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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF

HLA class I/II binding tumour associated peptides (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS) for the treatment of renal cell carcinoma

On 15 February 2007, orphan designation (EU/3/07/433) was granted by the European Commission to Immatics Biotechnologies GmbH, Germany, for HLA class I/II binding tumour associated peptides (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS) for the treatment of renal cell carcinoma.

What is renal cell carcinoma?

Renal cell carcinoma (also called cancer of the kidney or renal adenocarcinoma) is a disease in which cancer (malignant) cells are found in certain tissues of the kidney. Inside each kidney there are tiny tubules that filter and clean the blood, taking out waste products, and making urine. Renal cell carcinoma is a cancer of the lining of the tubules in the kidney. Renal cell carcinoma accounts for approximately 85% of all kidney cancers. Signs of cancer are difficult to detect in early stages of the disease, and about half of the patients are diagnosed when the disease has spread around the kidney or to distant parts of the body. Renal cell carcinoma is life-threatening.

What are the methods of treatment available?

There are treatments for most patients with renal cell cancer. These may include surgery (taking out the cancer in an operation), chemotherapy (using drugs to kill cancer cells), radiation therapy (using high-dose x-rays or other high-energy rays to kill cancer cells), hormone therapy (using hormones to stop cancer cells from growing), and biological therapy (using the body's immune system to fight cancer). The primary therapy for advanced renal cell carcinoma is with biologic agents such as interleukin-2 and interferon- α . Recently, other anticancer drugs (sorafenib and sunitinib) aimed at stopping cancer cells growth have also been authorised in the Community for the treatment of renal cell carcinoma.

HLA class I/II binding tumour associated peptides (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS) might be of potential significant benefit for the treatment of renal cell carcinoma because it might improve the long-term outcome of the patients. This assumption will have to be confirmed at the time of marketing authorisation.

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

Based on the information provided by the sponsor and previous knowledge of the Committee, renal cell carcinoma was considered to affect up to 4.2 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 193,000 persons.

* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

How is this medicinal product expected to act?

HLA receptors on the cell surface display peptide fragments derived from internal cellular proteins on the surface of the cell, thus enabling T cells from the immune system to differentiate between healthy cells and diseased cells including virus-infected and tumour cells. HLA class I/II binding tumour associated peptides (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS) are derived from cancer cells. Once administered to the patient, it may increase ability to create an immune response that attacks and destroys tumour cells.

What is the stage of development of this medicinal product?

The effects of HLA class I/II binding tumour associated peptides (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS) were evaluated in experimental models.

At the time of submission of the application for orphan designation, one clinical trial in patients with renal cell carcinoma was completed.

HLA class I/II binding tumour associated peptides (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS) was not authorised anywhere worldwide for treatment of renal cell carcinoma or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 10 January 2007 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

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**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	HLA class I/II binding tumour associated peptides (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Treatment of renal cell carcinoma
Bulgarian	Тумор асоциирани пептиди свързани с HLA class I/II (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Лечение на бъбречно клетъчен карцином
Czech	S nádorem asociované peptidy vázající se na HLA třídu I/II (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Léčba karcinomu ledvin
Danish	HLA klasse I/II bindende tumorassocierede peptider (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Behandling af renalcellekarcinom
Dutch	HLA klassen I/II bindende tumorgeassocieerde peptiden (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Behandeling van niercelcarcinoom
Estonian	HLA I/II klassi siduvad kasvajaga seotud peptiidid (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Neeru vähi-ravi
Finnish	HLA-luokkaan I/II sitoutuvat tuumoriin liittyvät peptidit (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Munuaiskarsinooman hoito
French	Peptides tumoraux se liant aux molécules HLA de classe I/II (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Traitement du carcinome rénal
German	Tumor-assoziierte Peptide mit Bindung an die HLA-Klassen I/II (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Behandlung des Nierenzellkarzinoms
Greek	Πεπτίδια που συσχετίζονται με όγκους και συνδέονται με τις κατηγορίες HLA I/II (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Θεραπεία του νεφροκυτταρικού καρκινώματος
Hungarian	HLA I/II-höz kötődő tumorasszociált peptidek (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Vesekarcinoma kezelése
Italian	Peptidi tumore-associati leganti gli HLA di classe I/II (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Trattamento del carcinoma renale
Latvian	HLA klases I/II saistošie audzēja asociētie peptīdi (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Nieru karcinomas ārstēšana
Lithuanian	Prie I/II klasės ŽLA antigeno, besijungiantys su vėžiu susiję peptidai (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Inkstų adenokarcinomos gydymas
Polish	Peptydy nowotworowe HLA klasy I/II towarzyszące wiązaniom (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Leczenie raka nerki

Portuguese	Peptídeos associados ao tumor com capacidade de ligação a HLA classe I/II (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Tratamento do carcinoma das células renais
Romanian	Peptide asociate tumorii care se leagă de HLA clasele I/II (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS)	Tratamentul carcinomului renal
Slovak	S tumorom asociované peptidy s väzbou na HLA triedu I/II (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS)	Liečba karcinómu obličky
Slovenian	S tumorji asociirani peptidi z vezavo na HLA razredov I/II (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS)	Zdravljenje raka ledvičnih celic
Spanish	Péptidos asociados al tumor con capacidad de unión a HLA clases I/II (ADF-APO-CCN-GUC-K67-MET-MMP-MUC-RGS)	Tratamiento del carcinoma de células renales
Swedish	HLA klass I/II-bindande tumörassocierade peptider (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Behandling av njurcellscancer
Norwegian	HLA-klasse I/II bindende tumorassosierte peptider (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Behandling av nyrecellekarsinom
Icelandic	HLA af flokki I/II sem binda æxlistengd peptíð (ADF-APO-CCN-GUC-K67-MET- MMP-MUC-RGS)	Meðferð á nýrnafrumukrabbameini