19 July 2012
EMA/COMP/393059/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Talarozole for the treatment of autosomal recessive congenital ichthyosis

On 4 July 2012, orphan designation (EU/3/12/1005) was granted by the European Commission to Stiefel Laboratories (Maidenhead) Limited, United Kingdom, for talarozole for the treatment of autosomal recessive congenital ichthyosis.

What is autosomal recessive congenital ichthyosis?

Autosomal recessive congenital ichthyosis is an inherited skin disorder caused by abnormalities in genes responsible for producing proteins which are important for making up the outer layer of the skin. The main feature is dry, thick, scaly or flaky skin. In its most severe form, children are born prematurely and are covered by thick, hard, armour-like scales that severely restrict movement.

Autosomal recessive congenital ichthyosis is a long-term debilitating disease due to the appearance of symptoms at birth or in early childhood. It can bring life-threatening complications in new born babies including breathing and feeding problems and infections.

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

At the time of designation, autosomal recessive congenital ichthyosis affected less than 0.1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 5,000 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 autosomal recessive congenital ichthyosis.

The sponsor has provided sufficient information to show that talarozole might be of significant benefit for patients with autosomal recessive congenital ichthyosis because it is potentially associated with

[*]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).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

fewer side 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 autosomal recessive congenital ichthyosis had been started.

At the time of submission, talarozole was not authorised anywhere in the EU for autosomal recessive congenital 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

Sponsor's contact details:

Stiefel Laboratories (Maidenhead) Limited
Eurasia Headquarters
Concord Road
Maidenhead
Berkshire SL6 4BY

United Kingdom

Telephone: +44 208 990 4723
Telefax: +44 208 990 2746
E-mail: marta.x.kollb-sielecka@stiefel.com

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	Talarozole	Treatment of autosomal recessive congenital ichthyosis
Bulgarian	Таларозол	Лечение на автозомно-рецесивна вродена ихтиоза
Czech	Talarozol	Léčba kongenitální autozomálně recesivní ichtyózy
Danish	Talarozol	Behandling af autosomal recessiv kongenit ichthyosis
Dutch	Talarozole	Behandeling van autosomaal recessief congenitale ichtyosis
Estonian	Talarosool	Kaasasündinud autosoom-retsessiivse ihtüoosi ravi
Finnish	Talarotsoli	Autosomaalisesti, peittyvästi periytyvän synnynnäisen kalansuomutaudin hoito
French	Talarozole	Traitement de l'ichtyose congénitale autosomale recessive
German	Talarozol	Behandlung der autosomal-rezessiven, angeborenen Ichthyose
Greek	Ταλαροζόλη	Θεραπεία της αυτοσωματικής υπολειπόμενης συγγενούς ιχθύασης
Hungarian	Talarozol	Autoszomális recesszív kongenitális ichthyosis kezelése
Italian	Talarozole	Trattamento dell'ittiosi congenita autosomica recessiva
Latvian	Talarozols	Autosomāli recisīvas iedzimtas ihtiozes ārstēšana
Lithuanian	Talarozolas	Autosominės recesyvinės įgimtos ichtiozės gydymas
Maltese	Talarozole	Kura tal-ittjosi konġenitali awtosomali reċessiva
Polish	Talarozol	Leczenie autosomalnie recesywnie dziedziczonej wrodzonej rybiej łuski
Portuguese	Talarozol	Tratamento da ictiose autosómica recessiva humana
Romanian	Talarozol	Tratamentul ihtiozei congenitale autosomal recesive
Slovak	Talarozol	Liečba autozomálne recesívnej vrodenej ichtyózy
Slovenian	Talarozol	Zdravljenje avtosomno recesivne vrojene ihtioze
Spanish	Talarozol	Tratamiento de la ictiosis congénita de herencia autosómica recesiva
Swedish	Talarozol	Behandling av autosomal recessiv kongenital iktyos
Norwegian	Talarozol	Behandling av autosomal recessiv medfødt iktyose
Icelandic	Talarózól	Meðferð á autósómal víkjandi meðfæddu ichthýósis

[1] At the time of designation