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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human interleukin-7 for the treatment of progressive multifocal leukoencephalopathy

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 4 July 2012, orphan designation (EU/3/12/1013) was granted by the European Commission to Cytheris SA, France, for recombinant human interleukin-7 for the treatment of progressive multifocal leukoencephalopathy.

The sponsorship was transferred to Inserm-ANRS (Agence Nationale de Recherches sur le Sida et les Hépatites Virales), France, in May 2014.

What is progressive multifocal leukoencephalopathy?

Progressive multifocal leukoencephalopathy (PML) is a rare brain infection caused by a virus called the JC virus. This virus does not cause disease in the general population but only leads to PML in people with a weakened immune system (the body's natural defences), such as HIV-infected patients. In PML the virus has spread to the brain where it causes damage to the protective sheath surrounding nerves, causing symptoms such as progressive weakness, clumsiness, visual problems, difficulty speaking and decline in mental abilities.

PML is a long-term debilitating and life-threatening disease because of severe damage to motor (movement) and cognitive (mental) functions.

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

At the time of designation, PML affected approximately 0.04 in 10,000 people in the European Union (EU). This was equivalent to a total of around 2,000 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, no satisfactory methods were authorised for the treatment of PML. Management of PML patients varied depending on the cause of the weakened immune system and consisted of strengthening the immune system.

How is this medicine expected to work?

This medicine is similar to a messenger molecule in the body called interleukin-7. Interleukin-7 is involved in the production of T cells, cells in the immune system that help to fight infections. This medicine is expected to stimulate the production of T cells, thereby helping the immune system to fight against the JC virus.

This medicine is made by a method known as 'recombinant DNA technology': it is made by a cell that has received a gene (DNA) that makes it able to produce interleukin-7.

What is the stage of development of this medicine?

The effects of recombinant human interleukin-7 have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with the medicine in patients with PML was planned.

At the time of submission, the medicine was not authorised anywhere in the EU for PML 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 11 May 2012 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.

*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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).

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:

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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-7	Treatment of progressive multifocal leukoencephalopathy
Bulgarian	Рекомбинантен човешки интерлевкин 7	Лечение на прогресивна мултифокална левкоенцефалопатия
Croatian	Rekombinantni ljudski interleukin-7	Liječenje progresivne multifokalne leukoencefalopatije
Czech	Rekombinantní lidský interleukin-7	Léčba progresivní multifokální leukoencefalopatie
Danish	Rekombinant humant interleukin 7	Behandling af progressiv multifokal leukoencefalopati
Dutch	Recombinant humaan interleukine-7	Behandeling van progressief multifocale leukoencefalopathie
Estonian	Rekombinantne inimese interleukiin-7	Progresseeruva multifokaalse leukoentsefalopaatia ravi
Finnish	Rekombinanttitekniikalla tehty ihmisen interleukiini 7	Etenevän monipesäkkeisen leukoenkefalopatian hoito
French	Interleukine-7 humaine recombinante	Traitement de la leucoencephalopathie multifocale progressive
German	Rekombinantes humanes Interleukin-7	Behandlung der progressiven multifokalen Leukenzephalopathie (PML)
Greek	Ανασυνδυασμένη ανθρώπινη ιντερλευκίνη-7	Θεραπεία της προοδευτικής πολυεστιακής λευκοεγκεφαλοπάθειας
Hungarian	Rekombináns humán interleukin-7	Progresszív multifokális leukoenkefalopátia kezelése
Italian	Interleuchina-7 umana ricombinante	Trattamento della leucoencefalopatia multifocale progressiva
Latvian	Rekombinēts cilvēka interleikīns-7	Progresējošas multifokālas leikoencefalopātijas ārstēsana
Lithuanian	Rekombinantinis žmogaus interleukinas - 7	Progresuojančios daugiažidininės leukoencefalopatijos gydymas
Maltese	Interleukin 7 uman rikombinanti	Kura tal-lewkoenċefalopatija multifokali progressiva
Polish	Rekombinowana ludzka interleukina 7	Leczenie postępującej leukoencefalopatii wieloogniskowej
Portuguese	Interleucina-7 recombinante humana	Tratamento da leucoencefalopatia multifocal progressiva.
Romanian	Interleukina 7 recombinantă umană	Tratamentul leucoencefalopatiei multifocale progresive
Slovak	Rekombinantný ľudský interleukín-7	Liečba progresívnej multifokálnej leukoencefalopatie
Slovenian	rekombinantni humani interlevkin-7	Zdravljenje progresivne multifokalne levkoencefalopatije

[1] At the time of transfer of sponsorship

Language	Active ingredient	Indication
Spanish	Interleukina 7 recombinante humana	Tratamiento de la leucoencefalopatía multifocal progresiva
Swedish	Rekombinant humant interleukin-7	Behandling av progressiv multifokal leukoencefalopati
Norwegian	Rekombinant humant interleukin-7	Behandling av progressiv multifokal leukoencefalopati
Icelandic	Raðbrigða manna interleukín-7	Meðferð við fjölhreiðra hvítavefsheilabólgu