4 April 2019
EMADOC-628903358-449

# Public summary of opinion on orphan designation

Losartan for the treatment of epidermolysis bullosa

On 26 February 2019, orphan designation (EU/3/19/2142) was granted by the European Commission to 3R Pharma Consulting GmbH, Germany, for losartan for the treatment of epidermolysis bullosa.

## What is epidermolysis bullosa?

Epidermolysis bullosa is a group of inherited diseases in which the skin is very fragile and forms severe blisters after even minor friction (rubbing) or injury. In most cases, symptoms of epidermolysis bullosa appear from birth, but for some forms, symptoms may not occur until adulthood. The diseases are caused by mutations (changes) 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 reduce life expectancy.

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

At the time of designation, Epidermolysis bullosa affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 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. Good 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 for complications such as deformed hands or skin cancer.

*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 518,400,000 (Eurostat 2019).

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## How is this medicine expected to work?

In patients with epidermolysis bullosa, the severe skin blisters lead to the formation of scars on the skin. Losartan is expected to block the action of a protein called TGFβ which is involved in the processes that cause scarring and thereby reduces the symptoms of the disease.

## What is the stage of development of this medicine?

The effects of losartan have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with losartan in patients with epidermolysis bullosa was ongoing.

At the time of submission, losartan tablets were widely used for the treatment of high blood pressure.

At the time of submission, losartan 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 24 January 2019 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:

Contact details of the current sponsor for this orphan designation can be found on the EMA website.

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

[1] At the time of designation