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Public summary of opinion on orphan designation

Pegylated adrenomedullin for the treatment of acute respiratory distress syndrome

On 27 July 2020, orphan designation EU/3/20/2301 was granted by the European Commission to Bayer AG, Germany, for pegylated adrenomedullin (also known as PEG-ADM or BAY 1097761) for the treatment of acute respiratory distress syndrome.

What is acute respiratory distress syndrome?

Acute respiratory distress syndrome (ARDS) 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; transfusion of high amounts of blood products; 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, ARDS affected approximately 3.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 166,000 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).

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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What treatments are available?

At the time of designation, corticosteroid medicines were authorised for treating ARDS in some EU countries. Physical methods and other medicines were used to help patients with their breathing and to reduce some symptoms.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with ARDS because laboratory studies showed that it works in a different way to reduce build-up of fluid in the lungs and improve the amount of oxygen passing into the blood.

How is this medicine expected to work?

The medicine contains a human hormone called adrenomedullin, which has several effects in the body including reducing inflammation and stopping blood vessels becoming leaky. By reducing inflammation and leakage of fluid from the blood vessels into the lung tissue, the medicine is expected to help the lungs work normally and allow oxygen to enter the blood more efficiently in patients with ARDS.

This medicine is made up of adrenomedullin attached to a chain of polyethylene glycol (PEG) to make it remain in the body for longer. It is expected to be used through a nebuliser, a device that creates a fine spray of the medicine for the patient to breathe in.

What is the stage of development of this medicine?

The effects of pegylated adrenomedullin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with pegylated adrenomedullin in patients with ARDS had been started.

At the time of submission, pegylated adrenomedullin was not authorised anywhere in the EU for the treatment of ARDS or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 18 June 2020, 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

Contact details of the current sponsor for this orphan designation can be found on EMA website.

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	Pegylated adrenomedullin	Treatment of acute respiratory distress syndrome
Bulgarian	Пегилиран адреномедулин	Лечение на остър респираторен дистрес синдром
Croatian	Pegilirani adrenomedulin	Liječenje akutnog respiratornog distres sindroma
Czech	Pegylovaný adrenomedulin	Léčba syndromu akutní dechové tísně
Danish	Pegyleret adrenomedullin	Behandling af akut respiratorisk distress syndrom
Dutch	Gepegyleerd adrenomedulline	Behandeling van Acuut Respiratoir Distress Syndroom
Estonian	Pegüleeritud adrenomedulliin	Ägeda respiratoorse distressi sündroomi ravi
Finnish	Pegyloitu adrenomedulliini	Akuutin hengitysvaikeusoireyhtymän hoito
French	Adrénomédulline pégylée	Traitement du Syndrome de Détresse Respiratoire Aiguë
German	Pegyliertes adrenomedullin	Behandlung des Akuten Atemnotsyndroms
Greek	Πεγκυλιωμένη αδρενομεντουλίνη	Θεραπευτική αγωγή για το σύνδρομο οξείας αναπνευστικής δυσχέρειας
Hungarian	Pegilált adrenomedullin	Akut respiratorikus distressz szindróma kezelése
Italian	Adrenomedullina pegilata	Trattamento della sindrome da sofferenza respiratoria acuta
Latvian	Pegilēts adrenomedulīns	Akūta respiratorā distresa sindroma ārstēšana
Lithuanian	Pegiliuotas adrenomedulinas	Ūminio kvėpavimo sutrikimo sindromo gydymas
Maltese	Adrenomedullin pegilat	Kura ta' sindrome ta' tbatija respiratorja akuta
Polish	Pegylowana adrenomedulina	Leczenie zespołu ostrej niewydolności oddechowej
Portuguese	Adrenomedulina peguilada	Tratamento da Síndrome de Deficiência Respiratória Aguda
Romanian	Adrenomedulină pegilată	Tratamentul sindromului de detresă respiratorie acută
Slovak	Pegylovaný adrenomedulín	Liečba syndrómu akútnej respiračnej tiesne
Slovenian	Pegiliran adrenomedulin	Zdravljenje sindroma akutne dihalne stiske
Spanish	Adrenomedulina pegilada	Tratamiento del síndrome de insuficiencia respiratoria aguda
Swedish	Pegylerat adrenomedullin	Behandling av akut respiratoriskt distress-syndrom

[1] At the time of designation

Language	Active ingredient	Indication
Norwegian	Pegylert adrenomedullin	Behandling av akutt lungesviktsyndrom
Icelandic	Pegýlerað adrenómedúllín	Meðferð á bráðu andnauðarheilkenni