3 July 2013
EMA/COMP/317369/2005 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant modified vaccinia virus Ankara expressing tuberculosis antigen 85A for the prevention of tuberculosis disease in BCG vaccinated individuals

First publication	13 March 2006
Rev.1: transfer of sponsorship	27 August 2009
Rev.2: sponsor's change of address	18 June 2013
Rev.3: transfer of sponsorship	3 July 2013

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 28 October 2005, orphan designation (EU/3/05/318) was granted by the European Commission to University of Oxford, United Kingdom, for recombinant modified vaccinia virus Ankara expressing tuberculosis antigen 85A for the prevention of tuberculosis disease in BCG vaccinated individuals.

The sponsorship was transferred to Emergent Product Development UK Limited, United Kingdom, in February 2009 and subsequently back to University of Oxford, United Kingdom, in June 2013.

What is tuberculosis?

Tuberculosis (TB) is an infection caused by a group of bacteria called *Mycobacteria*. It spreads from person-to-person by inhaling the infected airborne droplets generated by sneezing and coughing. The manifestation of the disease is variable and not all patients who are infected will develop the disease. The disease is characterised by fever, cough and breathing difficulties. Granulomas (accumulation of a large number of cells leading to chronic inflammatory lesions) can develop in any body tissue by the formation of tubercles. TB most commonly affects the lungs (pulmonary TB) but can also affect the central nervous system (meningitis), lymphatic system, genitourinary system, bones and joints. Tuberculosis is a life-threatening condition.

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What methods of prevention?

The Bacille Calmette-Guerin (BCG) vaccine was authorised for prevention of tuberculosis in the Community, at the time of submission of the application for orphan designation. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that recombinant modified vaccinia virus Ankara expressing tuberculosis antigen 85A might be of potential significant benefit for the prevention of tuberculosis as it is designed to enhance (boost) the effect of the BCG vaccine. This assumption of benefit will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

What is the potential for return on investment?

Usually applications for Orphan Designation are made on the grounds of the rarity of the disease. However, an alternative criterion based on the expected return on investment also exists.

According to the information provided by the sponsor, considering the estimated total discovery and development costs of bringing recombinant modified vaccinia virus Ankara expressing tuberculosis antigen 85A to the market, and the expected revenue that will be generated from the sales of the vaccine in the Community, it is unlikely that sufficient return will be generated to justify the necessary investment.

How is this medicine expected to work?

BCG is a vaccine used for tuberculosis prevention. Administering BCG exposes the body's defence system (immune system) to a microorganism similar to the *Mycobacterium*, aimed at building an immune response against the infection. Recombinant modified vaccinia virus Ankara expressing tuberculosis antigen 85A is a vaccine that has been developed to boost the body immune response following BCG vaccination. The vaccine is expected to boost the individual's immune system against the *Mycobacterium* and thereby might improve the body's protection against the development of the disease.

What is the stage of development of this medicine?

The evaluation of the effects of recombinant modified vaccinia virus Ankara expressing tuberculosis antigen 85A in experimental models is ongoing.

At the time of submission of the application for orphan designation, clinical trials in BCG vaccinated individuals were ongoing.

Recombinant modified vaccinia virus Ankara expressing tuberculosis antigen 85A was not authorised anywhere worldwide for prevention of tuberculosis disease in BCG vaccinated individuals or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 September 2005 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:

