EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allogeneic adipose-derived adult mesenchymal stem cells contained in a fibrin-based bioengineered dermis for the treatment of epidermolysis bullosa

On 16 December 2014, orphan designation (EU/3/14/1407) was granted by the European Commission to Biodan Yelah S.L., Spain, for allogeneic adipose-derived adult mesenchymal stem cells contained in a fibrin-based bioengineered dermis for the treatment of epidermolysis bullosa.

What is epidermolysis bullosa?

Epidermolysis bullosa describes a group of diseases of the skin, in which the skin is very fragile and forms severe blisters upon minor mechanical friction or injury. The condition is usually present from birth, although some forms occur in adults. The diseases are caused by abnormalities in the genes responsible for the production of certain proteins that make the skin strong and elastic, such as collagen or keratins.

Epidermolysis bullosa is a long-term debilitating and life-threatening condition because the blistering and associated scarring and deformities can be severe, result in poor quality of life and may also reduce life expectancy.

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

At the time of designation, epidermolysis bullosa affected less than 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 41,000 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

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 511,100,000 (Eurostat 2014).

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

How is this medicine expected to work?

This medicine is made up of 'mesenchymal stem cells' that are extracted from the fat tissue of a donor. Mesenchymal stem cells are known to reduce inflammation, and promote the formation of blood vessels and healing of wounds. To make this medicine, the mesenchymal stem cells are embedded and grown in an 'artificial skin' made from a protein called fibrin derived from the donor's blood. The artificial skin is applied as a patch to damaged, blistered areas present in the skin of patients with epidermolysis bullosa. The medicine is expected to help these heal by reducing inflammation, preventing wound infection and increasing the blood supply to the wounded area.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with epidermolysis bullosa had been started.

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 13 November 2014 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 adipose-derived adult mesenchymal stem cells contained in a fibrin-based bioengineered dermis	Treatment of epidermolysis bullosa
Bulgarian	Фибрин-базирана биоинженерна дерма, съдържаща зрели мезенхимни стволови клетки от алогенен произход, извлечени от мастната тъкан	Лечение на булозна епидермолиза
Croatian	Alogene odrasle mezenhimalne matične stanice dobivene iz masnog tkiva koje su sadržane u bioinženjeringom dobivenom dermisu na bazi fibrina	Liječenje bulozne epidermolize
Czech	Dermis na bázi fibrinu zhotovená pomocí bioinženýrských metod obsahuje dospělé, mezenchymální kmenové buňky z tukové tkáně allogenního původu	Léčba bulózní epidermolýzy
Danish	Fibrinbaseret, bioteknologisk fremstillet dermis indeholdende mesenkymale voksenstamfedtcellederivater af allogen oprindelse	Behandling af epidermolysis bullosa
Dutch	Biotechnologische dermis op basis van fibrine met van vetweefsel afgeleide, volwassen mesenchymale stamcellen van allogene herkomst	Behandeling van epidermolysis bullosa
Estonian	Fibriinil põhinev bio-konstrueeritud nahk, mis sisaldab allogeenseid rasvkoest pärinevaid täiskasvanu mesenhümaalseid tüvirakke	Bulloosse epidermolüüsi ravi
Finnish	Fibriinipohjainen biotekniikkaa soveltaen valmistettu verinahka, joka sisältää adipoosista johdettuja täysikasvuisia mesenkyymikantasoluja, joiden alkuperä on allogeeninen	Epidermolysis bullosan hoito
French	Derme bio-ingénierisé basé sur une matrice de fibrine avec des cellules mésenchymales troncales adultes dérivées de tissu adipeux d'origine allogénique	Traitement de l'épidermolyse bulleuse
German	Allogene, aus Fettgewebe abgeleitete mesenchymale Stammzellen, eingebettet in einen biotechnologisch generierten, Fibrin basierten Dermisersatz	Behandlung der Epidermolysis bullosa
Greek	Αλλογενή μεσεγχυματικά βλαστικά κύτταρα προερχόμενα από λιπώδη ιστό, που περιέχονται εντός μίας βιομηχανικής δερμίδας με βάση το ινώδες	Θεραπεία της πομφολυγώδους επιδερμόλυσης

[1] At the time of designation

Language	Active ingredient	Indication
Hungarian	Fibrin-alapú biotechnológiai úton tenyésztett, zsírszövetből izolált allogén eredetű érett mesenchymalis őssejteket tartalmazó dermis	Epidermolysis bullosa kezelése
Italian	Derma bio-ingegnerizzata basata sulla matrice di fibrina con cellule staminali mesenchimali adulte derivate da tessuto adiposo con origine allogenico	Trattamento della epidermolisi bollosa
Latvian	No taukaudiem iegūtas alogēnas izcelsmes pieauguša organisma mezenhīmas cilmes šūnas, kas iekļautas ar biotehnoloģiskām metodēm uz fibrīna bāzes iegūtā dermas slānī	Bulozās epidermolīzes ārstēšanai
Lithuanian	Fibrino pagrindu bioinžinieriniu metodu sukurta oda, turinti alogeninių riebalų išgautų iš suaugusių mezenchimos kamieninių ląstelių	Pūslinės epidermolizės gydymas
Maltese	Ċelloli staminali adulti mesenkimali alloġeniċi imnissla mix-xaħam imrażżnin f'dermis ibbażat fuq il-fibrin maħdum bil-bijoinġinerija	Kura tal-epidermolisi bullosa
Polish	Allogeniczne dorosłe mezenchymalne komórki macierzyste uzyskane z tkanki tłuszczowej zawarte w fibrynowej skórze wytworzonej metodami bioinżynieryjnymi	Pęcherzowe oddzielanie się naskórka
Portuguese	Derme de bioengenharia baseada numa matriz de fibrina com células estaminais adultas derivadas do tecido adiposo alogénico	Tratamento da epidermólise bulhosa
Romanian	Celule stem mezenchimale adulte alogenice derivate din țesutul adipos înglobate în dermă pe bază de fibrină creată prin bioinginerie	Tratamentul epidermolizei buloase
Slovak	Alogénne dospelé mezenchýmové kmeňové bunky v na báze fibrínu bioinžiniersky vyrobenej dermis	Liečba epidermolysis bullosa
Slovenian	Biotehnološko izdelan dermis na fibrinski osnovi, ki vsebuje alogenske tkivne mezenhimske matične celice, pridobljene iz maščobnega tkiva	Zdravljenje bulozne epidrmolize
Spanish	Dermis bio- ingenierizada basada en una matriz de fibrina con células mesenquimales troncales adultas derivadas de tejido adiposo con origen alogénico	Tratamiento de la epidermolisis bullosa
Swedish	Fibrinbaserad, biotekniskt framställd dermis innehållande fettvävnadsderiverade adulta mesenkymala stamceller av allogent ursprung	Behandling av epidermolysis bullosa
Norwegian	Allogene fettvevsderiverte mesenkymale stamceller fra voksne innleiret i en fibrinbasert bioteknisk framstilt lærhud	Behandling av epidermolysis bullosa
Icelandic	Líftæknilega hönnuð leðurhúð á fíbríngrunni sem inniheldur fullvaxnar fituvefsafleiddar ósamgena himnustofnfrumur	Meðferð á epidermolysis bullosa