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EMA/COMP/660447/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Imatinib for the treatment of acute respiratory distress syndrome

On 19 November 2014, orphan designation (EU/3/14/1357) was granted by the European Commission to Numedicus Ltd, United Kingdom, for imatinib 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 failure to deliver enough oxygen to 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, toxins, 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 problems with breathing.

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

At the time of designation, acute respiratory distress syndrome affected less than 5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 256,000 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 511,100,000 (Eurostat 2014).

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preparations (substance similar to the one lining the airways that allows the lungs to expand properly) although they were not authorised for the condition.

How is this medicine expected to work?

Imatinib is a protein-tyrosine-kinase inhibitor. This means that it blocks some enzymes known as tyrosine kinases. In acute respiratory distress syndrome, imatinib is expected to work through several mechanisms, including by blocking a tyrosine kinase known as ARG (Abl-related gene). Early studies have shown that imatinib's blocking of ARG helps keep the barrier between the air sacs and the blood intact, suggesting that the medicine could reduce the amount of fluids entering air sacs and thereby reduce the symptoms of the disease.

What is the stage of development of this medicine?

The effects of imatinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with imatinib in patients with acute respiratory distress syndrome had been started.

At the time of submission, imatinib was authorised in the EU for the treatment of several types of cancer including chronic myeloid leukaemia.

At the time of submission, imatinib 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 9 October 2014 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	Imatinib	Treatment of acute respiratory distress syndrome
Bulgarian	Иматиниб	Лечение на остър респираторен дистрес синдром
Croatian	Imatinib	Liječenje akutnog respiratornog distres sindroma
Czech	Imatinib	Léčba syndromu akutní dechové tísně
Danish	Imatinib	Behandling af akut respiratorisk distress syndrom
Dutch	Imatinib	Behandeling van Acuut Respiratoir Distress Syndroom
Estonian	Imatiniib	Ägeda respiratoorse distressi sündroomi ravi
Finnish	Imatinibi	Akuutin hengitysvaikeusoireyhtymän hoito
French	Imatinib	Traitement du Syndrome de Détresse Respiratoire Aiguë
German	Imatinib	Behandlung des Akuten Atemnotsyndroms
Greek	Imatinib	Θεραπευτική αγωγή για το σύνδρομο οξείας αναπνευστικής δυσχέρειας
Hungarian	Imatinib	Akut respiratorikus distressz szindróma kezelése
Italian	Imatinib	Trattamento della sindrome da sofferenza respiratoria acuta
Latvian	Imatinibs	Akūta respiratorā distresa sindroma ārstēšana
Lithuanian	Imatinibas	Ūminio kvėpavimo sutrikimo sindromo gydymas
Maltese	Imatinib	Kura ta' sindrome ta' tbatija respiratorja akuta
Polish	Imatynib	Leczenie zespołu ostrej niewydolności oddechowej
Portuguese	Imatinib	Tratamento da Síndrome de Deficiência Respiratória Aguda
Romanian	Imatinib	Tratamentul sindromului de detresă respiratorie acută
Slovak	Imatinibi	Liečba syndrómu akútnej respiračnej tiesne
Slovenian	Imatinib	Zdravljenje sindroma akutne dihalne stiske
Spanish	Imatinib	Tratamiento del síndrome de insuficiencia respiratoria aguda
Swedish	Imatinib	Behandling av akut respiratoriskt distress-syndrom
Norwegian	Imatinib	Behandling av akutt lungesviktsyndrom
Icelandic	Imatíníb	Meðferð á bráðu andnauðarheilkenni

[1] At the time of designation