3 March 2015
EMA/COMP/1161/2014 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allantoin for the treatment of epidermolysis bullosa

First publication	21 March 2014
Rev.1: transfer of sponsorship	3 March 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.	

On 16 January 2014, orphan designation (EU/3/13/1232) was granted by the European Commission to ORS Oxford Ltd, United Kingdom, for allantoin for the treatment of epidermolysis bullosa.

The sponsorship was transferred to Scioderm Limited, Ireland, in October 2014.

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 usually is 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 severe blistering and associated scarring and deformities 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

*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.
At the time of designation, this represented a population of 512,900,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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?

Allantoin has been used for many years in the treatment of wounds and ulcers. It is thought to act by reducing inflammation and stimulating the body's natural mechanisms for removing damaged tissue and encouraging the growth of new replacement tissue. In addition, it may act to prevent the growth of bacteria that can infect damaged areas. In this medicine, these actions could help to heal the blistering associated with epidermolysis bullosa.

What is the stage of development of this medicine?

The effects of allantoin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with allantoin in patients with epidermolysis bullosa were ongoing.

At the time of submission, allantoin was not authorised anywhere in the EU for epidermolysis bullosa. Orphan designation of allantoin had been granted in the USA for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 December 2013 recommending the granting of this designation.

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:

Scioderm Limited
70 Sir John Rogerson's Quay
Dublin 2
Ireland
Tel. +353 1 232 2000
Fax +353 1 232 3333

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	Allantoin	Treatment of epidermolysis bullosa
Bulgarian	алантоин	Лечение на булозна епидермолиза
Croatian	Alantoin	Liječenje bulozne epidermolize
Czech	Allantoin	Léčba bulózní epidermolýzy
Danish	Allantoin	Behandling af epidermolysis bullosa
Dutch	Allantoïne	Behandeling van epidermolysis bullosa
Estonian	Allantoin	Bulloosse epidermolüüsi ravi
Finnish	Allantoiini	Epidermolysis bullosan hoito
French	Allantoïne	Traitement de l'épidermolyse bulleuse
German	Allantoin	Behandlung der Epidermolysis bullosa
Greek	Αλλαντοϊνη	Θεραπεία της πομφολυγώδους επιδερμόλυσης
Hungarian	Allantoin	Epidermolysis bullosa kezelése
Italian	Allantoina	Trattamento della epidermolisi bollosa
Latvian	Allantoīns	Bulozās epidermolīzes ārstēšanai
Lithuanian	Alantoinas	Pūslinės epidermolizės gydymas
Maltese	Allantoin	Kura tal-epidermolisi bullosa
Polish	Alantoina	Pęcherzowe oddzielanie się naskórka
Portuguese	Allantoin	Tratamento da epidermólise bulhosa
Romanian	Alantoina	Tratamentul epidermolizei buloase
Slovak	Alantoín	Liečba epidermolysis bullosa
Slovenian	Alantoin	Zdravljenje bulozne epidrmolize
Spanish	Alantoína	Tratamiento de la epidermolisis bullosa
Swedish	Allantoin	Behandling av epidermolysis bullosa
Norwegian	Allantoin	Behandling av epidermolysis bullosa
Icelandic	Allantóín	Meðferð á epidermolysis bullosa

[1] At the time of designation