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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 languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Recombinant modified vaccinia virus Ankara expressing tuberculosis antigen 85A	Prevention of tuberculosis disease in BCG vaccinated individuals
Bulgarian	Рекомбинантен модифициран vaccinia вирус Ankara експресиращ туберкулозен антиген 85A	Превенция на туберкулозна болест у BCG ваксинирани индивиди
Czech	Rekombinantní modifikovaná tuberkulozní vakcina viru Ankara exprimující antigen 85A	Prevence tuberkulózy u BCG očkovaných jedinců
Danish	Rekombinant modificeret vaccinia virus Ankara tuberculose antigen 85A	Forebyggelse af tuberkuløs sygdom hos individer, der er BCG vaccinerede
Dutch	Recombinant gemodifiëerd vaccinia virus Ankara welke het tuberculosis antigen 85A uitdrukt	Preventie van tuberculosis aandoening in BCG gevaccineerden
Estonian	Rekombinantne modifitseeritud vaktsiinia viirus Ankara, mis ekspresseerib tuberkuloosi antigeeni 85A	Tuberkulooshaiguse preventsioon BCG-vaktsineeritud indiviididel
Finnish	Rekombinantti modifioitu vaccinia virus Ankara, joka ekspressoi tuberkuloosiantigeeni 85A:ta	Tuberkuloosisairauden ehkäiseminen BCG-rokotetuilla henkilöillä
French	Vaccin recombinant du virus Ankara exprimant l'antigène tuberculinique 85A	Prévention de la tuberculose chez les individus vaccinés par le BCG
German	Rekombinant modifizierter Ankaravirus welcher das Tuberkuloseantigen 85A exprimiert	Tuberkuloseprävention in BCG-geimpften Individuen
Greek	Ανασυνδυασμένος τροποποιημένος ιό δαμαλίτιδας τύπου Ankara που εκφράζει το αντιγόνο της φυματίωσης 85A	Πρόληψη της φυματίωσης σε άτομα εμβολιασμένα με BCG
Hungarian	85A tuberculosis antigént expresszáló rekombináns módosított Ankara-vírus vakcina	Tuberculosis prevenció BCG oltott egyénekben

[1] At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Italian	Vaccino ricombinante derivato dal vaccino virus del vaiolo Ankara modificato a esprimere l'antigene tubercolare 85A	Prevenzione della tubercolosi in soggetti vaccinati con BCG
Latvian	Rekombinēta modificēta vaccinia vīrusa Ankāra ekspresēts tuberkulozes antigēns 85°	Tuberkulozes profilakse ar BCG vakcinētiem indivīdiem
Lithuanian	Rekombinantinė modifikuota karvių raupų viruso Ankara vakcina, ekspresuojanti tuberkuliozės antigeną 85A	Tuberkuliozės, vakcinuotiems BCŽ vakcina individams, prevencija
Maltese	Virus tat-tilqim kontra l-ġidri Ankara rikombinanti, modifikat biex jesprimi l-antiġene tat-tuberkuloži 85A	Prevenzjoni tal-marda tat-tuberkuloži f'persuni imlaqqmin bil-BCG
Polish	Rekombinowany modyfikowany, szczepionkowy wirus Ankara z ekspresją antygenu tuberkulinowego 85A	Zapobieganie gruźlicy u osób szczepionych BCG
Portuguese	Vacina recombinante do vírus Ankara com antigénio 85 A da tuberculose	Prevenção da tuberculose em indivíduos vacinados com BCG
Romanian	Virus vaccinal Ankara recombinant modificat care exprimă antigenul 85A al tuberculozei	Prevenţia tuberculozei la pacienţii vaccinaţi BCG
Slovak	Rekombinantný modifikovaný vakcínia vírus Ankara exprimujúci tuberkulózový antigén 85A	Prevencia tuberkulózy u osôb po BCG vakcinácii
Slovenian	Rekombinantno modificiran virus vakcijije Ankara z izraženim antigenom 85A bacila tuberkuloze	Preprečevanje tuberkuloze pri osebah cepljenih z BCG
Spanish	Virus Ankara de la vacuna recombinante modificado que expresa el anfígeno 85A de la tuberculosis	Prevención de la tuberculosis en individuos vacunados con BCG
Swedish	Rekombinant modifierad vaccinia virus Ankara som uttrycker tuberkulos antigen 85A	Profylax av tuberkulos sjukdom hos BCG vaccinerade individer
Norwegian	Rekombinant modifisert vaccinia virus Ankara som uttrykker tuberkulose antigen 85A	Forebygging av tuberkuløs sykdom hos BCG-vaksinerte personer

Language	Active Ingredient	Indication
Icelandic	Raðbrigða aðlöguð kúabólu veira Ankara, sem tjáir berkla mótefnavaka 85A	Til að fyrirbyggja berkla hjá einstaklingum sem hafa verið bólusettir með BCG