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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Drotrecogin alfa (activated) for the treatment of acute respiratory distress syndrome

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 22 September 2008, orphan designation (EU/3/08/565) was granted by the European Commission to Drugrecure Aps, Denmark, for drotrecogin alfa (activated) for the treatment of acute respiratory distress syndrome.

What is acute respiratory distress syndrome?

In Acute Respiratory Distress Syndrome (ARDS) the lungs are injured and this results in respiratory failure. There are many possible causes of the type of lung injury that leads to ARDS. These include 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.

Lungs contain alveoli, which are tiny air sacs where the oxygen is passed into the blood. In ARDS blood and fluid begin to leak into the alveoli. When this happens, oxygen cannot enter the alveoli, which means oxygen is no longer getting into the blood. Because the lungs are inflamed and filled with fluid, the patient finds it increasingly difficult to breathe. The inflammation (swelling and redness) in the lungs leads to scarring and vascular damage including formation of small clots. The lungs eventually become stiff with scar tissue and breathing becomes very difficult. ARDS is life-threatening because it makes breathing extremely difficult and can cause death.

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

At the time of designation ARDS affected less than 3.5 in 10,000 people in the European Union (EU). This was below the threshold for orphan designation, which is 5 people in 10,000, and is equivalent to a total of around 176,000 people*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

There were no authorised products for the condition in the Community at the time of submission of the application for orphan drug designation.

Existing treatments for patients affected by the condition included surfactant preparations that were used by not authorised for ARDS.

How is this medicine expected to work?

Drotrecogin alfa (activated) is a recombinant human Activated Protein C (rhAPC) with similar properties as the Activated Protein C produced by the body. Activated protein C (APC) prevents blood from clotting. Blood clotting can worsen the situation in ARDS when this happens in small vessels which become obstructed and further reduce oxygen exchange. This medicinal product is expected to act as the natural APC, stop blood clotting and preventing this way the worsening of the process that brings about ADRS.

What is the stage of development of this medicine?

The effects of activated drotrecogin alfa were evaluated in experimental models.

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

Activated drotrecogin alfa was not authorised anywhere worldwide for acute respiratory distress syndrome or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 July 2008 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 European Union) or insufficient returns on investment.

*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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 502,800,000 (Eurostat 2008).

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:

Pharmaorigin ApS
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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	Drotrecogin alfa (activated)	Treatment of acute respiratory distress syndrome
Bulgarian	Алфа дротрекогин (активиран)	Лечение на остър респираторен дистрес синдром
Czech	Drotrekogin alfa (aktivovaný)	Léčba syndromu akutní dechové tísně
Danish	Drotrecogin alfa (aktiveret)	Behandling af akut respiratorisk distress syndrom
Dutch	Drotrecogin alfa (geactiveerd)	Behandeling van Acuut Respiratoir Distress Syndroom
Estonian	Alfa-drotrekogiini (aktiveeritud)	Ägeda respiratoorse distressi sündroomi ravi
Finnish	Drotrekogiini alfa (aktivoitu)	Akuutin hengitysvaikeusoireyhtymän hoito
French	Drotrécogine alfa (activée)	Traitement du Syndrome de Détresse Respiratoire Aiguë
German	Drotrecogin alfa (aktiviert)	Behandlung des Akuten Atemnotsyndroms
Greek	Δροτρεκογίνη άλφα (ενεργοποιημένη)	Θεραπευτική αγωγή για το σύνδρομο οξείας αναπνευστικής δυσχέρειας
Hungarian	Drotrekogin alfa (aktivált)	Akut respiratorikus distressz szindróma kezelése
Italian	Drotrecogin alfa (attivato)	Trattamento della sindrome da sofferenza respiratoria acuta
Latvian	Alfa drotrekogīns (aktivēts)	Akūta respiratorā distresa sindroma ārstēšana
Lithuanian	Drotrekoginas alfa (aktyvintas)	Ūminio kvėpavimo sutrikimo sindromo gydymas
Maltese	Drotrecogin alfa (attivat)	Kura ta' sindrome ta' tbatija respiratorja akuta
Polish	Drotrekogina alfa (aktywowaną)	Leczenie zespołu ostrej niewydolności oddechowej
Portuguese	Drotrecogina alfa (activada)	Tratamento da Síndrome de Deficiência Respiratória Aguda
Romanian	Drotrecogin alfa (activat)	Tratamentul sindromului de detresă respiratorie acută
Slovak	Alfadrotrekogín (aktivovaný)	Liečba syndrómu akútnej respiračnej tiesne
Slovenian	Drotrekogin alfa (aktivirani)	Zdravljenje sindroma akutne dihalne stiske
Spanish	Drotrecogina alfa (activada)	Tratamiento del síndrome de insuficiencia respiratoria aguda
Swedish	Drotrecogin alfa (aktiverat)	Behandling av akut respiratoriskt distress-syndrom
Norwegian	Drotrecogin alfa (aktiveret)	Behandling av akutt lungesviktsyndrom
Icelandic	Drótrecogín alfa (virkjað)	Meðferð á bráðu andnauðarheilkenni

[1] At the time of designation