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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human embryonic stem-cell-derived retinal pigment epithelial cells for the treatment of Stargardt's disease

On 21 June 2011, orphan designation (EU/3/11/874) was granted by the European Commission to TMC Pharma Services Ltd, United Kingdom, for human embryonic stem-cell-derived retinal pigment epithelial cells for the treatment of Stargardt's disease.

What is Stargardt's disease?

Stargardt's disease is a genetic disorder of the eye that leads to the gradual loss of sight. It affects cells in the retina (the light-sensitive surface at the back of the eye) called retinal pigment epithelial cells. Patients with Stargardt's disease lack a protein called ABCR, which controls the movement of substances into and out of these cells. This causes deposits to build up inside the cells, which become damaged and eventually die.

Stargardt's disease is a long-term debilitating disease because it leads to the patient's sight getting worse and eventually to blindness.

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

At the time of designation, Stargardt's disease affected approximately 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 51,000 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?

At the time of submission of the application for orphan designation, no satisfactory methods were authorised in the EU for the treatment of Stargardt's disease. Patients with the disease were usually given physical aids such as sunglasses to reduce the rate of damage to the retina, or spectacles, magnifiers or telescopes to help them see during the early stages of the disease.

*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 506,300,000 (Eurostat 2011).

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How is this medicine expected to work?

This medicine is made up of retinal pigment epithelial cells. It is expected to be injected directly into the eye, under the retina, so that the cells can settle in the right part of the eye. Once implanted, the cells are expected to help the functioning of the retina.

The retinal pigment epithelial cells in the medicine have been produced from embryonic stem cells. These are cells obtained from a human embryo that can develop into different types of cell.

What is the stage of development of this medicine?

The effects of human embryonic stem-cell-derived retinal pigment epithelial cells have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with Stargardt's disease had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for Stargardt's disease. Orphan designation of the medicine had been granted in the United States of America for the treatment of Stargardt's macular dystrophy.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 March 2011 recommending the granting of this 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 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 embryonic stem-cell-derived retinal pigment epithelial cells	Treatment of Stargardt's disease
Bulgarian	Епителни клетки от ретинален пигмент, получени от човешки ембрионални стволови клетки	Лечение на Болест на Stargardt
Czech	Epiteliální pigmentové buňky oční sítnice získané z lidských embryonálních kmenových buněk	Léčba Stargardtovy choroby
Danish	Retinale pigmentepitelceller dannet fra humane embryonale stamceller	Behandling af Stargardt sygdom
Dutch	Aan humane embryonale stamcel ontleende retinale epitheliale pigmentcellen	Behandeling van de ziekte van Stargardt
Estonian	Reetina epiteeli pigmentrakkudest pärit inimese embrüonaalsed tüvirakud	Stargardt'tõve ravi
Finnish	Ihmisen alkion kantasolusta peräisin olevat verkkokalvon pigmentti-epiteelisolut	Stargardtin taudin hoito
French	Cellules de l'épithélium pigmentaire rétinien dérivées des cellules souches embryonnaires humaines	Traitement de la maladie de Stargardt
German	Von embryonalen Stammzellen abgeleitete humane Pigmentepithelzellen der Netzhaut	Behandlung der Stargardt-Krankheit
Greek	Ανθρώπινα εμβρυϊκά κύτταρα μελάγχρου επιθηλίου, προερχόμενα από βλαστοκύτταρα	Θεραπευτική αγωγή για την νόσο του Stargardt
Hungarian	Humán embrionális őssejtből származó retinalis pigmenthámsejtek	Stargardt-kór kezelése
Italian	Cellule dell'epitelio pigmentato retinico derivate da cellule staminali embrionali umane	Trattamento della malattia di Stargardt
Latvian	No cilvēka embrionālām cilmes šūnām iegūtas tīklenes pigmentu epitēlija šūnas	Stargardta slimības ārstēšana
Lithuanian	Tinklainės pigmentinio epitelio ląstelės išvestos iš kamieninių žmogaus ląstelių	Stargardt ligos gydymas
Maltese	Ċelluli epiteljali tal-pigment retinali imnisslin minn ċelluli steminali embrijoniċi umani	Kura tal-marda ta' Stargardt
Polish	Ludzkie komórki barwnikowe nabłonka siatkówki uzyskane z zarodkowych komórek macierzystych	Leczenie choroby Stargardta
Portuguese	Células do epitélio pigmentar da retina derivadas de células estaminais embrionárias humanas	Tratamento da doença de Stargardt
Romanian	Celule epiteliale pigmentare retininene derivate din celule stem embrionare umane	Tratamentul bolii Stargardt
Slovak	Bunky pigmentového epitelu sietnice získané z ľudských embryonálnych kmeňových buniek	Liečba Stargardtovej choroby
Slovenian	Pigmentne epitelijske celice retine iz izvornih celic človeškega embrija	Zdravljenje Stargardtjeve bolezni

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Células epiteliales del pigmento retiniano derivadas de células madre humanas de origen embrionario	Tratamiento de la enfermedad de Stargardt
Swedish	Retinala pigmentceller härledda från mänskliga embryonala stamceller	Behandling av Stargardts sjukdom
Norwegian	Humane embryonale stamcellederiverte retinale pigmentepitelceller	Behandling av Stargardts sykdom
Icelandic	Lithimnufrumur af fósturstofnfrumna uppruna úr mönnum	Meðferð við Stargardts sjúkdómi