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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Tazarotene for the treatment of congenital ichthyoses

First publication	1 April 2009
Rev.1: sponsor's change of address	6 April 2011
Rev.2: withdrawal from the Community Register	26 February 2015

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in January 2015 on request of the Sponsor.

On 18 December 2006, orphan designation (EU/3/06/423) was granted by the European Commission to Orfagen, France, for tazarotene for the treatment of congenital ichthyoses.

What are congenital ichthyoses?

The term ichthyosis describes a group of diseases that affect skin. The skin becomes dry and forms tiny scales all over the body. Congenital ichthyoses are present at birth, and are due to defects in certain genes. This is different from other ichthyoses, which start later in life and are due to other causes. Normally, skin acts as a barrier against external agents. The most superficial layer of the skin contains a protein called keratin that is produced by cells called keratinocytes. This protein is very important for the skin to build up the barrier against infections and to protect the body from chemical and physical agents. However, if there are too many keratin-containing cells in the skin, it can lead to scaling. The scales give people with the disease a parchment- or mosaic-like fragile skin, that can cause a negative reaction from other people. Also, the skin is no longer working properly as a barrier against external agents. In newborns this can lead to more infections.

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

At the time of designation, congenital ichthyosis affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 94,000 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?

Treatment of ichthyoses depends on several factors, including how severe the disease is, how much of the skin is affected, and the way the skin looks. Most treatments aim to moisturize the skin and to decrease scaling. Sometimes, there can be complications, such as infection, and then additional treatment is necessary. Another type of treatment is medicines that contain retinoids. Retinoids are substances that are found in the body, and that stimulate the full growth of cells in the skin, and this can be used to decrease scales. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that tazarotene might be of potential significant benefit for the treatment of congenital ichthyoses mainly because it acts on the retinoid receptor. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

By binding to specific structures in the keratinocytes, retinoids regulate the growth of these cells. After administration, tazarotene is converted into a product that acts on retinoid receptors, and this active form of the medicine is thought to affect the development of special characters (differentiation) and to decrease the division of keratinocytes. Finally, these effects are expected to reduce the forming of scales.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, no clinical trials in patients with congenital ichthyosis were initiated.

The medicinal product was not authorised anywhere worldwide for congenital ichthyosis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 November 2006 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.

*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).

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	Tazarotene	Treatment of congenital ichthyoses
Czech	Tazaroten	Léčba ichthyosis congenita
Danish	Tazaroten	Behandling af medfødt iktyose
Dutch	Tazaroteen	Behandeling van congenitale ichthyoses
Estonian	Tazaroteen	Kaasasündinud ihtüooside ravi
Finnish	Tatsaroteeni	Synnynnäisten suomutautien (iktyoosien) hoito
French	Tazarotène	Traitement des ichtyoses congénitales
German	Tazaroten	Behandlung der kongenitalen Ichtyose
Greek	Tazarotene	Θεραπεία των συγγενών ιχθυώσεων
Hungarian	Tazaroten	Congenitalis ichthyosis kezelése
Italian	Tazarotene	Trattamento delle ittiosi congenite
Latvian	Tazarotene	Iedzimtas ihtiozes ārstēšana
Lithuanian	Tazarotenas	Įgimtos ichtiozės gydymas
Polish	Tazaroten	Leczenie wrodzonej rybiej łuski
Portuguese	Tazaroteno	Tratamento das ictioses congénitas
Slovak	Tazarotén	Liečba vrodenej ichtyózy
Slovenian	Tazaroten	Zdravljenje kongenitalne ihtioze
Spanish	Tazaroteno	Tratamiento de las ictiosis congénitas
Swedish	Tazaroten	Behandling av medfödd iktyos
Norwegian	Tazaroten	Behandling av kongenital iktyose
Icelandic	Tazaróten	Meðferð á meðfæddri hreisturhúð

[1] At the time of designation