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# Public summary of opinion on orphan designation

## Allogeneic ABCB5-positive limbal stem cells for the treatment of limbal stem cell deficiency

On 14 December 2018, orphan designation (EU/3/18/2111) was granted by the European Commission to RHEACELL GmbH & Co. KG, Germany, for allogeneic ABCB5-positive limbal stem cells (also known as LSC2) for the treatment of limbal stem cell deficiency.

### What is limbal stem cell deficiency?

Limbal stem cell deficiency is an eye condition in which the patient lacks cells called limbal stem cells, which are found at the edge of the cornea (the transparent layer in front of the eye) and which continuously renew and repair the cornea. The deficiency of limbal stem cells leads to clouding of the cornea and may result in impaired vision or blindness.

Limbal stem cell deficiency is a long-term debilitating disease because of the impaired vision and discomfort it causes.

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

At the time of designation, limbal stem cell deficiency affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 103,000 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, the medicine Holoclar was authorised to treat patients with moderate to severe limbal stem cell deficiency caused by burns, including chemical burns, to the eyes.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with limbal stem cell deficiency because laboratory studies showed that the medicine could be used in a broader patient population than the currently authorised treatment. This

*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 517,400,000 (Eurostat 2018).

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

assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

## How is this medicine expected to work?

This product is made of limbal stem cells that are obtained from a donor's eye and grown in the laboratory. When the stem cells are placed onto the surface of the patient's damaged eye, they are expected to help the cornea to regenerate, restoring the patient's vision.

## 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 limbal stem cell deficiency had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for limbal stem cell deficiency 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 November 2018 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 | Allogeneic ABCB5-positive limbal stem cells | Treatment of limbal stem cell deficiency |
| Bulgarian | Алогенни ABCB5-положителни лимбални стволови клетки | Лечение на дефицит на лимбални стволови клетки |
| Croatian | Allogene ABCB5-pozitivne limbalne matične stanice | Léčba deficitu limbálních kmenových buněk |
| Czech | Alogenní ABCB5- pozitivní limbální zárodečné buňky | Liječenje nedostatka limbalnih matičnih stanica |
| Danish | Allogen ABCB5-positive limbal stamceller | Behandling af limbal stamcellemangel |
| Dutch | Allogene ABCB5-positieve limbale stamcellen | Behandeling van limbale stamceldeficiëntie |
| Estonian | Allogeensed ABCB5-positiivsed limbaalsed tüvirakud | Limbaalsete tüvirakkude puudulikkuse ravi |
| Finnish | Allogeeniset ABCB5-positiiviset sarveiskalvon kantasolut | Limbaalisen kantasolupuutoksen hoito |
| French | Cellules souches allogéniques ABCB5-positives limbiques | Traitement du déficit en cellules souches limbiques |
| German | Allogene ABCB5-positive limbale Stammzellen | Behandlung der Limbusstammzellinsuffizienz |
| Greek | Αλλογενή βλαστικά κύτταρα κερατοειδούς θετικά για ABCB5 | Θεραπεία βλαστοκυτταρικής ανεπάρκειας του πρόσθιου θαλάμου |
| Hungarian | Allogén ABCB5-pozitív limbal őssejtek | Limbal őssejt hiány kezelése |
| Italian | Cellule staminali limbiche allogeneiche ABCB5-positive | Trattamento del deficit delle cellule staminali limbali |
| Latvian | Alogēnas ABCB5-pozitīvas limbālās cilmes šūnas | Limbālo cilmes šūnu nepietiekamības ārstēšana |
| Lithuanian | Alogeninės *ABCB5-teigiamos limbo kamieninės ląstelės* | Limbo kamieninių ląstelių stokos gydymas |
| Maltese | Ċelloli staminali limbali alloġeniċi pożittivi għal ABCB5 | Kura tan-nuqqas ta' ċelloli staminali limbali |
| Polish | Allogeniczne ABCB5-pozytywne komórki macierzyste rąbka rogówki | Leczenie niedoboru komórek macierzystych rąbka rogówki |
| Portuguese | Células estaminais alogénicas limbares ABCB5 | Tratamento da deficiência de células estaminais limbais |
| Romanian | Celule stem limbice alogenice ABCB5-pozitive | Tratamentul deficientei de celule stem de la nivelul limbului |
| Slovak | Alogenické ABCB5-pozitívne limbálne kmeňové bunky | Liečba deficiencie limbálnych kmeňových buniek |
| Slovenian | Alogene ABCB5 pozitivne limbalne matične celice | Zdravljenje pomanjkanja limbalnih matičnih celic |
| Spanish | Células madres alogénicas limbares ABCB5 | Tratamiento de la deficiencia de células madre limbares |

[1] At the time of designation

| Language | Active ingredient | Indication |
|---|---|---|
| Swedish | Allogena ABCB5-positiva limbala stamceller | Behandling av brist på limbala stamceller |
| Norwegian | Allogene ABCB5-positive limbale stamceller | Behandling av mangel på limbale stamceller |
| Icelandic | Ósamgena ABCB5 jákvæðar stofnfrumur af limbal gerð | Meðferð á limbal stofnfrumu skorti |