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# Public summary of opinion on orphan designation

## Vocimagene amiretrorepvec for the treatment of glioma

On 22 February 2018, orphan designation (EU/3/18/1987) was granted by the European Commission to Richardson Associates Regulatory Affairs Ltd, United Kingdom, for vocimagene amiretrorepvec (also known as Toca 511) for the treatment of glioma.

### What is glioma?

Glioma is a type of brain tumour that affects the 'glial' cells (the cells that surround and support the nerve cells). Patients with glioma can have severe symptoms, but the types of symptoms depend on where the tumour develops in the brain.

Symptoms can include headaches, nausea (feeling sick), loss of appetite, vomiting, and changes in personality, mood, mental capacity and concentration. About one-fifth of patients with glioma have seizures (fits) for months or years before the disease is diagnosed.

Glioma is a long-term debilitating and life-threatening disease because of the severe damage to the brain, and is associated with poor long-term survival.

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

At the time of designation, glioma affected approximately 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 135,000 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 for the treatment of glioma in the EU. Treatments for glioma included surgery, radiotherapy (treatment with radiation), and chemotherapy (medicines to treat cancer) to improve survival. Patients also received treatments for the symptoms of glioma, including corticosteroids to reduce pressure within the skull and medicines to prevent seizures.

[*]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).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with glioma because early results showed that patients who had glioma that kept coming back responded to treatment with this medicine given together with another medicine called flucytosine. 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?

The medicine is intended to be given to the patient together with another medicine called flucytosine. The medicine is made of a virus that has been genetically modified to contain a gene for the enzyme that converts flucytosine to fluorouracil. Fluorouracil is a well-known chemotherapy that prevents cancer cells from multiplying and the cancer from growing.

This medicine is to be given to patients before they are given flucytosine. The virus is able to target cancer cells but not normal cells. Once inside the cancer cell, the enzyme made by the virus converts flucytosine to fluorouracil, which kills the cancer cell and also helps the immune system (the boy's natural defences) to kill other cancer cells.

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

The effects of the medicine, used together with flucytosine, have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine, used together with flucytosine, in patients with glioma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for glioma. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 January 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 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[1], Norwegian and Icelandic

| Language | Active ingredient | Indication |
|---|---|---|
| English | Vocimagene amiretrorepvec | Treatment of glioma |
| Bulgarian | Воцимаген амиретрорепвец | Лечение на глиома |
| Croatian | Vocimagen amiretrorepvec | Liječenje glioma |
| Czech | Vocimagene amiretrorepvec | Léčba gliomů |
| Danish | Vocimagene amiretrorepvec | Behandling af gliom |
| Dutch | Vocimagene amiretrorepvec | Behandeling van glioma |
| Estonian | Votsimageen amiretrorepvek | Glioomi ravi |
| Finnish | Vocimagene amiretrorepvec | Glioman hoito |
| French | Vocimagène amirétrorépvec | Traitement des gliomes |
| German | Vocimagene Amiretrorepvec | Behandlung von Gliomen |
| Greek | Vocimagene amiretrorepvec | Θεραπεία του γλοιώματος |
| Hungarian | Vocimagen amiretrorepvec | Glioma kezelése |
| Italian | Vocimagene amiretrorepvec | Trattamento del glioma |
| Latvian | Vocimagēna amiretrorepveks | Gliomas ārstēšana |
| Lithuanian | Vocimagenas amiretrorepvekas | Gliomos gydymas |
| Maltese | Voċimaġen amiretrorepvek | Kura tal-glioma |
| Polish | Vocimagene amiretrorepvek | Leczenie glejaka |
| Portuguese | Vocimagene amiretrorepvec | Tratamento do glioma |
| Romanian | Vocimagen amiretrorepvec | Tratamentul gliomului |
| Slovak | Vocimagene amiretrorepvec | Liečba gliómu |
| Slovenian | Vocimagen amiretrorepvec | Zdravljenje glioma |
| Spanish | Vocimagén amiretrorepvec | Tratamiento del glioma |
| Swedish | Vocimagene amiretrorepvec | Behandling av gliom |
| Norwegian | Vocimagene amiretrorepvec | Behandling av gliom |
| Icelandic | Vócimagene amiretrórepvec | Meðferð á glíóma |

[1] At the time of designation