



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ecothiopate iodide for the treatment of Stargardt's disease

On 24 April 2015, orphan designation (EU/3/15/1474) was granted by the European Commission to JIGConsultancy Ltd, United Kingdom, for ecothiopate iodide for the treatment of Stargardt's disease.

What is Stargardt's disease?

Stargardt's disease is a genetic (hereditary) disorder of the eye that leads to progressive loss of sight. Stargardt's disease is caused by abnormalities in a gene called *ABCA4*. The *ABCA4* gene is responsible for the production of a protein called ABCR that regulates the transport of substances in and out of some cells in the retina (the light-sensitive surface at the back of the eye). In patients with Stargardt's disease, ABCR does not work properly. This causes deposits to build up inside the retina cells, which become damaged and eventually die.

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

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

At the time of designation, Stargardt's disease affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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 designation, no satisfactory methods were authorised in the EU for the treatment of Stargardt's 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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,900,000 (Eurostat 2015).



How is this medicine expected to work?

The medicine contains ecothiopate iodide, which has been used in higher doses as eye drops for the treatment of open-angle glaucoma (a disease in which the pressure inside the eye rises because fluid cannot drain out of the eye) for over 40 years. The exact way ecothiopate iodide works in Stargardt's disease is not fully known but it is thought to involve stimulating the cells of the retina and reducing the formation of the substances that build up to form deposits within them. This is expected to improve the symptoms of the disease and reduce progression to blindness.

What is the stage of development of this medicine?

The effects of ecothiopate iodide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with ecothiopate iodide in patients with Stargardt's disease were ongoing.

At the time of submission, ecothiopate iodide was not authorised anywhere in the EU for Stargardt's disease. Orphan designation of the medicine had been granted in the United States for treatment of this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 March 2014 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Ecothiopate iodide	Treatment of Stargardt's disease
Bulgarian	Екотиофeйт йодид	Лечение на болест на Stargardt
Croatian	Ekotiofatjodid	Liječenje Stargardtove bolesti
Czech	Echothiopate jodidu	Léčba Stargardtovy choroby
Danish	Echothiopatiodid	Behandling af Stargardt sygdom
Dutch	Ecotiofaatjodide	Behandeling van de ziekte van Stargardt
Estonian	Ekotiofaatjodiid	Stargardt'tõve ravi
Finnish	Ekotiopaatti jodidi	Stargardtin taudin hoito
French	Iodure d'écothiopate	Traitement de la maladie de Stargardt
German	Echothiopat Iodid	Behandlung der Stargardt-Krankheit
Greek	Ιωδιούχος φωσφολίνη	Θεραπεία της νόσου Stargardt
Hungarian	Echotiofát jodid	Stargardt-kór kezelése
Italian	Ioduro d'ecotiofato	Trattamento della malattia di Stargardt
Latvian	Ekotiofāta jodīds	Stargardta slimības ārstēšana
Lithuanian	Echotiofato jodidas	Stargardt ligos gydymas
Maltese	Ecothiopate iodide	Kura tal-marda ta' Stargardt
Polish	Echotiopatu jodek	Leczenie choroby Stargardta
Portuguese	Iodeto de ecotiofato	Tratamento da doença de Stargardt
Romanian	Iodură de ecotiofat	Tratamentul bolii Stargardt
Slovak	Echothiopate iodide	Liečba Stargardtovej choroby
Slovenian	Ekotiofatjodid	Zdravljenje Stargardtove bolezni
Spanish	Ioduro de ecotiofato	Tratamiento de la enfermedad de Stargardt
Swedish	Ecothiopate jod	Behandling av Stargardts sjukdom
Norwegian	Ekotiofatjodid	Behandling av Stargardts sykdom
Icelandic	Echóthíopat jóðíð	Meðferð við Stargardts sjúkdómi

¹ At the time of designation