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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh for the treatment of epidermolysis bullosa

First publication	29 June 2011
Rev.1: transfer of sponsorship	14 January 2013
Rev.2: withdrawal from the Community Register	21 March 2013

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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2014 on request of the Sponsor.

On 21 June 2011, orphan designation (EU/3/11/873) was granted by the European Commission to TMC Pharma Services Ltd, United Kingdom, for human dermal fibroblasts cultured on a bioresorbable polyglactin mesh for the treatment of epidermolysis bullosa.

The sponsorship was transferred to Shire Pharmaceuticals Ireland Limited, Ireland, in June 2012.

What is epidermolysis bullosa?

Epidermolysis bullosa is an inherited disease of the skin that affects mainly babies and children, 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 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.

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

At the time of designation, epidermolysis bullosa affected less than 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 30,000 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 designation, no satisfactory methods were authorised in the EU to treat epidermolysis bullosa. A high standard of personal hygiene and skincare were recommended to help blisters heal, to avoid infections and to protect the skin from damage. Painkillers were 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?

The medicine is made up of 'fibroblasts' that have been grown in the laboratory to be used as a skin substitute. Fibroblasts are specialised cells in the inner layers of the skin that produce proteins such as collagen and form the 'connective tissue', the tissue that supports the skin and internal organs. The fibroblasts in this medicine have been grown on a mesh scaffold so that they can be used like a skin graft. Once implanted, the mesh dissolves naturally and the fibroblasts settle into the wound, where they are expected to help the healing process by providing proteins that promote the growth of blood vessels and epidermis.

What is the stage of development of this medicine?

The effects of human dermal fibroblasts cultured on a bioresorbable polyglactin mesh have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine for various wound repair indications were ongoing.

At the time of submission, the medicine was authorised in the USA under the trade name Dermagraft for the treatment of diabetic foot ulcer.

At the time of submission, the medicine was not authorised anywhere in the EU for epidermolysis bullosa 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 9 March 2011 recommending the granting of this designation.

[*]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.
At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).

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	Human dermal fibroblasts cultured on a bioresorbable polyglactin mesh	Treatment of epidermolysis bullosa
Bulgarian	Човешки дермални фибробласти, отгледани върху биорезорбируема полиглактинова мрежа	Лечение на булозна епидермолиза
Czech	Lidské dermální fibroblasty kultivované na vstřebatelné polyglaktinové síťovině	Léčba bulózní epidermolýzy
Danish	Humane dermale fibroblaster dyrket på et bioresorberbart polyglactinnet	Behandling af epidermolysis bullosa
Dutch	Dermale fibroblasten van humane oorsprong gecultiveerd op een bioresorbeerbaar polyglactine maas	Behandeling van epidermolysis bullosa
Estonian	Bioresorbeeruvale polüglaktiin võrgustikule kultiveeritud inimese naha fibroblastid.	Bulloosse epidermolüüsi ravi
Finnish	Bioresorboituvassa polyglaktiini-verkossa viljellyt ihmisen dermaaliset fibroblastit	Epidermolysis bullosan hoito
French	Fibroblastes de derme humain cultivés sur filet biorésorbable de polyglactine.	Traitement de l'épidermolyse bulleuse
German	Humane Hautfibroblasten kultiviert auf bioresorbierbarem Polyglactin-Netz	Behandlung der Epidermolysis bullosa
Greek	Ανθρώπινες δερματικές ινοβλάστες καλλιεργημένες σε βιοαπορροφώμενο πλέγμα πολυγλακτίνης	Θεραπεία της πομφολυγώδους επιδερμόλυσης
Hungarian	Felszívódó polyglactin rácson tenyésztett humán dermális fibroblasztok	Epidermolysis bullosa kezelése
Italian	Fibroblasti dermici umani coltivati su un substrato bioassorbibile di acido poliglicolico	Trattamento della epidermolisi bollosa
Latvian	Uz biouzsūcošā poliglaktīna sietiņa kultivēti Cilvēka dermālie fibroblasti	Bulozās epidermolīzes ārstēšanai
Lithuanian	Žmogaus odos fibroblastai užauginti ant biorezorbuojančio poliglaktino tinklelio	Pūslinės epidermolizės gydymas
Maltese	Fibroblasts mill-ġilda umana imkabbra fuq xibka ta' polyglactin li tiġi assorbita mill-ġdid bijoloġikament	Kura tal-epidermolisi bullosa
Polish	Fibroblasty skóry ludzkiej hodowane na ulegającej wchłonięciu siatce z poliglaktyny	Pęcherzowe oddzielanie się naskórka
Portuguese	Fibroblastos de derme humana cultivados em malha de poligelatina reabsorvível.	Tratamento da epidermólise bulhosa
Romanian	Fibroblaste dermice umane cultivate pe un substrat bioresorbabil de poliglactină	Tratamentul epidermolizei buloase
Slovak	Ľudské kožné fibroblasty kultivované na biorezorbovateľnej polyglaktínovej mriežke	Liečba epidermolysis bullosa

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Humani kožni fibroblasti vzgojeni na bioresorbilni poliglaktinski mreži	Zdravljenje bulozne epidrmolize
Spanish	Fibroblastos humanos cutáneos cultivados en una malla biológica reabsorbible de poliglactina	Tratamiento de la epidermolisis bullosa
Swedish	Humana dermala fibroblaster odlade i ett bioresorberbart polyglaktin nät	Behandling av epidermolysis bullosa
Norwegian	Humane dermale fibroblaster dyrket på et bioresorberbart polyglaktin-nett	Behandling av epidermolysis bullosa
Icelandic	Manna húð fíbróblastar á lífísogandi pólýglaktín neti	Meðferð á epidermolysis bullosa