

21 March 2014  
EMA/COMP/18107/2014  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Lonafarnib for the treatment of hepatitis delta virus infection

On 16 January 2014, orphan designation (EU/3/13/1225) was granted by the European Commission to Eiger Biopharmaceuticals Europe Limited, United Kingdom, for lonafarnib for the treatment of hepatitis delta virus infection.

#### What is hepatitis delta virus infection?

Hepatitis delta virus infection (hepatitis D) is an infection of the liver with the hepatitis delta virus. The hepatitis delta virus is known as an 'incomplete' virus, because it cannot replicate in cells without the help of another virus, the hepatitis B virus. Because of this, the virus is always present as an additional infection in patients with hepatitis B, and results in higher rates of liver failure and cirrhosis (scarring of the liver) than is seen with hepatitis B infection alone.

Hepatitis delta virus infection is life threatening and debilitating in the long term as it can lead to cirrhosis, liver failure and portal hypertension (high blood pressure in the vessels that connect the liver and the gut).

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

At the time of designation, hepatitis delta virus infection affected not more than 4 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 205,000 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 application for orphan designation, there were no satisfactory treatments for hepatitis delta virus infection in the EU.

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\*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 512,200,000 (Eurostat 2013).

## **How is this medicine expected to work?**

This medicine works by blocking an enzyme in the virus responsible for a chemical reaction called ‘farnesylation’, which involves the addition of a chemical group (farnesyl) to certain proteins. The hepatitis delta virus requires the farnesylation of its major protein in order to reproduce itself. By blocking this reaction, it is expected that the medicine will interfere with the ability of the hepatitis delta virus to reproduce, reducing the amount of the virus in the body and thereby helping to reduce damage to the liver.

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

The effects of lonafarnib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with lonafarnib in patients with hepatitis delta virus infection were ongoing.

At the time of submission, lonafarnib was not authorised anywhere in the EU for hepatitis delta virus infection 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 12 December 2013 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:

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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<sup>1</sup>, Norwegian and Icelandic

| Language   | Active ingredient | Indication                                                     |
|------------|-------------------|----------------------------------------------------------------|
| English    | Lonafarnib        | Treatment of hepatitis delta virus infection                   |
| Bulgarian  | Лонафарниб        | Лечение на хепатит делта вирусна инфекция                      |
| Czech      | Lonafarnib        | Léčba infekce zapříčiněné hepatitis delta virem                |
| Croatian   | Lonafarnib        | Liječenje infekcije virusom hepatitisa delta                   |
| Danish     | Lonafarnib        | Behandling af infektion med hepatitis delta virus              |
| Dutch      | Lonafarnib        | Behandeling van infectie met hepatitis delta virus             |
| Estonian   | Lonafarniib       | D-hepatiidi viiruse infektsiooni ravi                          |
| Finnish    | Lonafarnibi       | Hepatiitti D -virusinfektion hoito                             |
| French     | Lonafarnib        | Traitement de l'infection par le virus de l'hépatite D         |
| German     | Lonafarnib        | Behandlung der Hepatitis-Delta-Virus-Infektion                 |
| Greek      | Λοναφαρνίμπη      | Θεραπεία λοίμωξης από τον ιό της ηπατίτιδας δέλτα              |
| Hungarian  | Lonafarnib        | Hepatitis delta vírus fertőzés kezelése                        |
| Italian    | Lonafarnib        | Trattamento dell'infezione da virus delta dell'epatite         |
| Latvian    | Lonafarnibs       | Hepātīta delta vīrusa infekcijas ārstēšana                     |
| Lithuanian | Lonafarnibas      | Delta viruso sukelta infekcinio hepatito gydymas               |
| Maltese    | Lonafarnib        | Kura ta' infezzjoni mill-virus tal-epatite delta               |
| Polish     | Lonafarnib        | Leczenie wirusowego zapalenia wątroby typu D                   |
| Portuguese | Lonafarnib        | Tratamento da infecção pelo vírus da Hepatite Delta            |
| Romanian   | Lonafarnib        | Tratamentul infecției cu virusul hepatitic delta               |
| Slovak     | Lonafarnib        | Liečba infekcie zapríčinenej vírusom hepatitídy delta          |
| Slovenian  | Lonafarnib        | Zdravljenje okužbe z virusom hepatitisa delta                  |
| Spanish    | Lonafarnib        | Tratamiento de la infección por el virus de la hepatitis delta |
| Swedish    | Lonafarnib        | Behandling av hepatit D-virusinfektion                         |
| Norwegian  | Lonafarnib        | Behandling av hepatitt D-virusinfeksjon                        |
| Icelandic  | Lónafarníð        | Meðferð við sýkingu af völdum delta-lifrabólguveiru            |

<sup>1</sup> At the time of designation