



EMA/COMP/170034/2008 Rev. 3  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Human papilloma virus type 16 E6/E7 synthetic long peptides for the treatment of epithelial neoplasia of the vulva positive for human papilloma virus

First publication	29 July 2008
Rev.1: sponsor's change of address	20 August 2009
Rev.2: sponsor's name and address change	16 December 2011
Rev.3: sponsor's change of address	18 June 2013
<b>Disclaimer</b> 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 20 December 2007, orphan designation (EU/3/07/520) was granted by the European Commission to ISA Pharmaceuticals BV, The Netherlands, for human papilloma virus type 16 E6/E7 synthetic long peptides for the treatment of epithelial neoplasia of the vulva, positive for human papilloma virus.

In November 2011, ISA Pharmaceuticals BV changed name to ISA Therapeutics B.V.

### What is epithelial neoplasia of the vulva positive for human papilloma virus?

An epithelial neoplasia is when cells lining the outer layers of the body (for instance the skin and inside the mouth, intestines and the genitalia) start dividing and growing in an unusual way. An epithelial neoplasia can manifest itself as a wart or a lump, and in some cases it can develop into cancer, if left untreated. A majority of epithelial neoplasias of the vulva (the outer parts of the female genitalia) are caused by an infection of human papilloma virus type 16. Symptoms of the disease include discomfort, itching, irritation, pain and local bleeding. Epithelial neoplasia of the vulva is chronically debilitating and life-threatening due to the associated symptoms and the high risk of developing invasive cancer.



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

At the time of designation, epithelial neoplasia of the vulva positive for human papilloma virus affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 100,000 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?**

There are no medicinal products authorised for the treatment of the condition in the Community. Depending on how advanced the disease is at diagnosis, some patients can be treated surgically with a removal of the abnormal tissue.

## **How is this medicine expected to work?**

Human papilloma virus type 16 E6/E7 synthetic long peptides is a vaccine. A vaccine is a compound that resembles part of a disease-inducing foreign organism, such as a virus or a bacteria, designed to "teach" the body's natural defence system (the immune system) to recognise, in this case, human papilloma virus type 16. E6 and E7 are two protein molecules that are found in cells infected by the virus. According to the sponsor, vaccination with this medicinal product will help induce the immune system to naturally destroy neoplastic epithelial cells in the vulva.

## **What is the stage of development of this medicine?**

The effects of human papilloma virus type 16 E6/E7 synthetic long peptides were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with epithelial neoplasia of the vulva positive for human papilloma virus were ongoing.

Human papilloma virus type 16 E6/E7 synthetic long peptides was not authorised anywhere worldwide for the treatment of epithelial neoplasia of the vulva positive for human papilloma virus nor 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 8 November 2007 recommending the granting of this designation.

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\*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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 500,300,000 (Eurostat 2007).

