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EMA/COMP/437325/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Brincidofovir for the treatment of adenovirus infection in immunocompromised patients

On 14 July 2016, orphan designation (EU/3/16/1697) was granted by the European Commission to Chimerix UK Ltd, UK, for brincidofovir for the treatment of adenovirus infection in immunocompromised patients.

What is adenovirus infection?

Adenovirus is a common virus that causes a wide range of illnesses such as colds, sore throat and eye infection. Infection with adenoviruses is rarely severe in healthy people but it can lead to life-threatening infections of lung, liver, gut, bladder and brain in immunocompromised patients (patients with weakened immune systems). These include patients who have had haematopoietic (blood) stem cell transplantation or organ transplantation and those with diseases that weaken the immune system, including AIDS.

Adenovirus infection in immunocompromised patients is life threatening and debilitating in the long term due to the spread of severe infection.

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

At the time of designation, adenovirus infection in immunocompromised patients affected approximately 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 154,000 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 for treatment of adenovirus infection in the EU.

^{*}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 513,700,000 (Eurostat 2016).

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 adenovirus, 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 the medicine in patients with adenovirus infection in immunocompromised patients were ongoing.

At the time of submission, brincidofovir was not authorised anywhere in the EU for adenovirus infection in immunocompromised patients 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 16 June 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	Treatment of adenovirus infection in immunocompromised patients
Bulgarian	Бринцидофовир	Лечение на аденовирусна инфекция у имунокомпрометирани пациенти
Croatian	Brincidofovir	Liječenje adenoviruse infekcije imunokompromitiranih bolesnika
Czech	Brincidofovir	Léčení infekci adenovirem u imunokompromitovaných nemocných
Danish	Brincidofovir	Behandling af adenovirusinfektion i immunokompromitterede patienter
Dutch	Brincidofovir	Behandeling van adenovirus infectie in immunogecompromiteerde patiënten
Estonian	Brincidofovir	Adenoviiruse infektsiooni ravi immuunkompromiseeritud patsientidel
Finnish	Brinsidofoviiri	Adenovirusinfektion hoito immuunipuutteisilla potilailla
French	Brincidofovir	Traitement de l'infection par l'adénovirus chez les patients immunocompromis
German	Brincidofovir	Die Behandlung von Adenovirus-Infektion in immunkompromierten Patienten
Greek	Μπρινσιντοφοβίρη	Η θεραπεία της λοίμωξης με αδενοϊό σε ανοσοκατεσταλμένους ασθενείς
Hungarian	Brincidofovir	Immunhiányos betegek adenovírus –fertőzésének kezelése
Italian	Brincidofovir	Trattamento dell'infezione da adenovirus nei pazienti immunocompromessi
Latvian	Brincidofovir	Adenovīrusa infekcijas ārstēšana imūnokompromitētiem pacientiem
Lithuanian	Brincidofovirus	Adenovirusinės infekcijos gydymas imunosupresuotiems pacientams
Maltese	Brincidofovir	Kura ta' infezzjoni minn adenovirus f'pazjenti bl-immunità mdgħajfa
Polish	Brincidofovir	Leczenie infekcji adenowirusowej u pacjentów z obniżoną odpornością
Portuguese	Brincidofovir	Tratamento da infeção por adenovírus em doentes imunodeprimidos
Romanian	Brincidofovir	Tratamentul infecției cu adenovirus la pacienții imunocompromiși
Slovak	Brincidofovir	Liečba infekcie adenovírusom u imunokompromitovaných pacientov
Slovenian	Brincidofovir	Zdravljenje adenovirusne okužbe pri imunsko kompromitiranih pacientih
Spanish	Brincidofovir	Tratamiento de la infección por adenovirus en pacientes inmunodeficientes
Swedish	Brincidofovir	Behandling av adenovirusinfektion i immunokomprimerade patienter
Norwegian	Brincidofovir	Behandling av adenovirus infeksjoner hos immunsupprimerte pasienter
Icelandic	Brincídófór	Meðferð adenóveiru sýkinga hjá ónæmisbældum sjúklingum

¹ At the time of designation