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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human MHC non-restricted cytotoxic T cell line for the treatment of ovarian cancer

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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 30 November 2009, orphan designation (EU/3/09/696) was granted by the European Commission to Abiogen Pharma S.p.A., Italy, for human MHC non-restricted cytotoxic T-cell line for the treatment of ovarian cancer.

The sponsorship was transferred to Galileo Research S.r.l., Italy, in March 2013.

What is ovarian cancer?

Ovarian cancer is cancer of the ovaries (two organs in the female reproductive system that produce eggs). Most ovarian cancers occur in women over the age of 50 years. Due to the absence of symptoms in the early stages of the disease, the majority of patients are diagnosed when the cancer has started to spread to other parts of the body.

Ovarian cancer is a life-threatening disease that is associated with poor long-term survival.

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

At the time of designation, ovarian cancer affected approximately 3 in 10,000 people in the European Union (EU). This is equivalent to a total of around 151,000 people*, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and 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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 504,800,000 (Eurostat 2009).

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What treatments are available?

At the time of designation, several medicines were authorised in the EU for the treatment of ovarian cancer. The choice of treatment depended mainly on the stage of the disease. Treatments included surgery and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that human MHC non-restricted cytotoxic T-cell line might be of significant benefit for patients with ovarian cancer because early studies indicate that it might improve the treatment of patients with this condition. 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?

Human MHC non-restricted cytotoxic T-cell line is an advanced therapy medicine that belongs to the group called 'somatic cell therapy products'. These are medicines that contain cells or tissues that have been manipulated so that they can be used to cure, diagnose or prevent a disease.

Human MHC non-restricted cytotoxic T-cell line is made up of a type of cells called cytotoxic T cells that are grown in the laboratory. Cytotoxic T cells form part of the immune system (the body's natural defences) and work by attacking diseased or infected cells in the body. In this medicine, the cells have been manipulated so that they are able to kill cancer cells. They also have the ability to discriminate between cancer cells and normal cells. This medicine is expected to kill the target cancer cells in different ways, including releasing cytotoxic (cell-killing) substances, activating the cell's processes that lead to it dying and stimulating the immune system to attack the cancer cells.

What is the stage of development of this medicine?

The effects of human MHC non-restricted cytotoxic T-cell line have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the product in patients with ovarian cancer were ongoing.

At the time of submission, human MHC non-restricted cytotoxic T-cell line was not authorised anywhere in the EU for ovarian cancer 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 7 October 2009 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:

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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	Human MHC non-restricted cytotoxic T-cell line	Treatment of ovarian cancer
Bulgarian	Човешки ГКТС нерестриктирана цитотоксична Т-клетъчна линия	Лечение на рак на яйчниците
Czech	Buněčné linie T buněk bez restrikce humánního MHC	Léčba karcinomu vaječníků
Danish	Human MHC-uafhængig cytotoksisk T-cellelinje	Behandling af ovarie cancer
Dutch	Humane cytotoxische T-cellijn zonder MHC restrictie	Behandeling van ovariumkanker
Estonian	Inimese peamise koesobivuskompleksist (MHC) sõltumatu tsütotoksilise T raku liin	Munasarjavähi ravi
Finnish	Ihmisen MHC- rajoittumaton sytotoksinen T-solulinja	Munasarjasyövän hoito
French	Lignée de lymphocytes T cytotoxiques humains non restreints par le CMH	Traitement du cancer de l'ovaire
German	Humane Zelllinie zytotoxischer T-Lymphozyten ohne MHC-Restriktion	Behandlung des Ovarialkarzinoms
Greek	Ανθρώπινη κυτταροτοξική Τ-κυτταρική σειρά χωρίς περιορισμό MHC	Θεραπεία του καρκίνου των ωοθηκών
Hungarian	Nem-MHC-korlátozott citotoxikus humán T-sejtvonal	Petefészekrák kezelése
Italian	Linea cellulare di linfociti T citotossici umani MHC non-ristretti	Trattamento del carcinoma dell'ovaio
Latvian	Cilvēka MHC neierobežota citotoksisko T šūnu līnija	Olnīcu vēža ārstēšanai
Lithuanian	Žmogaus MHC neapribotos citotoksinių T limfocitų linijos	Kiaušidžių vėžio gydymas
Maltese	Linja ta' ċelluli T umani, ċitotossiċi u mhux ristretti mill-MHC	Kura tal-kanċer ta' l-ovarji
Polish	Linia ludzkich nieograniczonych MHC limfocytów T-cytotoksycznych	Leczenie raka jajnika
Portuguese	Linha celular humana de linfócitos T citotóxicos sem restrição ao MHC	Tratamento do carcinoma do ovário
Romanian	Linie celulară de limfocite T citotoxice nerestricţionate de CMH uman	Tratamentul cancerului ovarian
Slovak	Ľudské cytotoxické T-bunky bez MHC reštrikcie	Liečba rakoviny vaječníkov
Slovenian	Človeška MHC neomejena citotoksična T-celična linija	Zdravljenje raka na jajčnikih

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Línea de células T citotóxicas humanas no restringidas para el complejo mayor de histocompatibilidad (CMH)	Tratamiento del cáncer de ovario
Swedish	Human MHC-oreglerad cytotoxisk T-cellinje	Behandling av ovarialcancer
Norwegian	Human MHC ikke-avgrenset cytotoksisk t-cellelinje	Behandling av eggstokkreft
Icelandic	Ótakmörkuð manna MHC dráps T-frumulína	Meðferð eggjastokkakrabbameins