



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Talarozole for the treatment of recessive X-linked ichthyosis

On 4 July 2012, orphan designation (EU/3/12/1017) was granted by the European Commission to Stiefel Laboratories (Maidenhead) Limited, United Kingdom, for talarozole for the treatment of recessive X-linked ichthyosis.

What is recessive X-linked ichthyosis?

Recessive X-linked ichthyosis is an inherited skin disorder, seen almost exclusively in males. It is caused by a defect in a gene, located on the X chromosome, responsible for producing a protein which is important for making up the outer layer of the skin. The main symptom is that the outer layer of skin becomes dry, thick and scaly. The scales are firmly attached to the underlying skin and can be dark brown or grey in color.

Recessive X-linked ichthyosis is a long-term debilitating disease due to major symptoms which include scaling of the skin, particularly on the neck, trunk and lower extremities.

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

At the time of designation, recessive X-linked ichthyosis affected less than 1.2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 61,000 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 application for orphan designation, acitretin and carbamide were authorised in some countries of the EU to treat recessive X-linked ichthyosis.

The sponsor has provided sufficient information to show that talarozole might be of significant benefit for patients with recessive X-linked ichthyosis because it is potentially associated with fewer side

*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).



effects than other authorised treatments for this condition. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Talarozole is expected to work by increasing the amount of naturally occurring retinoic acid (vitamin A) in skin cells, by blocking the action of certain enzymes involved in breaking down retinoic acid in the body. Retinoic acid and related substances called retinoids are known to improve skin health and are used to treat various skin conditions. This is expected to help to reduce the symptoms of the disease.

Talarozole is expected to be taken as tablets.

What is the stage of development of this medicine?

The effects of talarozole have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with talarozole in patients with recessive X-linked ichthyosis had been started.

At the time of submission, talarozole was not authorised anywhere in the EU for recessive X-linked ichthyosis 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 11 May 2012 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

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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	Talarozole	Treatment of recessive X-linked ichthyosis
Bulgarian	Таларозол	Лечение на рецесивна X-свързана ихтиоза
Czech	Talarozol	Léčba ichtyózy recesivně vázané na chromozom- X
Danish	Talarozol	Behandling af recessiv X-bundet ichthyosis
Dutch	Talarozole	Behandeling van recessief X-gebonden ichthyosis
Estonian	Talarosool	Retsessiivse X-liitelise ihtüoosi ravi.
Finnish	Talarotsoli	X-kromosomissa peittyvästi periytyvän kalansuomutaudin hoito
French	Talarozole	Traitement de l'ichtyose congenitale récessive liée à l'X
German	Talarozol	Behandlung der rezessiven, X-chromosomal vermittelten Ichthyose
Greek	Ταλαροζόλη	Θεραπεία της υπολειπόμενης φυλοσύνδετης ιχθύωσης
Hungarian	Talarozol	X-kromoszómához kötött recesszív ichthyosis kezelése
Italian	Talarozole	Trattamento dell'ittiosi congenita recessiva X-linked
Latvian	Talarozols	Recesīvas ar X hromosomu saistītas ihtiozes ārstēšana
Lithuanian	Talarozolas	Su X susijusios recesyvinės ichtiozės gydymas
Maltese	Talarozole	Kura tal-ittjosi reċessiva marbuta mal-kromosoma X
Polish	Talarozol	Leczenie recesywnej rybiej łuski sprzężonej z chromosomem X
Portuguese	Talarozol	Tratamento da ictiose recessiva associada ao cromossoma X
Romanian	Talarozol	Tratamentul ihtiozei recesive X-linkate
Slovak	Talarozol	Liečba recesívnej ichtyózy viazanej na chromozóm X
Slovenian	Talarozol	Zdravljenje na X-kromosom vezane ihtioze
Spanish	Talarozol	Tratamiento de la ictiosis recesiva ligada al cromosoma X
Swedish	Talarozol	Behandling av X-kromosombunden recessiv iktyos
Norwegian	Talarozol	Behandling av recessiv X-bundet iktyose
Icelandic	Talarózól	Meðferð á víkjandi X-tengdu ichthýosis

¹ At the time of designation