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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allogeneic human dermal fibroblasts for the treatment of epidermolysis bullosa

On 20 September 2010, orphan designation (EU/3/10/774) was granted by the European Commission to Intercytex Ltd, United Kingdom, for allogeneic human dermal fibroblasts for the treatment of epidermolysis bullosa.

What is epidermolysis bullosa?

Epidermolysis bullosa is a disease of the skin, in which the outer layer of the skin, the epidermis, separates from the inner layer, the dermis. This makes the skin very fragile and causes severe blistering and scarring. The disease is caused by abnormalities in the genes responsible for the production of the proteins that make the skin strong and elastic, such as collagen.

Epidermolysis bullosa is a disease that is debilitating in the long term and may be life threatening, mainly because of the severe blistering, which results in poor quality of life and reduced life expectancy.

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

At the time of designation, epidermolysis bullosa affected less than 0.5 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 25,000 people, and is below the threshold 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 epidermolysis bullosa. Patients were advised to maintain a high standard of personal hygiene and skincare to help blisters heal, to avoid infections and to protect the skin from damage. Painkillers were

[*]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 506,500,000 (Eurostat 2010).

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also used. Surgery was sometimes necessary if there were complications such as deformed hands or the development of skin cancer.

How is this medicine expected to work?

Allogeneic human dermal fibroblasts is an advanced therapy medicine that belongs to the group called 'tissue engineered products'. These are medicines that contain cells or tissues that have been 'engineered' (modified) so they can be used to repair, regenerate or replace tissue.

This medicine is made of dermal fibroblasts, a type of skin cell that produces collagen and other skin components. These cells are extracted from a donor, then grown in a laboratory to increase their number. When injected into the skin, they are expected to start producing collagen and other skin components, helping the dermis and epidermis to stick together and preventing blisters from forming.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, no preclinical studies in experimental models had been performed.

At the time of submission, clinical trials with the medicine in patients with epidermolysis bullosa had been planned.

At the time of submission, this medicine was not authorised anywhere in the EU for epidermolysis bullosa. Orphan designation of the medicine had been granted in the United States of America for dystrophic epidermolysis bullosa.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 2 June 2010 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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Allogeneic human dermal fibroblasts	Treatment of epidermolysis bullosa
Bulgarian	Алогенни човешки кожни фибробласти	Лечение на булозна епидермолиза
Czech	Allogenní lidské kožní fibroblasty	Léčba bulózní epidermolýzy
Danish	Allogene humane dermale fibroblaster	Behandling af epidermolysis bullosa
Dutch	Allogene, humane, dermale fibroblasten	Behandeling van epidermolysis bullosa
Estonian	Inimese allogeensed naha fibroblastid	Bulloosse epidermolüüsi ravi
Finnish	Allogeeniset ihmisen dermaaliset fibroblastit	Epidermolysis bullosan hoito
French	Fibroblastes dermiques humains allogéniques	Traitement de l'épidermolyse bulleuse
German	Allogene, humane, dermale Fibroblasten	Behandlung der Epidermolysis bullosa
Greek	Αλλογενείς ανθρώπινοι δερματικοί ινοβλάστες	Θεραπεία της πομφολυγώδους επιδερμόλυσης
Hungarian	Allogén humán bőrfibroblaszt	Epidermolysis bullosa kezelése
Italian	Fibroblasti dermici umani allogenici	Trattamento della epidermolisi bollosa
Latvian	Allogēni cilvēka ādas fibroblasti	Bulozās epidermolīzes ārstēšanai
Lithuanian	Alogeniniai žmogaus odos fibroblastai	Pūslinės epidermolizės gydymas
Maltese	Fibroblasts mill-ġilda alloġeniċi umani	Kura tal-epidermolisi bullosa
Polish	Alogeniczne fibroblasty skóry ludzkiej	Pęcherzowe oddzielanie się naskórka
Portuguese	Fibroblastos dérmicos humanos alogénicos	Tratamento da epidermolise bulhosa
Romanian	Fibroblaşti dermici umani alogenici	Tratamentul epidermolizei buloase
Slovak	Alogénne ľudské kožné fibroblasty	Liečba epidermolysis bullosa
Slovenian	Alogenski človeški dermalni fibroblasti	Zdravljenje bulozne epidrmolize
Spanish	Fibroblastos dérmicos alogénicos humanos	Tratamiento de la epidermolisis bullosa
Swedish	Allogena, humana, dermala fibroblaster	Behandling av epidermolysis bullosa
Norwegian	Allogene humane dermale fibroblaster	Behandling av epidermolysis bullosa
Icelandic	Ósamgena trefjakímfrumur úr mannshúð	Meðferð á epidermolysis bullosa

[1] At the time of designation