16 December 2019
EMADOC-628903358-1036

Public summary of opinion on orphan designation

Imidazolyl ethanamide pentandioic acid for the treatment of acute radiation syndrome

On 28 June 2019, orphan designation EU/3/19/2173 was granted by the European Commission to Myelo Therapeutics GmbH, Germany, for imidazolyl ethanamide pentandioic acid (also known as Myelo001) 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 of the body to a high dose of radiation in a very short period of time. 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. 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 failure 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 518,400,000 (Eurostat 2019).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

How is this medicine expected to work?

The exact way the medicine works is not clear but it is thought to affect the activity of a protein in the body called NFkB. When the body is exposed to radiation, NFkB causes release of substances that affect the production of blood cells. This medicine is expected to block the activity of NFkB and as a result slow down the destruction of blood cells and reduce the time it takes for the production of blood cells to recover.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

For ethical reasons, clinical trials with the medicine in patients with acute radiation syndrome were not planned, but clinical trials were conducted in patients with low white blood cell count who had been treated with cancer medicine or had received radiotherapy.

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 23 May 2019, 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.

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	Imidazolyl ethanamide pentandioic acid	Treatment of acute radiation syndrome
Bulgarian	Имидазолил етанамид пентандиева киселина	Лечение на остра лъчева болест
Croatian	Imidazolil etanamid pentandioična kiselina	Liječenje akutnog radijacijskog sindroma
Czech	Imidazolyl ethanamid pentandiová kyselina	Léčba akutní radiační syndromu
Danish	Imidazolylethanamidpentandionsyre	Behandling af akut bestråling syndrom
Dutch	Imidazolyl ethanamide pentaandionzuur	Behandeling van stralingsziekte
Estonian	Imidasolüületaanamiid pentaandioonhape	Ägeda kiirgus sündroomi ravi
Finnish	Imidatsolyylietanamidi pentaanidihappo	Säteilysairauden hoito
French	Acide imidazolyl éthanamide pentandioïque	Traitement du syndrome d'irradiation aiguë
German	Imidazolylethanamidpentandiosäure	Behandlung der Strahlenkrankheit
Greek	Ιμιδαζολυλ αιθαναμίδιο πεντανδιοϊκό οξύ	Θεραπεία του συνδρόμου οξείας ακτινοβόλησης
Hungarian	Imidazolil-etánamid pentandioinsav	Sugárbetegség kezelése
Italian	Acido pentandioico di imidazolil etanamide	Trattamento della sindrome acuta da radiazioni
Latvian	Imidazolil-etānamīda pentāndiskābe	Akūta radiācijas sindroma ārstēšana
Lithuanian	Imidazolilo etanamido pentandioinė rūgštis	Ūminio radiacinio sindromo gydymas
Maltese	Aċidu pentandijojku ta' imidazolyl ethanamide	Kura tas-sindrome ta' radjazzjoni akuta
Polish	Kwas imidazoliloetanamidowy pentandiowy	Leczenie ostrej choroby popromiennej
Portuguese	Ácido imidazolil etanamida pentandióico	Tratamento do sindrome agudo das radiações
Romanian	Acidi imidazolil etanamid pentandioic	Tratamentul sindromului acut de iradiere
Slovak	Imidazolyl-etánamidová kyselina pentánová	Liečba akútnehoradiačného syndrómu
Slovenian	Pentandioična kislina imidazolil etanamida	Zdravljenje akutnega radiacijskega sindroma
Spanish	Ácido Imidazolil etanamida pentandioico	Tratamiento del síndrome de radiación aguda
Swedish	Imidazolyletanamidpentandionsyra	Behandling av akut strålningssyndrom
Norwegian	Imidazolyletanamidpentandisyre	Behandling av akutt strålesyndrom
Icelandic	Imídasólýl etanamíð pentandíósýra	Meðferð bráðrar geislunar heilkenni

[1] At the time of designation