



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Brincidofovir for the prevention of cytomegalovirus disease

On 28 April 2016, orphan designation (EU/3/16/1644) was granted by the European Commission to Chimerix UK Ltd, United Kingdom, for brincidofovir for the prevention of cytomegalovirus disease.

What is cytomegalovirus disease?

Cytomegalovirus is a common virus that usually only causes mild infection such as a sore throat. Most people get infected at some stage during their lifetime but are very often unaware of it. After infection, the virus usually remains in the body in a 'latent' (inactive) state.

However, cytomegalovirus, which may have been latent, can become active again and cause severe infection if the immune system (the body's natural defences) is weakened, such as in people who receive a stem cell transplant.

Cytomegalovirus disease is long-term debilitating and life-threatening because of the complications it causes, such as inflammation of the eyes, lungs, liver and digestive tract, as well as reduced survival of transplanted organs or tissues.

What is the estimated number of patients at risk of developing the condition?

At the time of designation, the number of patients at risk of cytomegalovirus disease was estimated to be approximately 3.2 people in 10,000 per year in the European Union (EU). This was equivalent to a total of around 164,000 people per year*, and is below the ceiling for orphan designation. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

*Disclaimer: For the purpose of the designation, the number of patients at risk of developing 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 513,700,000 (Eurostat 2016).



What methods of prevention are available?

At the time of designation, several medicines were authorised in the EU for the prevention of cytomegalovirus disease (human cytomegalovirus immunoglobulin, ganciclovir, valganciclovir and valganciclovir).

The sponsor has provided sufficient information to show that brincidofovir might be of significant benefit for patients at risk of cytomegalovirus disease because data from the published literature show that this medicine may reduce the occurrence of cytomegalovirus disease in patients undergoing stem-cell transplantation. 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?

Brincidofovir is an antiviral medicine that belongs to the class 'nucleotide analogues'. It blocks the activity of enzymes called 'DNA polymerases' in cytomegalovirus, which the virus uses to produce DNA. When the virus cannot produce DNA, it cannot reproduce, slowing down the spread of infection.

What is the stage of development of this medicine?

The effects of brincidofovir have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with brincidofovir in patients at risk of cytomegalovirus disease were ongoing.

At the time of submission, brincidofovir was not authorised anywhere in the EU for prevention of cytomegalovirus disease 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 23 March 2016 recommending the granting of this designation.

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Brincidofovir	Prevention of cytomegalovirus disease
Bulgarian	Бринцидофовир	Профилактика на цитомегаловирусна болест
Croatian	Brincidofovir	Sprječavanje citomegalovirusne bolesti
Czech	Brincidofovir	Prevence cytomegalovirového onemocnění
Danish	Brincidofovir	Forebyggelse af cytomegalovirus (CMV)-sygdom
Dutch	Brincidofovir	Preventie van cytomegalovirusziekte
Estonian	Brincidofovir	Tsütomegaloviiruse haiguse ennetamine
Finnish	Brinsidofoviiri	Sytomegalovirussairauden ehkäisy
French	Brincidofovir	Prévention des maladies à cytomégalovirus
German	Brincidofovir	Prävention der CMV-Erkrankung
Greek	Μπρινσιντοφοβίρη	Προφύλαξη από τον μεγαλοκυτταροϊό
Hungarian	Brincidofovir	Citomegalovírus betegség megelőzése
Italian	Brincidofovir	Prevenzione della malattia da citomegalovirus
Latvian	Brincidofovir	Citomegalovīrusa slimības profilakse
Lithuanian	Brincidofoviras	Citomegalo viruso sukeltos infekcijos prevencija
Maltese	Brincidofovir	Prevenzjoni ta' mard ikkawżat mill-ċitomegalovirus
Polish	Brincidofovir	Zapobieganie choroby wywołanej przez wirusa cytomegalii
Portuguese	Brincidofovir	Prevenção da doença por citomegalovírus
Romanian	Brincidofovir	Profilaxia bolii produse de cytomegalovirus
Slovak	Brincidofovir	Prevenca cytomegalovírusového ochorenia
Slovenian	Brincidofovir	Preprečevanje citomegalovirusne viremije
Spanish	Brincidofovir	Prevenición de la enfermedad por citomegalovirus
Swedish	Brincidofovir	Förebyggande av cytomegalovirus sjukdom
Norwegian	Brincidofovir	Forebygging av cytomegalovirus sykdom
Icelandic	Brincidófór	Fyrirbyggjandi meðferð gegn cýtómegalóveiru sjúkdómi

¹ At the time of designation