European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF iodine (^{131}I) iobenguane for the treatment of neuroblastoma

On 31 January 2008, orphan designation EU/3/07/525 was granted by the European Commission to Molecular Insight Limited, United Kingdom, for iodine (^{131}I) iobenguane for the treatment of neuroblastoma.

What is neuroblastoma?

Neuroblastoma is a life-threatening condition which belongs to a wide group of tumours called neuroendocrine tumours. Neuroblastoma is the most common malignant intra-abdominal tumour in children. Neuroblastoma is often present at birth but is most often diagnosed later when the child begins to show symptoms of the disease. Neuroblastoma manifests as a lump or a mass in the abdomen or around the spinal cord.

What are the methods of treatment available?

At the time of submission of the application for orphan drug designation, removal of the tumour by surgery and radiotherapy was used. Chemotherapy acting both on the tumour and on the metastases was also used. Several products with chemotherapeutic activity were authorised for the condition in some countries in the Community at the time of submission of the application for orphan drug designation.

Iodine (^{131}I) iobenguane may be of potential significant benefit for the treatment of neuroblastoma in particular with regards to improved efficacy. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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, neuroblastoma was considered to affect approximately 1.3 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 65,000 persons in total.

How is this medicinal product expected to act?

Iodine (^{131}I) iobenguane contains a radioactive form of a chemical called Iodine (^{131}I) and a substance called iobenguane. Iobenguane is similar to the naturally occurring stress hormone noradrenaline and it can specifically be taken up and accumulated by tissues originating in embryonic neural crest cells, such as neuroblastoma tumours. The product is expected to bind to the tumour cells and to kill them with the radiation from the iodine component. The product is a new formulation.

*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 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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What is the stage of development of this medicinal product?
The effects of iodine (^{131}I) iobenguane were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with neuroblastoma were initiated.

Iodine (^{131}I) iobenguane was not authorised anywhere worldwide for the treatment of neuroblastoma, at the time of submission.

Orphan designation of iodine (^{131}I) iobenguane was granted in the United States for treatment of neuroendocrine tumours.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 5 December 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.

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

Language	Active Ingredient	Indication
English	Iodine (^{131}I) iobenguane	Treatment of neuroblastoma
Bulgarian	йод (^{131}I)йобенгуан	Лечение на невробластом
Czech	Jod (^{131}I) Jobenguan	Léčba neuroblastomu
Danish	Iod (^{131}I)-iobenguan	Behandling af neuroblastom
Dutch	Iodine (^{131}I) iobenguane	Behandeling van neuroblastoom
Estonian	Jood (^{131}I) jobenguaan	Neuroblastoomi ravi
Finnish	Jodi(^{131}I)jobenguani	Neuroblastooman hoito
French	Iobenguane-^{131}I	Traitement du neuroblastome
German	Iod (^{131}I) iobenguan	Behandlung des Neuroblastoms
Greek	ιωβενγουάνη 131 I	Θεραπεία Νευροβλάστωματος
Hungarian	^{131}J-jobenguan	Neuroblastoma kezelése
Italian	Iobenguano-^{131}I	Trattamento del neuroblastoma
Latvian	Joda (^{131}I) jodbenguāns	Neiroblastomas ārstēšana
Lithuanian	Jodo (^{131}I) jobenguanas	Neuroblastomos gydymas
Maltese	Iodine (^{131}I) iobenguane	Kura tan-newroblastoma
Polish	Jodowana (^{131}I) jobenguan	Leczenie nerwiaka płodowego
Portuguese	Iobenguano com Iodo (^{131}I)	Tratamento do neuroblastoma
Romanian	Iod (^{131}I) iobenguan	Tratamentul neuroblastomului
Slovak	Jód (^{131}I) jobenguán	Liečba neuroblastómu
Slovenian	Jod (131I) jobengvan 131 I	Zdravljenje nevroendokrinega nevroblastoma
Spanish	Iobenguano marcado con yodo (131 I)	Tratamiento del neuroblastoma
Swedish	Jod (131I) jobenguan	Behandling av neuroblastom
Norwegian	Jod (131I) Iobenguan	Behandling av neuroblastom
Icelandic	Joð (131I) jóbengúan	Meðferð við taugakímfrumuæxli