



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Interferon beta for the treatment of acute respiratory distress syndrome

On 27 June 2018, orphan designation (EU/3/07/505) was granted by the European Commission to Faron Pharmaceuticals Limited, Finland, for interferon beta 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); or damage 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 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 181,000 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 put on a ventilator (a machine that 'breathes' for the patient) and were given treatment for the underlying cause for the condition.

^{*}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 517,400,000 (Eurostat 2018).



How is this medicine expected to work?

Inflammation associated with acute respiratory distress syndrome makes blood vessels in the lung to become leaky. Interferon beta has an anti-inflammatory action; it is expected to reduce the leakage of blood and fluids from the blood vessels, thereby reducing the damage to the lungs in patients with the condition.

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 acute respiratory distress syndrome were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for acute respiratory distress syndrome or designated as an orphan medicinal product elsewhere for this condition.

This medicine had been designated orphan on 29 November 2007 for the treatment of acute lung injury. At the request of the sponsor and having assessed the additional information submitted, the COMP adopted an opinion on 24 May 2018 recommending the change of the orphan condition from acute lung injury to acute respiratory distress syndrome, in accordance to the most recent disease classification.

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

¹ At the time of designation