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EMA/COMP/25490/2006 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

26 base single stranded phosphodiester DNA oligonucleotide for the treatment of renal cell carcinoma

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in March 2011 on request of the sponsor.

On 16 February 2006, orphan designation (EU/3/06/353) was granted by the European Commission to Antisoma Research Ltd, United Kingdom, for 26 base single stranded phosphodiester DNA oligonucleotide 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 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 is the estimated number of patients affected by the condition?

At the time of designation, renal cell carcinoma affected approximately 3.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 161,000 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 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

What treatments are available?

There are treatments for most patients with renal cell carcinoma. 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 therapies for advanced cancer are biologic agents, such as interleukin-2 and interferon-alpha. Other anticancer agents had also been authorised in the Community for treatment of renal cell carcinoma at the time of submission of the application for orphan designation.

26 base single stranded phosphodiester DNA oligonucleotide might be of potential significant benefit for the treatment of renal cell carcinoma because it might act in a different way and thereby improve the long-term outcome of the patients. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Normal cells have proteins in their membranes (the outer sheet covering the whole cell and the thin envelop of the cell nucleus) that act as receptors. These receptors, after having interacted with different substances, then trigger some functions of the cell, like growth, migration, etc. These receptors are frequently altered in cancer cells, either in localisation, number or functional characteristics. 26 base single stranded phosphodiester DNA oligonucleotide seems to bind to a receptor that normally is present in the nuclear membrane only, but that in cancer cells is also present in the outer cell membrane. It is expected that when the product binds to the receptor on the tumour cell outer membrane, it might activate a series of events that eventually will kill the cancer cell by a process called apoptosis. By doing this, the product is expected to kill cancer cells.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with renal cell carcinoma were ongoing.

26 base single stranded phosphodiester DNA oligonucleotide was not authorised anywhere worldwide for renal cell carcinoma, at the time of submission. Orphan designation of 26 base single stranded phosphodiester DNA oligonucleotide was granted in the United States for treatment of renal cell carcinoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 January 2006 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:

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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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	26 base single stranded phosphodiester DNA oligonucleotide	Treatment of renal cell carcinoma
Czech	Jednořetězcový fosfodiesterový DNA oligonukleotid o 26 bázích	Léčba karcinomu ledvin
Danish	Enkeltstrenget phosphodiester-DNA-oligonukleotid med 26 baser	Behandling af renalcellekarcinom
Dutch	26 base enkelstrandig fosfodiëster DNA oligonucleotide	Behandeling van niercelcarcinoom
Estonian	26 alusega üheaheelalise fosfodiester-DNA oligonukleotiid	Neeru vähi-ravi
Finnish	26-emäksinen yksijuosteinen fosfodiesteri-DNA-oligonukleotidi	Munuaiskarsinooman hoito
French	Oligonucléotide ADN à monobrin phosphodiester de 26 bases	Traitement du carcinome rénal
German	26-Basen-, Einzelstrang-, Phosphodiester-, DNA-Oligonukleotid	Behandlung des Nierenzellkarzinoms
Greek	Φωσφοδιεστεράση μονής ολιγονουκλεοτιδικής DNA αλύσου 26 βάσεων	Θεραπεία του νεφροκυτταρικού καρκινώματος
Hungarian	26 bázisból álló, egyes szálú foszfodiészter DNS oligonukleotid	Vesekearcinoma kezelése
Italian	Oligonucleotide fosfodiesteri del DNA a catena singola a 26 basi	Trattamento del carcinoma renale
Latvian	26 bāzu vienpavediena fosfodiesterā DNS oligonukleotīds	Nieru karcinomas ārstēšana
Lithuanian	26 bazių DNR oligonukleotidas su viena fosfodiesterio jungtimi	Inkstų adenokarcinomos gydymas
Polish	26 zasadowy oligonukleotyd fosfodiestrowy pojedynczego łańcucha DNA	Leczenie raka nerki
Portuguese	Oligonucleótido de DNA fosfodiéster monocatenado com 26 bases	Tratamento do carcinoma das células renais
Slovak	Jednovláknový fosfodiester- oligonukleotid DNA zložený z 26 báz	Liečba karcinómu obličky
Slovenian	26 bazni enoverižni DNK olugonukleotid	zdravljenje raka ledvičnih celic
Spanish	Oligonucleótido ADN fosfodiéster monocatenario compuesto por 26 bases	Tratamiento de carcinoma de células renales
Swedish	26-bas enkelsträngad fosfordiester-DNA-oligonukleotid	Behandling av njurcellscancer
Norwegian	26-base enkelttrådig fosfodiester DNA-oligonukleotid	Behandling av nyrecellekarsinom
Icelandic	26 basa einþátta fosfótvíester DNA-óligónúkleótíð	Meðferð á nýrnafrumukrabbameini

¹ At the time of designation