



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Recombinant human serum amyloid P for the prevention of scarring post glaucoma filtration surgery

On 9 October 2009, orphan designation (EU/3/09/674) was granted by the European Commission to RegPak BioPharma Consulting, The Netherlands, for recombinant human serum amyloid P for the prevention of scarring post glaucoma filtration surgery.

The sponsorship was transferred to Appletree Europe S.à.r.l., Luxembourg, in August 2010.

#### **What is scarring post glaucoma filtration surgery?**

Scarring post glaucoma filtration surgery is the formation of excessive scar tissue following an operation to treat glaucoma. Glaucoma is a disease where the pressure in the eye rises because of an accumulation of fluid inside the eye that cannot drain away. This affects the optic nerve (the nerve that sends signals from the eye to the brain) and can cause loss of vision.

In the operation called 'filtration surgery', a small channel is created through the sclera (the white of the eye) to allow accumulated fluid to drain away. Scarring may occur after surgery when, during healing, too much collagen (a type of protein) is deposited in the channel. This causes the channel to seal up and the pressure inside the eye to build up again.

Scarring post glaucoma filtration surgery is a serious and long-term debilitating condition because it may lead to loss of vision.

#### **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 post glaucoma filtration surgery was estimated to be approximately 1.4 people in 10,000 in the European Union (EU)\*. This is equivalent to a total of around 71,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

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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. This represents a population of 504,800,000 (Eurostat 2009).



## **What methods of prevention are available?**

At the time of designation, no satisfactory methods were authorised in the EU for the prevention of scarring post glaucoma filtration surgery. Medicines that stop the growth of cells (and are often also used to treat cancer) can be injected at the incision site to slow down the formation of scar tissue and prevent the channel sealing up.

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

Recombinant human serum amyloid P is expected to work in the same way as the naturally-occurring substance human serum amyloid P, which circulates in the blood and is involved in wound healing. This medicine is expected to help the wound in the eye to heal by helping to remove damaged tissue and blocking the production of excess collagen at site of injury, thereby avoiding excessive scarring.

Recombinant human serum amyloid P is to be given by injection into the eyes of patients undergoing glaucoma filtration surgery.

Recombinant human serum amyloid P is made by a method known as 'recombinant DNA technology': it is made by a cell that has received the human gene (DNA) that makes it able to produce human serum amyloid P.

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

The effects of recombinant human serum amyloid P have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with scarring post glaucoma filtration surgery had been started.

At the time of submission, recombinant human serum amyloid P was not authorised anywhere in the EU for the prevention of scarring post glaucoma filtration surgery or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 July 2009 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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Luxembourg  
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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	Recombinant human serum amyloid P	Prevention of scarring post glaucoma filtration surgery
Bulgarian	Рекомбинантен човешки серумен амилоид P	Превенция на белези след филтрираща операция при глаукома
Czech	Rekombinantní humánní sérový amyloid	Prevence zjizvení post glaukomatozní filtrační operace
Danish	Rekombinant humant serum amyloid P	Forebyggelse af ardannelse efter glaukomfiltreringsoperation
Dutch	Recombinant humaan serum amyloid P	Preventie van littekenvorming na glaucomfiltratiechirurgie
Estonian	Rekombinantne inimese seerumi amüloid-P	Glaukoomi filtratsioonikirurgia järgsete armide ennetamine
Finnish	Rekombinantti ihmisen seerumin P-amyloidi	Arpikudoksen kasvun esto glaukooman kirurgisen hoidon jälkeen
French	Composant P amyloïde humain recombinant du sérum	Prévention des cicatrices après chirurgie filtrante du glaucome
German	Rekombinantes menschliches Serum Amyloid P	Prävention von Narbenbildung nach Glaukom-Filtrations-Operation
Greek	Ανασυνδυασμένο ανθρώπινο Αμυλοειδές P Ορού	Πρόληψη ουλών μετά από εγχείριση διήθησης γλαυκώματος
Hungarian	Rekombináns emberi szérum amyloid P	Zöldhályog-szűrő műtét utáni hegesedés megakadályozása
Italian	Amiloide P di siero umano, ricombinante	Prevenzione delle cicatrici post intervento chirurgico filtrante per glaucoma
Latvian	Rekombinantais cilvēka seruma amiloīds P	Rētaudu veidošanās profilakse pēc glaukomas filtrēšanas operācijas
Lithuanian	Rekombinantinis žmogaus serumo amiloidas P	Randų po glaukomos filtracinės operacijos prevencija
Maltese	Amilojde P tas-serum uman rikombinanti	Prevenzjoni ta' ċikatriċi wara kirurġija ta' filtrazzjoni għal glawkoma
Polish	Ludzkie rekombinowane białko amyloidowe surowicy	Zapobieganie bliznom po operacji udroźnienia kąta przesączania w jaskrze
Portuguese	P amiloide sérica humana recombinante	Prevenção de cicatrização pós-cirurgia de filtração glaucoma
Romanian	Amiloid P seric uman recombinant	Prevenirea cicatrizării după intervenția chirurgicală de filtrare în glaucom
Slovak	Rekombinantný ľudský sérový amyloid	Prevenia jazvenia po filtračnej operácii glaukómu
Slovenian	Rekombinantni človeški serumski amiloid P	Preprečevanje brazgotin po filtracijski operaciji glavkoma
Spanish	Amiloide sérico P humano recombinante	Prevención de la cicatrización posterior a la cirugía de filtración del glaucoma

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

Language	Active ingredient	Indication
Swedish	Recombinant mänsklig serum amyloid P	Förebyggande av ärrbildning efter glaukom filtrerings ingrepp
Norwegian	Rekombinant humant serum amyloid P	Forebygging av arrdannelse etter glaukom-filtrasjonskirurgi
Icelandic	Raðbrigða manna sermis sterkjúlíki	Til að koma í veg fyrir örmyndun eftir síunaraðgerð vegna gláku