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EMA/COMP/220/2004 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human engineered monoclonal antibody specific for transforming growth factor β2 (CAT-152) for the prevention of scarring in glaucoma filtration surgical procedure

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in June 2009 on request of the sponsor.

On 30 May 2001, orphan designation (EU/3/01/042) was granted by the European Commission to Cambridge Antibody Technology, United Kingdom, for human engineered monoclonal antibody specific for transforming growth factor β2 (CAT-152) for the prevention of scarring in glaucoma filtration surgical procedure.

What is scarring in glaucoma filtration surgical procedure?

Glaucoma is a condition that affects the nerve of the eye and can cause vision loss. Gradual increase of the pressure in the eye can injure the optical nerve. The risk factors for the development of glaucoma include diabetes, high blood pressure, and excess of blood cholesterol.

Glaucoma filtration surgical procedure is one of the available treatments. The procedure creates a new passageway by which aqueous fluid accumulated inside the eye can escape, thereby lowering the pressure. The filter allows the drainage of aqueous fluid from inside the anterior chamber of the eye to a pocket created between the conjunctiva (the outermost layer covering of the eye), and the sclera (the white of the eye). The aqueous fluid is eventually absorbed by blood vessels. However, collagen (the protein of the connective tissue) can be deposited on the site of filtration after surgery, resulting in the formation of excessive scar tissue. Glaucoma is a serious and chronically debilitating condition.

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What is the estimated number of patients at risk of developing the condition?

At the time of designation, the number of patients at risk of scarring in glaucoma filtration surgical procedure was estimated to be approximately 3.1 people in 10,000 in the European Union (EU)[*]. This is equivalent to a total of around 117,000 people, which 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 methods of prevention are available?

At the time of submission of the application for orphan drug designation, there were no authorised medicinal products to be used as adjunct therapy to glaucoma filtration surgery for prevention of excessive scarring and improve outcome. Corticosteroids and medicines modulating the healing process were frequently used, but were not authorised for this condition.

How is this medicine expected to work?

The transforming growth factor β2 (TGFß2) is expressed in the eye and is thought to promote formation of scarring following filtration surgery. The human engineered monoclonal antibody specific for transforming growth factor β2 (CAT-152) can bind to TGFß2, and to inhibit its effects. This way, the product is expected to stop scarring in the eye after the operation.

What is the stage of development of this medicine?

The effects of human engineered monoclonal antibody specific for transforming growth factor β2 (CAT-152) were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with the prevention of scarring in glaucoma filtration surgical procedure were ongoing.

The medicinal product was not marketed anywhere worldwide for the prevention of scarring in glaucoma filtration surgical procedure 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 10 April 2001 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 based on data from the European Union. This represents a population of 377,000,000 (Eurostat 2001).

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	Human engineered monoclonal antibody specific for transforming growth factor β2	Prevention of scarring in glaucoma filtration surgical procedures
Danish	Gensplejset humant monoklonalt antistof specifikt for transformerende vækstfaktor β2	Forebyggelse af ardannelse ved kirurgiske glaukom filtreringsindgreb
Dutch	Genetisch gemanipuleerd humaan monoclonaal antilichaam specifiek voor transformatie-groeifactor β2	Preventie van littekenvorming bij glaucoma filtratie chirurgie
Finnish	Ihmisen geeniteknologisesti muunneltu monoklonaalinen vasta-aine, joka on spesifinen transformoivalle kasvutekijälle β2	Arpeutumisen esto glaukoomaa filtroivan leikkauksen hoidossa
French	Anticorps monoclonal humain obtenu par génie génétique spécifique du facteur de croissance transformant β2	Traitement préventif de cicatrisation suite à chirurgie fistulisante des glaucomes
German	Gentechnisch hergestellter humaner monoklonaler Antikörper, spezifisch für Transforming Growth Factor β2	Verhütung von Narbenbildung bei chirurgischen Glaukom-Filtrationsverfahren
Greek	Ανθρώπινο μονοκλωνικό αντίσωμα επεξεργασμένο με γενετική μηχανική ειδικό για τον μετατρεπτικό αυξητικό παράγοντα β2	Πρόληψη της δημιουργίας ουλής σε χειρουργικές επεμβάσεις διηθήσεως του γλαυκώματος
Italian	Anticorpo monoclonale umano, ottenuto per ingegneria genetica, specifico per il fattore di crescita trasformante β2	Prevenzione della cicatrizzazione nelle procedure chirurgiche di filtrazione del glaucoma
Portuguese	Anticorpo monoclonal humano produzido por engenharia genética,específico do factor de crescimento transformante β2	Prevenção da cicatrização em intervenções cirúrgicas de filtração do glaucoma
Spanish	Anticuerpo monoclonal humano obtenido mediante ingeniería genética, específico para el factor de crecimiento transformante β2	Prevención de la cicatrización en las intervenciones quirúrgicas de filtración de glaucoma
Swedish	Med genteknik framtagen human monoklonal antikropp som är specifik för transformerande tillväxtfaktor β2	Förebyggande av ärrbildning vid operative glaukomfiltreringsingrepp

[1] At the time of designation