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EMA/COMP/733185/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Heat-killed *Mycobacterium obuense* (whole cell) for the treatment of pancreatic cancer

On 16 December 2014, orphan designation (EU/3/14/1385) was granted by the European Commission to Immodulon Therapeutics Ltd, United Kingdom, for heat-killed *Mycobacterium obuense* (whole cell) for the treatment of pancreatic cancer.

What is pancreatic cancer?

Pancreatic cancer is cancer of the pancreas, a small organ that lies behind the stomach. The pancreas has two functions: to produce a fluid that helps with the digestion of food, and to produce hormones such as insulin. Due to the absence of symptoms in the early stages of pancreatic cancer, the majority of patients are diagnosed when the cancer has spread locally or to other parts of the body.

Pancreatic cancer is a very severe and life-threatening disease that is associated with shortened life expectancy.

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

At the time of designation, pancreatic cancer affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 102,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, several medicines were authorised in the EU for treating pancreatic cancer. The choice of treatment depended on several factors, including how far the disease had advanced. Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 511,100,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with pancreatic cancer because early results show improved responses in patients when the medicine was added to currently authorised treatments. 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?

In the early stages of cancer, the body's immune system (its natural defences) can often combat the growth and spread of tumour cells, but over time the immune system may become less effective in controlling the cancer, allowing it to grow. This medicine contains a species of bacteria called *Mycobacterium obuense* (NCTC 13365) that have been killed by heating so they can no longer grow or cause infection. When the medicine is injected, the body's immune system is activated to become more effective, because the bacteria are considered a possible new threat. This activation of the immune system is expected to help the body also combat the cancer more effectively.

What is the stage of development of this medicine?

The effects of heat-killed *Mycobacterium obuense* (whole cell) have been evaluated in experimental models.

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

At the time of submission, heat-killed *Mycobacterium obuense* (whole cell) was not authorised anywhere in the EU for pancreatic cancer. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 November 2014 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:

Immodulon Therapeutics Ltd
22 Devonshire Place - B2
London W1G 6JA
United Kingdom
Tel. +44 (0)20 3219 3568
Fax +44 (0)20 3219 3574
E-mail: IMAGE1@immodulon.com

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	Heat-killed *Mycobacterium obuense* (whole cell)	Treatment of pancreatic cancer
Bulgarian	Термично-убит *Mycobacterium obuense* (цели клетки)	Лечение на рак на панкреаса
Croatian	Toplinom usmrćene *Mycobacterium obuense (*cijela stanica)	Liječenje raka gušterače
Czech	Teplem usmrcené *Mycobacterium obuense* (celobuněčná)	Léčba karcinomu pankreatu
Danish	Varmedræbte *Mycobacterium obuense* (helcelle)	Behandling af pancreascancer
Dutch	Hitte geïnactiveerd *Mycobacterium obuense* (complete cel)	Behandeling van pancreaskanker
Estonian	Kuumsurmatud *Mycobacterium obuense* (täisrakuline)	Pankreasevähi ravi
Finnish	Lämpökäsittelyllä tuhottu *Mycobacterium obuense* (kokosolu)	Haimasyövän hoito
French	*Mycobacterium obuense* inactivé par la chaleur (germes entiers),	Traitement du cancer pancréatique
German	Hitze-inaktiviertes *Mycobacterium obuense* (Ganzzellen)	Behandlung des Pankreaskarzinoms
Greek	Μυκοβακτήριο *obuense* που έχει θανατωθεί με θερμότητα (ολοκυτταρικό)	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Hőkezeléssel elölt *Mycobacterium obuense* (teljes sejtes)	Hasnyálmirigyrák kezelése
Italian	*Mycobacterium obuense* ucciso col calore (cellula intera)	Trattamento del cancro pancreatico
Latvian	Ar karstumu iznīcināta *Mycobacterium obuense* (visa šūna)	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Karščiu užmušama *Mycobacterium obuense* (vientisa ląstelė)	Kasos vėžio gydymas
Maltese	*Mycobacterium obuense* maqtul bis-sħana (ċellula sħiħa)	Kura tal-kanċer tal-frixa
Polish	Bakterie *Mycobacterium obuense* (całe komórki) inaktywowane termicznie	Leczenie raka trzustki
Portuguese	*Mycobacterium obuense* inactivada pelo calor (célula completa)	Tratamento do carcinoma do pâncreas
Romanian	*Mycobacterium obuense* inactivatetermic (germeni întregi)	Tratamentul cancerului pancreatic
Slovak	Teplom inaktivované *Mycobacterium obuense* (celá bunka)	Liečba rakoviny pankreasu
Slovenian	Toplotno inaktivirana *Mycobacterium*	Zdravljenje raka trebušne slinavke

[1] At the time of designation

Language	Active ingredient	Indication
	obuense (celotna celica)	
Spanish	*Mycobacterium obuense* termoinactivado (de célula entera)	Tratamiento del cáncer de páncreas
Swedish	Värmeinaktiverad *Mycobacterium obuense* (helcells)	Behandling av pankreascancer
Norwegian	Varmeinaktiverte *Mycobacterium obuense* (hele celler)	Behandling av pankreascancer
Icelandic	Hitadeydd *Mycobacterium obuense* (heilfrumu)	Meðferð briskrabbameins