



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 keratinopathic ichthyosis

On 4 July 2012, orphan designation (EU/3/12/1014) was granted by the European Commission to Stiefel Laboratories (Maidenhead) Limited, United Kingdom, for talarozole for the treatment of keratinopathic ichthyosis.

#### What is keratinopathic ichthyosis?

Keratinopathic ichthyosis is an inherited skin disorder caused by abnormalities in a gene responsible for producing keratin, a protein which is important for making up the outer layer of the skin. If there are too many keratin-containing cells in the skin, the skin can become dry, thick and scaly. The most distinctive features of keratinopathic ichthyosis are erythema (reddening of the skin) and blistering, including on the palms and the soles of the feet.

Keratinopathic ichthyosis is a long-term debilitating disease because it causes a range of severe skin problems including widespread blistering typically seen in newly born babies.

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

At the time of designation, keratinopathic ichthyosis affected less than 0.05 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of fewer than 2,500 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 keratinopathic ichthyosis.

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

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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 506,300,000 (Eurostat 2011).



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 keratinopathic ichthyosis had been started.

At the time of submission, talarozole was not authorised anywhere in the EU for keratinopathic 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.

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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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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    | Talarozole        | Treatment of keratinopathic ichthyosis       |
| Bulgarian  | Таларозол         | Лечение на кератинопатична ихтиоза           |
| Czech      | Talarozol         | Léčba keratinózní ichtyózy                   |
| Danish     | Talarozol         | Behandling af keratinopatisk ichthyosis      |
| Dutch      | Talarozole        | Behandeling van keratinopathische ichthyosis |
| Estonian   | Talarosool        | Keratinopaatilise ihtüoosi ravi              |
| Finnish    | Talarotsoli       | Keratinopaattisen kalansuomutaudin hoito     |
| French     | Talarozole        | Traitement de la keratinopathie ichthyosique |
| German     | Talarozol         | Behandlung der keratinopathischen Ichthyosen |
| Greek      | Ταλαροζόλη        | Θεραπεία της κερατινοπαθούς ιχθύωσης         |
| Hungarian  | Talarozol         | Keratinopátiás ichthyosis kezelése           |
| Italian    | Talarozole        | Trattamento dell'ittiosi cheratinopatica     |
| Latvian    | Talarozols        | Keratinopātiskas ihtiozes ārstēšana          |
| Lithuanian | Talarozolas       | Keratinopatinės ihtiozės gydymas             |
| Maltese    | Talarozole        | Kura tal-ittjosi keratinopatika              |
| Polish     | Talarozol         | Leczenie rybiej łuski rogówki                |
| Portuguese | Talarozol         | Tratamento da ictiose queratinopática        |
| Romanian   | Talarozol         | Tratamentul ihtiozei keratinopatice          |
| Slovak     | Talarozol         | Liečba keratinopatickej ichtyózy             |
| Slovenian  | Talarozol         | Zdravljenje keratinopatične ihtioze          |
| Spanish    | Talarozol         | Tratamiento de las ictiosis queratinopáticas |
| Swedish    | Talarozol         | Behandling av keratinopatisk iktyos          |
| Norwegian  | Talarozol         | Behandling av keratinopatisk iktyose         |
| Icelandic  | Talarózól         | Meðferð á keratínópathic ichthýósis          |

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