13 December 2016
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Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Brincidofovir for the treatment of smallpox

On 18 November 2016, orphan designation (EU/3/16/1777) was granted by the European Commission to Chimerix UK Ltd, United Kingdom, for brincidofovir for the treatment of smallpox.

### What is smallpox?

Smallpox is a very contagious disease caused by variola virus. Symptoms usually begin with high fever, feeling unwell, headache and backache, followed by development of red spots in the throat and mouth that spread to other parts of the body. The spots then form fluid-filled blisters that scar heavily on healing.

Throughout history, smallpox regularly killed large numbers of people around the world. Following effective vaccination, the disease was declared eradicated in 1980. The main risk of future infections comes from potential bioterrorist attacks or accidents in manipulating live virus stock (two declared live stocks remain in the United States and Russia).

Smallpox is debilitating and life-threatening because it causes scarring of the skin, blindness, excessive bleeding and deformities of the arms and legs and the rate of death is high.

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

At the time of designation, there were no patients affected by smallpox in the European Union (EU). This 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 submission of the application for orphan drug designation, no satisfactory methods were authorised in the EU for the treatment of smallpox.

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## How is this medicine expected to work?

In order for the smallpox virus to multiply and cause disease, copies of its DNA (genetic material) must be made by an enzyme called DNA polymerase. This medicine is expected to block the DNA polymerase of the virus from making new DNA and so prevent the virus from multiplying.

## 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, no clinical trials with brincidofovir in patients with smallpox had been carried out. A clinical trial with brincidofovir in patients infected with other viruses was ongoing.

At the time of submission, brincidofovir was not authorised anywhere in the EU for smallpox 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 6 October 2016 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 | Brincidofovir | Treatment of smallpox |
| Bulgarian | Бринцидофовир | Лечение на едра шарка |
| Croatian | Brincidofovir | Liječenje velikih boginja |
| Czech | Brincidofovir | Léčba neštovic |
| Danish | Brincidofovir | Behandling af kopper |
| Dutch | Brincidofovir | Behandeling van pokken |
| Estonian | Brincidofovir | Rõugete ravi |
| Finnish | Brinsidofoviiri | Isorokon hoito |
| French | Brincidofovir | Traitement de la variole |
| German | Brincidofovir | Behandlung der Pocken |
| Greek | Μπρινσιντοφοβίρη | Θεραπεία της ευλογιάς |
| Hungarian | Brincidofovir | Himlő kezelése |
| Italian | Brincidofovir | Trattamento del vaiolo |
| Latvian | Brincidofovir | Baku ārstēšana |
| Lithuanian | Brincidofoviras | Raupų gydymas |
| Maltese | Brincidofovir | Kura ta ġidri |
| Polish | Brincidofovir | Leczenie ospy prawdziwej |
| Portuguese | Brincidofovir | Tratamento da varíola |
| Romanian | Brincidofovir | Tratamentul variolei |
| Slovak | Brincidofovir | Liečba kiahní |
| Slovenian | Brincidofovir | Zdravljenje črnih koz |
| Spanish | Brincidofovir | Tratamiento de la viruela |
| Swedish | Brincidofovir | Behandling av smittkoppor |
| Norwegian | Brincidofovir | Behandling av kopper |
| Icelandic | Brincídófór | Meðferð við bólusótt |

[1] At the time of designation