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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Dry extract from Birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V) for the treatment of epidermolysis bullosa

On 23 February 2011, orphan designation (EU/3/10/845) was granted by the European Commission to Birken GmbH, Germany, for dry extract from birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V) for the treatment of epidermolysis bullosa.

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 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 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, no satisfactory methods were authorised in the EU to treat 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 also used.

[*]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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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?

Dry extract from Birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V) is a herbal product that contains naturally-occurring substances known as triterpenes. Triterpenes are expected to have an effect on the cells in the epidermis, called keratinocytes, by speeding up the multiplication and development of these cells. When applied to wounds and blisters in the form of an ointment, its effect on the keratinocytes is expected to speed up the body's wound repair process.

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 dry extract from Birch bark in patients with epidermolysis bullosa had been started.

At the time of submission, dry extract from Birch bark 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 8 December 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	Dry extract from birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V)	Treatment of epidermolysis bullosa
Bulgarian	Кора от бяла бреза (DER 0.1-0.2:1), сух екстракт, екстракционен разтворител n-хептан 95% (V/V)	Лечение на булозна епидермолиза
Czech	Suchý extrakt z březové kůry (DER 0.1-0.2:1), extrahováno heptanem 95% (V/V)	Léčba bulózní epidermolýzy
Danish	Tørekstrakt fra birkebark (DER 0.1-0.2:1), ekstraktionsmiddel n-heptan 95 % (V/V),	Behandling af epidermolysis bullosa
Dutch	Droog extract van berkenschors (DER 0,1-0,2-1), extractiesolvent n-heptaan 95% (v/v)	Behandeling van epidermolysis bullosa
Estonian	Kasekoore kuivekstrakt(DER 0,1–0,2 : 1), ekstraheeritud 95 % n-heptaaniga (V/V)	Bulloosse epidermolüüsi ravi
Finnish	Koivunkuoresta peräisin oleva kuiva uute (DER 0.1-0.2:1), liuotinaine 95 % n-heptaani (V/V)	Epidermolysis bullosan hoito
French	Extrait sec d'écorce de bouleau (DER 0.1-0.2:1) dans n-heptane à 95 % (v/v)	Traitement de l'épidermolyse bulleuse
German	Betulae cortex-Trockenextrakt (DER 0.1-0.2:1), Auszugsmittel: n-Heptan 95 % (V/V)	Behandlung der Epidermolysis bullosa
Greek	Φλοιός σημύδας (DER 0.1-0.2:1), ξηρό εκχύλισμα ν-επτάνιο 95% (κ.ό.)	Θεραπεία της πομφολυγώδους επιδερμόλυσης
Hungarian	Betulae cortex n-heptánnal (DER 0.1-0.2:1) nyert száraz kivonata 95% (V/V)	Epidermolysis bullosa kezelése
Italian	Corteccia di betulla, estratto secco (DER 0.1-0.2:1) (n-eptanico 95% v/v)	Trattamento della epidermolisi bollosa
Latvian	Bērza mizas sauss ekstrakts (DER 0.1-0.2:1), 95% n heptāns (T/T)	Bulozās epidermolīzes ārstēšanai
Lithuanian	Sausas beržo žievės ekstraktas (DER: 0,1-0,2:1), ekstrahuotas 95 % n-heptano (V/V) tirpikliu	Pūslinės epidermolizės gydymas
Maltese	Estratt xott mill-qoxra tal-betula (DER 0.1-0.2:1), solvent għall-estrazzjoni n-heptane 95% (V/V)	Kura tal-epidermolisi bullosa
Polish	Suchy wyciąg z kory brzozy (DER 0,1-0,2:1), rozpuszczalnik n-heptan 95% (v/v)	Pęcherzowe oddzielanie się naskórka
Portuguese	Extracto seco de casca de bétula (DER 0.1-0.2: 1), solvente de extracção n-heptano a 95% (V/V).	Tratamento da epidermólise bulhosa
Romanian	Extract uscat de scoarţă de mesteacăn (DER 0.1-0.2:1), solvent de extracţie n-heptan 95% (V/V)	Tratamentul epidermolizei buloase
Slovak	Suchý extrakt z brezovej kôry,(DER 0.1-0.2:1), extrakčný roztok – n-heptán 95% (V/V),	Liečba epidermolysis bullosa
Slovenian	suhi ekstrakt brezovega lubja (DER 0.1-0.2:1), ekstrahiran s 95 odstotnim (V/V)	Zdravljenje bulozne epidrmolize

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Extracto seco de corteza de abedul (DER 0.1-0.2:1), solvente de extracción n-heptano 95% (v/v)	Tratamiento de la epidermolisis bullosa
Swedish	Torrextrakt från björkbark (DER 0.1-0.2:1), lösningsmedel n-heptan 95 % (V/V)	Behandling av epidermolysis bullosa
Norwegian	Bjørkebarkekstrakt, tørret. Ekstraksjonsmiddel: n-heptan (95 % V/V) , DER: (0,1-0,2) : 1	Behandling av epidermolysis bullosa
Icelandic	Þurrbirkibarkar, extrakt (DER 0.1-0.2:1), í n heptani 95% (V/V)	Meðferð á epidermolysis bullosa