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<sup>1</sup>, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Human papilloma virus type 16 E6/E7 synthetic long peptides	Treatment of epithelial neoplasia of the vulva positive for human papilloma virus
Bulgarian	Синтетични дълги пептиди на човешки папиломен вирус тип 16 E6/E7	Лечение на епителна неоплазия на вулвата, положителна за човешки папиломен вирус
Czech	Syntetická dlouhých peptidů proti proteinům E6/E7 lidského papilomaviru typu 16	Léčba epitelálních neoplazií vulvy HPV pozitivních
Danish	Syntetiske lange peptider mod human papillomavirus type 16 E6/E7	Behandling af vulval epitelial neoplasier, som er positiv for human papillomavirus
Dutch	Synthetische lange peptiden gericht tegen humaan papilloma virus type 16 E6/E7	Behandeling van epitheliale vulvaire neoplasieën
Estonian	Inimese papilloomiviiruse tüüp 16 proteiinide E6/E7 vastased sünteetilised pikad peptiidid	Inimese papilloomiviirus-positiivse vulva epiteeliaalkasvaja ravi
Finnish	Synteettisiä pitkiä peptidejä ihmisen papilloomaviruksen tyyppiä 16 E6/E7 vastaan	Vulvan ihmisen papilloomavirukselle positiivisen epiteeliaalisen neoplasian hoitoon
French	Longs peptides de synthèse dirigés contre les protéines E6 et E7 du papillomavirus humain de type 16	Traitement des néoplasies épithéliales de la vulve positives pour le papillomavirus humain
German	Synthetische lange Peptide gegen humanes Papillomavirus Typ 16 E6/E7	Behandlung epithelialer Neoplasien der Vulva, die positiv für humanes Papillomavirus sind
Greek	Συνθετικά μακριά πεπτιδία ανθρώπινου Θηλωματοϊού ιού τύπου 16 E6/E7	Θεραπεία των επιθηλιακών νεοπλασιών του αιδοίου θετικών για Ανθρώπινο Θηλωματοϊό ιό
Hungarian	16 E6/E7 típusú humán papillomavírusnak megfelelő hosszú szintetikus peptidek	Humán papillomavírusra pozitív epitelialis vulvárak kezelése
Italian	Peptidi lunghi sintetici delle proteine E6/E7 del virus del papilloma umano di tipo 16	Trattamento delle neoplasie epiteliali vulvari positive per il virus del papilloma umano
Latvian	Sintētiskie cilvēka papilomas vīrusa (tips 16. E6/E7) garie peptīdi	Tādu vulvas epiteļiālo audzēju ārstēšana, kuros atrasts cilvēka papilomas vīruss
Lithuanian	Sintetiniai žmogaus papilomos viruso 16 tipo E6/E7 ilgieji peptidai	Vulvos epitelinių neoplazijų, teigiamų žmogaus papilomos virusui, gydymas

<sup>1</sup> At the time of designation

Language	Active Ingredient	Indication
Maltese	Peptidi sintetiċi tal-proteini E6/E7 tal-virus tal-papilloma uman tat-tip 16	Kura tan-neoplażmi epiteljali tal-vulva pożittivi għall-virus tal-papilloma uman
Polish	Szczepionka przeciwko białkom E6/E7 ludzkiego wirusa brodawczaka typ 16 zawierająca syntetyczne długołańcuchowe peptydy	Leczenie nowotworów nabłonkowych sromu u chorych z pozytywnym wynikiem w kierunku ludzkiego wirusa brodawczaka
Portuguese	Sequência de peptídeos longos contra as proteínas E6/E7 do papilomavirus humano tipo 16	Tratamento das neoplasias epiteliais da vulva positivas para o papilomavirus humano.
Romanian	Peptide cu lanț lung sintetice împotriva proteinelor E6/E7 ale virusului Papiloma uman de tip 16	Tratamentul neoplaziei epiteliale vulvare pozitivă pentru virusul Papiloma uman
Slovak	Syntetický dlhoreťazcový peptid proti ľudskému papilómovému vírusu typu 16 E6/E7	Liečba epitelovej neoplázie vulvy pozitívnej na ľudský papilómový vírus
Slovenian	Sintetični dolgi peptidi proti beljakovinam E6/E7 humanega virusa papiloma tipa 16	Zdravljenje epitelijske neoplazije vulve pri pozitivnem humanem virusu papiloma
Spanish	Péptidos largos contra las proteínas E6/E7 del virus del papiloma humano tipo 16	Tratamiento de las neoplasias epiteliales vulvares positivas para virus del papiloma humano
Swedish	Långa peptider mot mänskligt papillomvirus typ 16 E6/E7	Behandling av epitel neoplasi på vulvan positiv för human papilloma virus
Norwegian	Humant papillomvirus type 16 E6/E7 syntetiske lange peptider	Behandling av epitelial neoplasi i vulva positivt for humant papillomvirus
Icelandic	Mannavörtuveira af stofngerð 16 E6/E7, með nýmynduðum löngum peptíðum	Meðferð við æxlismyndun í þekjuvef ytri skapa sem er jákvæð fyrir mannavörtuveiru