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Public summary of opinion on orphan designation

18-(p-(^{131}I)-Iodophenyl)octadecyl phosphocholine for the treatment of multiple myeloma

On 17 October 2019, orphan designation EU/3/19/2204 was granted by the European Commission to Voisin Consulting S.A.R.L., France, for 18-(p-(^{131}I)-iodophenyl)octadecyl phosphocholine (also known CLR 131) for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma (also called plasma cell myeloma) is a cancer of a type of white blood cell called plasma cells. Plasma cells are produced in the bone marrow, the spongy tissue inside the large bones in the body. In multiple myeloma, the division of plasma cells is uncontrolled, resulting in abnormal, immature plasma cells multiplying and filling up the bone marrow. This interferes with production of normal white blood cells, red blood cells and platelets (components that help the blood to clot), leading to complications such as anaemia (low red blood cell counts), bone pain and fractures, raised blood calcium levels and kidney disease.

Multiple myeloma is a debilitating and life-threatening disease particularly because it disrupts the normal functioning of the bone marrow, damages the bones and causes kidney failure.

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

At the time of designation, multiple myeloma affected approximately 4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 207,000 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).

What treatments are available?

At the time of designation, several medicines were authorised for multiple myeloma in the EU. The main treatment for multiple myeloma was chemotherapy (medicines to treat cancer) usually combined with corticosteroids to reduce the activity of the immune system, the body's natural defences. After

*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
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chemotherapy, patients received stem-cell transplantation if it was appropriate. Stem-cell transplantation is a procedure where the patient's bone marrow is replaced with stem cells to form new bone marrow that produces healthy blood cells.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with multiple myeloma because early studies have found that treatment with the medicine in combination with dexamethasone worked in patients who had received extensive treatment previously. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

The medicine is a type of a phospholipid (a fatty substance), which is attached to a radioactive form of iodine. This type of phospholipid can be found in small amounts in the coating (cell membrane) of body cells, but it is particularly taken up by cancer cells. This means that the medicine builds up in cancer cells and the radioactivity from the radioactive iodine damages and kills the cells. The medicine is therefore expected to reduce the number of abnormal plasma cells and reduce symptoms of the disease.

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, clinical trials with the medicine in patients with multiple myeloma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of multiple myeloma. Orphan designation of the medicine had been granted in the United States for the treatment of multiple myeloma.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 12 September 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	18-(p-(^{131}I)-iodophenyl)octadecyl phosphocholine	Treatment of multiple myeloma
Bulgarian	18-(p-(^{131}I)-йодофенил)октадецил фосфохолин	Лечение на мултиплен миелом
Croatian	18-(p-(^{131}I)-iodofenil)oktadecil fosfoholin	Liječenje multiplog mijeloma
Czech	18-(p-(^{131}I)-jodfenyl)oktadecyl fosfocholin	Léčba mnohočetného myelomu
Danish	18-(p-(^{131}I)-iodophenyl)octadecyl fosphocholine	Behandling af multipelt myelom
Dutch	18-(p-(^{131}I)-iodophenyl)octadecyl phosphocholine	Behandeling van multipel myeloom
Estonian	18-(p-(^{131}I)-jodofenüül)oktadetsüül fosfokoliin	Multiibelse müeloomi ravi
Finnish	18-(p-(131I)-jodofenyyli)oktadesyyli fosfokoliini	Multippeli myelooman hoito
French	18-(p-(^{131}I)-iodophenyl)octadecyl phosphocholine	Traitement du myélome multiple
German	18-(p-(^{131}I)-iodophenyl)octadecyl phosphocholin	Behandlung des multiplen Myeloms
Greek	18-(p-(^{131}I)-ιοδοφαινυλ)οκταδεκυλ φωσφοχολίνη	Θεραπεία πολλαπλού μυελώματος
Hungarian	18-(p-(^{131}I)-jodofenil)oktadecil foszfokolin	Myeloma multiplex kezelése
Italian	18-(p-(^{131}I)-iodofenil)octadecil fosfocoline	Trattamento del mieloma multiplo
Latvian	18-(p-(^{131}I)-jodofenil)oktadecil fosfoholīns	Multiplās mielomas ārstēšana
Lithuanian	18-(p-(^{131}I)-jodofenil)oktodecil fosfocholinas	Dauginės mielomos gydymas
Maltese	18-(p-(^{131}I)-jodofenil)oktadeċil fosfokolin	Kura tal-mjeloma multipla
Polish	18-(p-(^{131}I)-jodofenylo)oktadecylo fosfocholina	Leczenie szpiczaka mnogiego
Portuguese	18-(*p*-(^{131}I)-iodofenill)octadecil fosfatidilcolina	Tratamento do mieloma múltiplo
Romanian	18-(p-(^{131}I)-iodofenil)octadecil fosfocolină	Tratamentul mielomului multiplu
Slovak	18-(p-(^{131}I)-jódofenyl)oktadecyl fosfocholín	Liečba mnohopočetného myelómu
Slovenian	18-(p-(^{131}I)-jodofenil)oktadecil fosfoholin	Zdravljenje multiplega mieloma
Spanish	fosfocholina 18-(p-(^{131}I)-iodofenil)octadecil	Tratamiento del mieloma múltiple
Swedish	18-(p-(^{131}I)-iodofenyl)octadecyl fosfocholin	Behandling av multipelt myelom
Norwegian	18-(p-(^{131}I)-jodofenyl)oktadekylfosfokolin	Behandling av myelomatose
Icelandic	18-(p-(^{131}I)-iodophenyl)oktadecyl phosphocholin	Meðferð við mergfrumuæxli

[1] At the time of designation