



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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## Public summary of opinion on orphan designation

### Allogeneic skin-derived ABCB5-positive mesenchymal stem cells for the treatment of epidermolysis bullosa

On 29 May 2019, orphan designation (EU/3/19/2160) was granted by the European Commission to RHEACELL GmbH & Co. KG, Germany, for allogeneic skin-derived ABCB5-positive mesenchymal stem cells (also known as allo-APZ2-EB) 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 epidermolysis bullosa?

At the time of designation, epidermolysis bullosa affected approximately 0.70 in 10,000 people in the European Union (EU). This was equivalent to a total of around 36,000 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

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\*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).



and to protect the skin from damage. Painkillers were also used. Surgery was sometimes necessary for complications such as deformed hands.

## **How is this medicine expected to work?**

This medicine is made up of 'mesenchymal stem cells' that are extracted from the skin of a donor and grown in the laboratory. Although the way these stem cells work in epidermolysis bullosa is not fully known, when injected into the patient they are expected to send signals that reduce inflammation. By reducing inflammation, the medicine is expected to help the blisters in the skin of patients with epidermolysis bullosa to heal.

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

The effects of allogeneic skin-derived ABCB5-positive mesenchymal stem cells 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 the treatment of epidermolysis bullosa or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 17 April 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 EMA website, on the medicine's [rare disease designations page](#).

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<sup>1</sup>, Norwegian and Icelandic

| Language   | Active ingredient                                                                   | Indication                               |
|------------|-------------------------------------------------------------------------------------|------------------------------------------|
| English    | Allogeneic skin-derived ABCB5-positive mesenchymal stem cells                       | Treatment of epidermolysis bullosa       |
| Bulgarian  | Алогенни ABCB5-положителни мезенхимни стволови клетки, получени от кожа             | Лечение на булозна епидермолиза          |
| Croatian   | Alogene ABCB5-pozitivne mezenhimne matične stanice dobivene iz kože                 | Liječenje bulozne epidermolize           |
| Czech      | Alogenní ABCB5-pozitivní mesenchymální kmenové buňky derivované z kůže              | Léčba bulózní epidermolýzy               |
| Danish     | Allogen hud-deriverede ABCB5-positive mesenchymale stamceller                       | Behandling af epidermolysis bullosa      |
| Dutch      | Allogene, uit huid afgeleide ABCB5-positieve mesenchymale stamcellen                | Behandeling van epidermolysis bullosa    |
| Estonian   | Allogeensed nahast saadud ABCB5- positiivsed mesenhümaalsed tüvirakud               | Bulloosse epidermolüüsi ravi             |
| Finnish    | Allogeeniset, ihosta peräisin olevat ABCB5-positiiviset, mesenkymaaliset kantasolut | Epidermolysis bullosan hoito             |
| French     | Cellules souches mésenchymateuses allogéniques dérivées de la peau ABCB5-positives  | Traitement de l'épidermolyse bulleuse    |
| German     | Allogene ABCB5-positive mesenchymale Stammzellen gewonnen aus der Haut              | Behandlung der Epidermolysis bullosa     |
| Greek      | Αλλογενή ABCB5-θετικά μεσεγχυματικά βλαστικά κύτταρα προερχόμενα από δέρμα          | Θεραπεία της πομφολυγώδους επιδερμόλυσης |
| Hungarian  | Allogén bőreredetű ABCB5-pozítív mezenchimális őssejtek                             | Epidermolysis bullosa kezelése           |
| Italian    | Cellule staminali mesenchimali allogeniche ABCB5-positive di origine dermica        | Trattamento della epidermolisi bollosa   |
| Latvian    | Alogēnas, no ādas iegūtas ABCB5-pozitīvas mezenhimālās cilmes šūnas                 | Bulozās epidermolīzes ārstēšanai         |
| Lithuanian | Alogeninės, išskirtos iš odos, ABCB5-teigiamos mezenchimos kamieninės ląstelės      | Pūslinės epidermolizės gydymas           |
| Maltese    | Ċelloli staminali mesenkimali pożittivi għal ABCB5 derivati mill-ġilda alloġenejka  | Kura tal-epidermolisi bullosa            |
| Polish     | Allogeniczne mezenchymalne komórki macierzyste ABCB5-pozytywne pozyskane ze skóry   | Pęcherzowe oddzielenie się naskórka      |
| Portuguese | Células estaminais mesenquimais alogénicas derivadas da pele ABCB5-positivas        | Tratamento da epidermólise bulhosa       |

<sup>1</sup> At the time of designation

| Language  | Active ingredient                                                                  | Indication                              |
|-----------|------------------------------------------------------------------------------------|-----------------------------------------|
| Romanian  | Celule stem mezenchimale alogenice ABCB5- pozitive derivate din piele              | Tratamentul epidermolizei buloase       |
| Slovak    | Alogenické mezenchymálne kmeňové bunky odvodené z kožných ABCB5 pozitívnych buniek | Liečba epidermolysis bullosa            |
| Slovenian | Alogene ABCB5-pozitivne kožne mezenhimske matične celice                           | Zdravljenje bulozne epidermolize        |
| Spanish   | Células madre mesenquimales alogénicas derivadas de la piel ABCB5-positivas        | Tratamiento de la epidermolisis bullosa |
| Swedish   | Allogena ABCB5-positiva mesenkymalstamceller utvunna från hud                      | Behandling av epidermolysis bullosa     |
| Norwegian | Allogene ABCB5-positive mesenkymale stamceller fra hud                             | Behandling av epidermolysis bullosa     |
| Icelandic | Ósamgena ABCB5 jákvæðar bandvefsstofnfrumur úr húð                                 | Meðferð á epidermolysis bullosa         |