



Document Date: London, 24 April 2009
Doc.Ref.: EMEA/COMP/107517/2006

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of parathyroid hormone (1-34) transglutaminase fusion protein fibrin matrix complex for the treatment of solitary bone cysts

On 11 April 2006, orphan designation (EU/3/06/367) was granted by the European Commission to Kuros Biosurgery International AG, Lichtenstein, for parathyroid hormone (1-34) transglutaminase fusion protein fibrin matrix complex for the treatment of solitary bone cysts.

What are solitary bone cysts?

Solitary bone cysts (also called unicameral or simple bone cysts) are cavities that develop in the interior of bones by no apparent reason. These cavities are called solitary because appear at single localisations, most frequently in areas close to joints of the femur or the upper arm. Solitary bone cysts can either produce symptoms or remain unnoticed. Because of the presence of the solitary cysts, patients bone becomes fragile and spontaneous bone fracture is one of the most common symptoms. Spontaneous bone fracture can be a repeated problem in some patients and impair bone's growth. Solitary bone cysts are chronically debilitating in particular due to serious sequelae secondary to bone fracture and bone growth retardation.

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

At the time of designation solitary bone cysts affected less than 1 in 10,000 people in the European Union (EU)*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 46,000 people.

What treatments are available?

At the time of submission of the application for orphan designation, no satisfactory method had been authorised in the European Union for treatment of the condition.

How is this medicine expected to work?

Parathyroid hormone is a hormone present in the body that, amongst other functions, stimulates the formation of new bone tissue. Parathyroid hormone (1-34) transglutaminase fusion protein fibrin matrix complex is composed by a part (sequence) of the parathyroid hormone that keeps the hormone activity on bone formation. In the proposed product the hormone sequence is fixed to a support base made of fibrin that will keep the hormone in place and will allow local activity. Fibrin is a natural component of many body tissues and does not have any bone growth stimulating activity by itself. By stimulating the local production of bone the product could induce the filling of the solitary bone cysts.

* 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 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

What is the stage of development of this medicine?

The effects of parathyroid hormone (1-34) transglutaminase fusion protein fibrin matrix complex had been evaluated in experimental models.

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

Parathyroid hormone (1-34) transglutaminase fusion protein fibrin matrix complex was not authorised anywhere worldwide for solitary bone cysts or designated as 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 8 March 2006 a positive opinion recommending the grant of the above-mentioned designation.

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 Community) 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:

Kuros Biosurgery International AG

C/o Forum Trust reg.

Landstrasse 34

FL-9494 Schaan

Liechtenstein

Telephone: +41 44 200 5614

Telefax: +41 44 200 5714

E-mail: virginia.jamieson@kuros.ch

Patients' association contact point: Not available

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

Language	Active Ingredient	Indication
English	Parathyroid hormone (1-34) transglutaminase fusion protein fibrin matrix complex	Treatment of solitary bone cysts
Czech	Parathyreoidní hormon (1-34) a transglutaminázni fúzní protein kovalentně vázaný na fibrin matrixového komplexu	Léčba solitárních kostních cyst
Danish	Parathyreoideahormon (1-34) transglutaminase fusionsprotein fibrinmatrix kompleks	Behandling af solitære knoglecyster
Dutch	Parathyroïdhormoon (1-34) transglutaminase fusieproteïne fibrinmatrixcomplex	Behandeling van solitaire botcysten
Estonian	Parathormooni (1-34) transglutaminaasi siduvvalk fibriini maatrikskompleksis	Solitaarse luutsüsti ravi
Finnish	Lisäkilpirauhashormoni(1-34)- transglutaminaasifuusioproteiinin ja fibriinimatriisin kompleksi	Yksittäisten luurakkuloiden hoitoon
French	Complexe matriciel de fibrine et d'une protéine de fusion de la transglutaminase et de l'hormone parathyroïdienne 1-34	Traitement des kystes osseux isolés
German	Parathyroidhormon (1-34)- transglutaminase-Fusionsprotein- Fibrinmatrixkomplex	Behandlung solitärer Knochenzysten
Greek	πρωτεΐνη σύντηξης της τρανσγλουταμινάσης της παραθυρεοειδικής ορμόνης (1-34) σε υπόστρωμα συμπλέγματος ινώδους.	Θεραπεία μονήρων οστικών κύστεων
Hungarian	Parathyroid hormon (1-34) transzglutamináz fúziós fehérje fibrin matrix komplex	Szoliter csontciszta kezelése
Italian	Complesso a matrice di fibrina di ormone paratiroideo (1-34) con proteina di fusione transglutaminasi	Trattamento delle cisti ossee solitarie
Latvian	Paravairogdziedzera hormona (1-34) transglutamināzes un hibrīdroteīnu fibrīnmatriksa komplekss	Savrupu kaula cistu ārstēšana
Lithuanian	Prieskydinės liaukos hormono (1-34) transgliutaminazės sujungimo baltymo fibrino formos kompleksas	Pavienių kaulų cistų gydymas
Polish	Białko F transglutaminazy hormonu przytarczyc 1-34 z fibrynową macierzą	Leczenie pojedynczej torbieli kostnej
Portuguese	Complexo da matriz de fibrina da proteína de fusão da transglutaminase da hormona paratiroideia 1-34	Tratamento de quistos ósseos isolados
Slovak	Transglutaminázový fúzny protein	Liečba izolovaných kostných cyst

	hormónu prištítnych teliesok 1-34	
Slovenian	Fuzijski protein transglutaminaze in paratiroidnega hormona (1-34) v fibrinskem matriksu	Zdravljenje solitarnih kostnih cist
Spanish	Proteína de fusión compuesta por hormona paratiroidea (1-34) y transglutaminasa en un complejo de matiz de fibrina	Tratamiento de los quistes óseos solitarios
Swedish	Paratyroidhormon 1-34 transglutaminas fusionsprotein sammansatt fibrinmatris	Behandling av solitära bencystor
Norwegian	Parathyroideahormon (1-34) transglutaminase fusjonsprotein bundet til en fibrin matriks	Behandling av solitære bencyster
Icelandic	Kalkkirtilshormón (I-34) transglútamínasa samrunaprótín fíbrín grindar flækja	Meðferð á stökum beinholum