome were authorised in the EU at the time of designation. Management involved treatment of individual symptoms, with measures to support the working of vital organs in severe disease.

*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 512,900,000 (Eurostat 2015).

How is this medicine expected to work?

This medicine contains a type of interferon called interferon alfa-n3. Interferons are natural substances produced by the body that have a number of actions; one of these is to help fight off viruses. The Middle East respiratory syndrome virus overcomes the body's natural defences by stopping the body's own interferons from working properly. It is expected that giving additional interferon in the form of interferon alfa-n3 will help to restore the body's defence against the virus.

What is the stage of development of this medicine?

The effects of interferon alfa-n3 have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with interferon alfa-n3 in patients with Middle East respiratory syndrome had been started.

At the time of submission, interferon alfa-n3 was not authorised anywhere in the EU for Middle East respiratory syndrome or designated as an 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 8 October 2015 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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	Interferon alfa-n3	Treatment of Middle East respiratory syndrome
Bulgarian	Интерферон алфа-н3	Лечение на близкоизточен респираторен синдром
Croatian	Interferon alfa-n3	Liječenje bliskoistočnog respiratornog sindroma
Czech	Interferon alfa-n3	Léčba respiračního syndromu Středního východu
Danish	Interferon alfa-n3	Behandling af Mellemøsten respiratorisk syndrom
Dutch	Interferon alfa-n3	Behandeling van het Midden-Oosten respiratoir syndroom
Estonian	Interferoon alfa-n3	Lähis-Ida respiratoorse sündroom ravi.
Finnish	Interferoni alfa-n3	Lähi-idän hengitystieoireyhtymän hoito
French	Interféron alfa-n3	Traitemennt du syndrome respiratoire du Moyen Orient
German	Interferon Alfa-N3	Behandlung der Middle East Respiratory Syndrome Erkrankung
Greek	Ιντερφερόνη α-ν3	Θεραπεία της αναπνευστικού συνδρόμου της Μέσης Ανατολής
Hungarian	Interferon alfa-n3	Közel keleti légúti szindróma kezelése
Italian	Interferone alfa-N3	Trattamento della sindrome respiratoria medio-orientale
Latvian	Interferons alfa-N3	Tuvo Austrumu respiratorā sindroma ārstēšana
Lithuanian	Interferonas alfa-N3	Artimujų Rytų kvėpavimo sindromo gydymas
Maltese	Interferon alfa-N3	Kura tas-sindrome respiratorju tal-Lvant Nofsani
Polish	Interferon alfa-N3	Leczenie zespołu oddechowego Bliskiego Wschodu
Portuguese	Interferão alfa-n3	Tratamento do síndrome respiratório do Médio Oriente
Romanian	Interferon alfa-n3	Tratamentul sindromului respirator din Orientalul Mijlociu
Slovak	Interferón alfa-n3	Liečba respiračného syndrómu Stredného východu
Slovenian	Interferon alfa-n3	Zdravljenje bližnjevzhodnega respiratornega sindroma
Spanish	Interferón alfa-n3	Tratamiento de Síndrome Respiratorio del Medio Oriente
Swedish	Interferon alfa-N3	Behandling av Mellanöstern luftvägsinfektion
Norwegian	Interferon alfa-N3	Behandling av Midtøsten luftveisinfeksjon
Icelandic	Interferón alfa-N3	Meðferð Miðausturs loftvegasýkingar heilkennis

¹ At the time of designation