



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

6 April 2018
EMA/52859/2018

Public summary of opinion on orphan designation

Rusalatide acetate for the treatment of acute radiation syndrome

On 22 February 2018, orphan designation (EU/3/18/1985) was granted by the European Commission to Raremoon Consulting Ltd, United Kingdom, for rusalatide acetate (also known as TP508) for the treatment of acute radiation syndrome.

What is acute radiation syndrome?

Acute radiation syndrome (also known as radiation sickness) is a severe illness caused by exposure to a high dose of radiation in a very short period. This can occur, for example, following an accident at a nuclear power plant or the use of radioactive material for medical purposes.

Symptoms can begin within a few hours of exposure and their severity depends on the amount of radiation absorbed by the body. Relatively small amounts result in gastrointestinal effects such as nausea (feeling sick), vomiting and diarrhoea, reduction in blood cell count, and tendency to infection and bleeding. Relatively large amounts of radiation can result in effects on the central nervous system (brain and spinal cord) and rapidly lead to death.

Acute radiation syndrome is a life-threatening condition because it can lead to damage of multiple organs and death.

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

At the time of designation, acute radiation syndrome affected less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 500 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, no satisfactory methods were authorised in the EU for the treatment of acute radiation syndrome.

^{*}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?

The medicine mimics the action of thrombin, one of the substances involved in stimulating tissue repair after injury. In laboratory studies the medicine has been shown to help cells repair breaks in their DNA caused by radiation, as well as stimulating the growth of blood vessels, reducing inflammation and stimulating stem cells for tissue repair. These actions are expected to help reduce the damage caused by radiation.

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, no clinical trials with the medicine in patients with acute radiation syndrome had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for acute radiation syndrome. Orphan designation of the medicine had been granted in the United States for acute radiation syndrome.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 January 2018 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

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	Rusalatide acetate	Treatment of acute radiation syndrome
Bulgarian	Русалатид ацетат	лечение на остра лъчева болест
Croatian	Rusalatid acetat	Liječenje akutnog radijacijskog sindroma
Czech	Rusalatid acetát	Léčba akutní radiační syndromu
Danish	Rusalatidacetat	Behandling af akut bestråling syndrom
Dutch	Rusalatide acetate	Behandeling van stralingsziekte
Estonian	Rusalatiidatsetaat	ägeda kiirgus sündroomi ravi
Finnish	Rusalatidi asetaatti	Säteily sairauden hoito
French	Acétate de rusalatide	Traitement du syndrome d'irradiation aiguë
German	Rusalatid-acetat	Behandlung der Strahlenkrankheit
Greek	Οξική ρησαλατιδίη	θεραπεία του συνδρόμου οξείας ακτινοβόλησης
Hungarian	Ruzalatid-acetát	Sugárbetegség kezelése
Italian	Rusalatide acetato	Trattamento della sindrome acuta da radiazioni
Latvian	Rusalatīda acetāts	Akūta radiācijas sindroma ārstēšana
Lithuanian	Rusalatido acetatas	Ūminio radiacinio sindromo gydymas
Maltese	Aċetat rusalaturiku	Kura tas-sindrome ta' radjazzjoni akuta
Polish	Octan rusalatydu	Leczenie ostrej choroby popromiennej
Portuguese	Acetato de rusalatida	Tratamento do síndrome agudo das radiações
Romanian	Acetat de rusalatidă	Tratamentul sindromului acut de iradiere
Slovak	Acetát rusalatidu	Liečba akútneho radiačného syndrómu
Slovenian	Rusalatid acetat	Zdravljenje akutnega radiacijskega sindroma
Spanish	Acetato de rusalatida	Tratamiento del síndrome de radiación aguda
Swedish	Rusalatidacetat	Behandling av akut strålningsyndrom
Norwegian	Rusalatidacetat	Behandling av akutt strålesyndrom
Icelandic	Rúsalatíð asetat	Meðferð bráðrar geislunar heilkenni

¹ At the time of designation