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EMA/COMP/510065/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human interleukin-12 for treatment of acute radiation syndrome

On 29 August 2016, orphan designation (EU/3/16/1727) was granted by the European Commission to Coté Orphan Consulting UK Limited, United Kingdom, for recombinant human interleukin-12 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. 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).

[*]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 513,700,000 (Eurostat 2016).

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What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of acute radiation syndrome.

How is this medicine expected to work?

This medicine is a copy of a natural protein known as interleukin-12 that has been made by 'recombinant DNA technology': it is made by cells into which genes (DNA) have been introduced that make the cells able to produce the protein. Interleukin-12 has several actions in the body, including helping the immune cells to fight infections and, along with other proteins, stimulating the production of blood cells. Based on results of studies in animals, the medicine is expected to be able to limit the effects of radiation on the bone marrow (where blood cells are produced) and the gut.

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, no clinical trials in patients with acute radiation syndrome are planned.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2016 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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant human interleukin-12	Treatment of acute radiation syndrome
Bulgarian	Рекомбинантен човешки интерлевкин 12	Лечение на остра лъчева болест
Croatian	Rekombinantni ljudski interleukin-12	Liječenje akutnog radijacijskog sindroma
Czech	Rekombinantní lidský interleukin-12	Léčba akutní radiační syndromu
Danish	Rekombinant humant interleukin 12	Behandling af akut bestråling syndrom
Dutch	Recombinant humaan interleukine-12	Behandeling van stralingsziekte
Estonian	Rekombinantne inimese interleukiin-12	Ägeda kiirgus sündroomi ravi
Finnish	Rekombinanttitekniikalla tehty ihmisen interleukiini 12	Säteilysairauden hoito
French	Interleukine-12 humaine recombinante	Traitement du syndrome d'irradiation aiguë
German	Rekombinantes humanes Interleukin-12	Behandlung der Strahlenkrankheit
Greek	Ανασυνδυασμένη ανθρώπινη ιντερλευκίνη-12	Θεραπεία του συνδρόμου οξείας ακτινοβόλησης
Hungarian	Rekombináns humán interleukin-12	Sugárbetegség kezelése
Italian	Interleuchina-12 umana ricombinante	Trattamento della sindrome acuta da radiazioni
Latvian	Rekombinēts cilvēka interleikīns-12	Akūta radiācijas sindroma ārstēšana
Lithuanian	Rekombinantinis žmogaus interleukinas–12	Ūminio radiacinio sindromo gydymas
Maltese	Interleukin 12 uman rikombinanti	Kura tas-sindrome ta' radjazzjoni akuta
Polish	Rekombinowana ludzka interleukina 12	Leczenie ostrej choroby popromiennej
Portuguese	Interleucina-12 recombinante humana	Tratamento do sindrome agudo das radiações
Romanian	Interleukina 12 recombinantă umană	Tratamentul sindromului acut de iradiere
Slovak	Rekombinantný ľudský interleukín-12	Liečba akútnehoradiačného syndrómu
Slovenian	rekombinantni humani interlevkin-12	Zdravljenje akutnega radiacijskega sindroma
Spanish	Interleukina 12 recombinante humana	Tratamiento del síndrome de radiación aguda
Swedish	Rekombinant humant interleukin-12	Behandling av akut strålningssyndrom
Norwegian	Rekombinant humant interleukin-12	Behandling av akutt strålesyndrom
Icelandic	Raðbrigða manna interleukín-12	Meðferð bráðrar geislunar heilkenni

[1] At the time of designation