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EMA/COMP/396041/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Molgramostim for the treatment of acute respiratory distress syndrome

On 27 June 2016, orphan designation (EU/3/16/1685) was granted by the European Commission to Serendex Pharmaceuticals A/S, Denmark, for molgramostim for the treatment of acute respiratory distress syndrome.

What is acute respiratory distress syndrome?

Acute respiratory distress syndrome is a condition in which lung injury leads to inflammation and fluid in the air sacs in the lungs, resulting in insufficient oxygen passing into the blood. The lungs eventually become stiff with scar tissue and breathing becomes very difficult.

There are many possible causes of acute respiratory distress syndrome, including inhaling high concentrations of smoke, harmful substances, or oxygen; severe burns; blood infection; pneumonia (infection of the lungs); pancreatitis (inflammation of the pancreas, an organ close to the stomach) or trauma to other parts of the body. Symptoms occur suddenly and include fast shallow breathing, shortness of breath, and severe tiredness.

Acute respiratory distress syndrome is a life-threatening condition because of the worsening problems with breathing.

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

At the time of designation, acute respiratory distress syndrome affected approximately 3.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 170,000 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).

What treatments are available?

At the time of designation, there were no authorised products for the treatment of acute respiratory distress syndrome in the EU. Patients affected by the condition were treated with surfactant

*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 513,700,000 (Eurostat 2016).

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preparations but they were not authorised for the condition. A surfactant lines the airways and allows gases to pass easily between the lung and blood. Healthy lungs produce their own surfactant.

How is this medicine expected to work?

This medicine is made of molgramostim to be given by inhalation. Molgramostim is a copy of a human protein known as granulocyte macrophage colony-stimulating factor (GM-CSF), which stimulates the immune system. How it would work in acute respiratory distress syndrome is not clear but it appears to act by repairing the cells lining the lung and clearing out microbes. In clinical studies in patients with severe lung conditions, molgramostim treatment improved the flow of oxygen into the blood.

What is the stage of development of this medicine?

The effects of molgramostim have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with molgramostim in patients with acute respiratory distress syndrome were ongoing.

At the time of submission, molgramostim given by injection was authorised in the EU for increasing neutrophils (a type of white blood cell) in certain conditions.

At the time of submission, molgramostim was not authorised anywhere in the EU for acute respiratory distress 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 19 May 2016 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:

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Molgramostim	Treatment of acute respiratory distress syndrome
Bulgarian	Молграмостим	Лечение на остър респираторен дистрес синдром
Croatian	Molgramostim	Liječenje akutnog respiratornog distres sindroma
Czech	Molgramostim	Léčba syndromu akutní dechové tísně
Danish	Molgramostim	Behandling af akut respiratorisk distress syndrom
Dutch	Molgramostim	Behandeling van Acuut Respiratoir Distress Syndroom
Estonian	Molgramostim	Ägeda respiratoorse distressi sündroomi ravi
Finnish	Molgramostim	Akuutin hengitysvaikeusoireyhtymän hoito
French	Molgramostim	Traitement du Syndrome de Détresse Respiratoire Aiguë
German	Molgramostim	Behandlung des Akuten Atemnotsyndroms
Greek	Μολγραμοστιμ	Θεραπευτική αγωγή για το σύνδρομο οξείας αναπνευστικής δυσχέρειας
Hungarian	Molgramostim	Akut respiratorikus distressz szindróma kezelése
Italian	Molgramostim	Trattamento della sindrome da sofferenza respiratoria acuta
Latvian	Molgramostīms	Akūta respiratorā distresa sindroma ārstēšana
Lithuanian	Molgramostimas	Ūminio kvėpavimo sutrikimo sindromo gydymas
Maltese	Molgramostim	Kura ta' sindrome ta' tbatija respiratorja akuta
Polish	Molgramostym	Leczenie zespołu ostrej niewydolności oddechowej
Portuguese	Molgramostim	Tratamento da Síndrome de Deficiência Respiratória Aguda
Romanian	Molgramostim	Tratamentul sindromului de detresă respiratorie acută
Slovak	Molgramostim	Liečba syndrómu akútnej respiračnej tiesne
Slovenian	Molgramostim	Zdravljenje sindroma akutne dihalne stiske
Spanish	Molgramostim	Tratamiento del síndrome de insuficiencia respiratoria aguda
Swedish	Molgramostim	Behandling av akut respiratoriskt distress-syndrom
Norwegian	Molgramostim	Behandling av akutt lungesviktsyndrom
Icelandic	Molgramostím	Meðferð á bráðu andnauðarheilkenni

[1] At the time of designation