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EMA/678539/2018

## Public summary of opinion on orphan designation

### Melatonin for the treatment of acute radiation syndrome

On 26 October 2018, orphan designation (EU/3/18/2077) was granted by the European Commission to Worphmed S.R.L., Italy, for melatonin 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. 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 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 approximately 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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.

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\*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?**

Melatonin is a naturally occurring hormone. The exact way it works in acute radiation syndrome is not clear. Based on results from laboratory studies, it is thought that melatonin may act as an 'antioxidant'. This means that it may protect the patient's tissues from damage caused by toxic substances containing oxygen that build up when the body is exposed to radiation.

## **What is the stage of development of this medicine?**

The effects of melatonin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with melatonin in patients with acute radiation syndrome had been started.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 September 2018 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 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 languages<sup>1</sup>, Norwegian and Icelandic

| Language   | Active ingredient | Indication                                     |
|------------|-------------------|------------------------------------------------|
| English    | Melatonin         | Treatment of acute radiation syndrome          |
| Bulgarian  | Мелатонин         | Лечение на остра лъчева болест                 |
| Croatian   | Melatonin         | Liječenje akutnog radijacijskog sindroma       |
| Czech      | Melatonin         | Léčba akutní radiační syndromu                 |
| Danish     | Melatonin         | Behandling af akut bestråling syndrom          |
| Dutch      | Melatonine        | Behandeling van stralingsziekte                |
| Estonian   | Melatoniin        | Ägeda kiirgus sündroomi ravi                   |
| Finnish    | Melatonini        | Säteilysauroden hoito                          |
| French     | Mélatonine        | Traitement du syndrome d'irradiation aiguë     |
| German     | Melatonin         | Behandlung der Strahlenkrankheit               |
| Greek      | Μελατονίνη        | Θεραπεία του συνδρόμου οξείας ακτινοβόλησης    |
| Hungarian  | Melatonin         | Akut sugárbetegség kezelése                    |
| Italian    | Melatonina        | Trattamento della sindrome acuta da radiazioni |
| Latvian    | Melatonīns        | Akūta radiācijas sindroma ārstēšana            |
| Lithuanian | Melatoninas       | Ūminio radiacinio sindromo gydymas             |
| Maltese    | Melatonina        | Kura tas-sindrome ta' radjazzjoni akuta        |
| Polish     | Melatonina        | Leczenie ostrej choroby popromiennej           |
| Portuguese | Melatonina        | Tratamento do síndrome agudo das radiações     |
| Romanian   | Melatonină        | Tratamentul sindromului acut de iradiere       |
| Slovak     | Melatonín         | Liečba akútnehoradiačného syndrómu             |
| Slovenian  | Melatonin         | Zdravljenje akutnega radiacijskega sindroma    |
| Spanish    | Melatonina        | Tratamiento del síndrome de radiación aguda    |
| Swedish    | Melatonin         | Behandling av akut strålningssyndrom           |
| Norwegian  | Melatonin         | Behandling av akutt strålesyndrom              |
| Icelandic  | Melatónín         | Meðferð á bráðu geislunarheilkenni             |

<sup>1</sup> At the time of designation