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# Public summary of opinion on orphan designation

Besilesomab for treatment in haematopoietic stem cell transplantation

On 17 October 2019, orphan designation EU/3/19/2211 was granted by the European Commission to Therapharm Deutschland GmbH, Germany, for besilesomab for treatment in haematopoietic stem cell transplantation.

## What is haematopoietic stem cell transplantation?

Haematopoietic stem cell transplantation (HSCT) is a procedure where the patient's bone marrow is cleared of cells and replaced by stem cells (cells that can develop into different types of cell) from a donor to form new bone marrow that produces healthy blood cells. It can be used to treat serious diseases of the blood and immune system such as leukaemia.

HSCT can be a debilitating and life-threatening procedure due to the risk of severe infections and developing graft-versus-host disease (when the transplanted cells regard the patient's body as 'foreign' and attack the patient's organs, leading to organ damage). Additionally, in some patients the stem cells do not establish themselves and succeed in forming new bone marrow.

## What is the estimated number of patients receiving haematopoietic stem cell transplantation?

At the time of designation, approximately 0.80 in 10,000 people in the European Union (EU) had received haematopoietic stem cell transplantation. This was equivalent to a total of around 40,000 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, several medicines were authorised in the EU for patients undergoing HSCT. These included radiation treatment or intensive treatment with cancer medicines such as busulfan to clear the bone marrow of existing cells, medicines to help restore the immune system, such as

*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).

**Official address** Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
**Address for visits and deliveries** Refer to www.ema.europa.eu/how-to-find-us
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filgrastim and immunoglobulin replacement therapy, and medicines to reduce the risk of infections, such as antiviral and antifungal medicines. Medicines that suppress the immune system, such as ciclosporin and corticosteroids, were used for the treatment of graft-versus-host disease.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients undergoing haematopoietic stem cell transplantation because early results in patients suggested that when added to standard medicines to clear the bone marrow the medicine increased the likelihood that the HSCT procedure would be successful.

This 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?

In order for new stem cells to successfully establish themselves, the patient's existing bone marrow needs to be cleared using radiation treatment or medicines to destroy the existing bone marrow. Besilesomab is a monoclonal antibody (a type of protein) that targets CD66 proteins found on the surface of bone marrow cells, linked to a component that can carry a radioactive element. When the medicine is loaded with a radioactive substance called yttrium $^{90}$Y and given to the patient it attaches to the bone marrow cells, which are then selectively killed by the radiation from the yttrium. This is expected to improve clearance of the old bone marrow and increase the chances for successful HSCT.

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

The effects of besilesomab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with besilesomab in patients receiving haematopoietic stem cell transplantation were ongoing.

At the time of submission, besilesomab was not authorised anywhere in the EU for treatment in haematopoietic stem cell transplantation 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 12 September 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.

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 | Besilesomab | Treatment in haematopoietic stem cell transplantation |
| Bulgarian | Безилезомаб | Лечение при трансплантация на хемопоетични стволови клетки |
| Croatian | Besilesomab | Liječenje u transplantaciji hematopoetskih matičnih stanica |
| Czech | Besilesomab | Léčba transplantace hemopoetickými zárodečnými buňkami |
| Danish | Besilesomab | Behandling i hæmatopoietisk stamcelletransplantation |
| Dutch | Besilesomab | Behandeling in haematopoiëtische stemceltransplantatie |
| Estonian | Besilesomab | Kasutamiseks hematopoeetiliste tüvirakkude transplantatsiooni ravis. |
| Finnish | Besilesomabi | Hoito hematopoeettisen kantasolusiirron yhteydessä |
| French | Besilesomab | Traitement dans la greffe de moëlle osseuse |
| German | Besilesomab | Behandlung in hämatopoetischer Stammzelltransplantation |
| Greek | Βεσιλεσομάμπη | θεραπεία σε μεταμόσχευση αρχέγονων αιμοποιητικών κυττάρων |
| Hungarian | Besilesomab | Hematopoietikus őssejt-transzplantáció esetén alkalmazandó |
| Italian | Besilesomab | Trattamento nel trapianto di cellule staminali ematopoietiche |
| Latvian | Besilesomabs | Ārstēšanai hematopoētisko cilmes šūnu transplantācijā |
| Lithuanian | Bezilezomabas | Taikoma hematopoetinių kamieninių ląstelių transplantacijų gydyme |
| Maltese | Besilesomab | Kura fi trapjant ta' ċelloli staminali ematopojetiċi |
| Polish | Bezilezomab | Leczenie w przebiegu przeszczepu hematopoetycznych komórek macierzystych |
| Portuguese | Besilesomab | Tratamento em transplantes de células estaminais hematopoiéticas |
| Romanian | Besilesomab | Tratament în transplantul de celule stem hematopoetice |
| Slovak | Besilesomab | Liečba pri transplantácii hematopoietických kmeňových buniek |
| Slovenian | Besilesomab | Zdravljenje pritransplantaciji hematopoetskih matičnih celic |
| Spanish | Besilesomab | Tratamiento en el trasplante de células madre hematopoyéticas |
| Swedish | Besilesomab | Behandling vid hematopoetisk stamcellstransplantation |
| Norwegian | Besilesomab | Behandling ved hematopoetisk stamcelletransplantasjon |
| Icelandic | Besilesomab | Meðferð á stofnfrumublóðfrumu ígræðslu |

[1] At the time of designation