The European Agency for the Evaluation of Medicinal Products
Pre-authorisation Evaluation of Medicines for Human Use

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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2003 on request from the sponsor.

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF humanized anti-KSA monoclonal antibody-human interleukin-2 fusion protein for the treatment of renal cell carcinoma

On 22 March 2002, orphan designation (EU/3/02/097) was granted by the European Commission to Merck KgaA, Germany, for the humanized anti-KSA monoclonal antibody-human interleukin-2 fusion protein for the treatment of renal cell carcinoma.

What is renal cell carcinoma?
Tumours that begin in the kidneys are known as renal tumours. When these tumours have certain characteristics, for example, originate from certain cells, grow rapidly and tend to infiltrate surrounding healthy tissues, they are called renal cell carcinoma. Renal cell carcinoma accounts for approximately 85% of all kidney cancers. Due to the absence of symptoms in early stages of the disease approximately half of the patients are diagnosed when tumours have spread locally or to distant parts of the body. Renal cell carcinoma is life-threatening.

What are the methods of treatment available?
Renal cell carcinoma is not very responsive to chemotherapy. The primary therapies for advanced cancer are biological agents, such as interleukin-2 (IL-2) and interferon-α (IFN-α). Other anti-cancer 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. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that humanized anti-KSA monoclonal antibody-human interleukin-2 fusion protein might be of potential significant benefit for the treatment of renal cell carcinoma, particularly in terms of its novel mechanism of action.

What is the estimated number of patients affected by the condition*?
According to the information provided by the sponsor, renal cell carcinoma was considered to affect about 117,000 persons in the European Union.

How is this medicinal product expected to act?
The humanized anti-KSA monoclonal antibody-human IL-2 fusion protein is composed of two parts: an antibody anti-KSA linked to IL-2. The resulting medicinal product reacts with an antigen expressed on the surface of renal carcinoma cells: the human adenocarcinoma–associated antigen (or KSA). After fixation on the cancer cell, IL-2 provides a local signal for generation of T-lymphocytes which are expected to recognise and kill tumour cells.

What is the stage of development of this medicinal product?
The effects of this medicinal product have been studied in experimental models of carcinomas At the time of submission of the application for orphan designation, clinical trials were ongoing.

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The humanized anti-KSA monoclonal antibody-human IL-2 fusion protein had not been marketed anywhere worldwide or designated as an 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 23 January 2002 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 have been 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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*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.