



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Synthetic 47-amino-acid N-myristoylated lipopeptide, derived from the preS region of hepatitis B virus for the treatment of hepatitis delta virus infection

On 19 June 2015, orphan designation (EU/3/15/1500) was granted by the European Commission to MYR GmbH, Germany, for synthetic 47-amino-acid N-myristoylated lipopeptide, derived from the preS region of hepatitis B virus 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 approximately 4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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 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 512,900,000 (Eurostat 2015).



## How is this medicine expected to work?

This medicine is made from a protein found in the outer envelope around the hepatitis B virus. It is expected to work by attaching to, and blocking, a receptor through which the hepatitis B and hepatitis delta viruses enter liver cells. By blocking the entry of hepatitis delta virus into cells, the medicine limits its ability to replicate and its effects in the body, so reducing symptoms of infection.

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

The effects of the medicine 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 hepatitis delta virus infection were ongoing.

At the time of submission, the medicine 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 13 May 2015 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	Synthetic 47-amino-acid N-myristoylated lipopeptide, derived from the preS region of hepatitis B virus	Treatment of hepatitis delta virus infection
Bulgarian	Синтетичен 47 аминокиселинен N-миристоилиран липопептид, извлечен от PreS региона на хепатитния B вирус	Лечение на хепатит делта вирусна инфекция
Croatian	Sintetički N-miristoiliran lipopeptid od 47 aminokiselina, izveden iz preS regije virusa hepatitisa B	Liječenje infekcije virusom hepatitisa delta
Czech	Syntetický 47 aminokyselinový N-myristoylovaný lipopeptid, odvozený z preS oblasti virem hepatitidy B	Léčba infekce zapříčiněné hepatitis delta virem
Danish	Syntetisk 47 aminosyre N-myristoyleret lipopeptid, fra PreS region af hepatitis B virus	Behandling af infektion med hepatitis delta virus
Dutch	Synthetisch 47-aminozuur N-gemyristoyleerde lipopeptide, afgeleid uit de preS regio van hepatitis B virus	Behandeling van infectie met hepatitis delta virus
Estonian	Süntetiline 47-aminohappe N-müristoüleeritud lipopeptiid, mis on saadud saadud B-hepatiidi viiruse preS piirkonnast.	D-hepatiidi viiruse infektsiooni ravi
Finnish	Synteettinen 47-aminohapon N-myristyloitu lipopeptidi, joka on peräisin hepatiitti B-viruksen PreS-alueelta	Hepatiitti D -virusinfektion hoito
French	Lipopeptide synthétique de 47 acides aminés N-myristoylés, dérivé de la région preS du virus de l'hépatite B	Traitement de l'infection par le virus de l'hépatite D
German	Synthetisches N-myristoyliertes Lipopeptid aus 47-Aminosäuren, das aus der PreS-Region des Hepatitis B-Virus abgeleitet ist	Behandlung der Hepatitis-Delta-Virus-Infektion
Greek	Συνθετικό N-μυριστοϋλοποιημένο λιποπεπίδιο 47-αμινοξέων, που προέρχεται από την περιοχή preS του ιού της ηπατίτιδας B	Θεραπεία λοίμωξης από τον ιό της ηπατίτιδας δέλτα
Hungarian	Hepatitis B virus preS régiójából származtatott, szintetikus 47 aminosav hosszúságú N-mirisztilezett lipopeptid	Hepatitis delta vírus fertőzés kezelése
Italian	Lipopeptide sintetico N-Myristoylato di 47-aminoacidi, derivato dalla regione PreS del virus dell'epatite B	Trattamento dell'infezione da virus delta dell'epatite
Latvian	Sintētisks 47 aminoskābju N-miristoilēts lipopeptīds, kas iegūts no B hepatīta vīrusa preS reģiona	Hepatīta delta vīrusa infekcijas ārstēšana

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

Language	Active ingredient	Indication
Lithuanian	Sintetinis 47 aminorūgščių N-miristoilo lipopeptidas, išskirtas iš hepatito B viruso preS srities	Delta viruso sukulto infekcinio hepatito gydymas
Maltese	Lipopeptide sintetiku b' 47-ačidu amminiku u grupp myristoyl magħqud mat-tarf N, imnissel mir-reġjun preS tal-virus tal-epatite B	Kura ta' infezzjoni mill-virus tal-epatite delta
Polish	Syntetyczny 47-aminokwasowy N-mirystylowany lipopeptyd uzyskany z regionu preS wirusa zapalenia wątroby typu B	Leczenie wirusowego zapalenia wątroby typu D
Portuguese	Lipopeptídeo sintético de 47-aminoácidos N-miristoilados, derivado da região preS vírus da Hepatite B	Tratamento da infecção pelo vírus da Hepatite Delta
Romanian	Lipopeptid sintetic compus din 47-aminoacizi N-miristoilați, derivat din regiunea preS a virusului hepatitic B	Tratamentul infecției cu virusul hepatitic delta
Slovak	Syntetický 47-aminokyselínový N-myristoylovaný lipopeptid, odvodený od oblasti preS z vírusu hepatitídy B	Liečba infekcie zapríčinenej vírusom hepatitídy delta
Slovenian	Sintetični 47-aminokislinski N-miristoiliran lipopeptid, izpeljan iz regije preS virusa hepatitsa B	Zdravljenje okužbe z virusom hepatitsa delta
Spanish	Lipopéptido sintético de 47 aminoácidos N-miristolado, derivado de la región preS del virus de la hepatitis B	Tratamiento de la infección por el virus de la hepatitis delta
Swedish	Syntetisk 47-aminosyra N-myristoylerade lipopeptide, från preS-regionen av hepatit B-virus	Behandling av hepatit D-virusinfektion
Norwegian	Syntetisk 47-aminosyre N-myristoylert lipopeptid, avledet fra preS-regionen i hepatitt B virus	Behandling av hepatitt D-virusinfeksjon
Icelandic	Tilbúið 47-aminósýru N-mýristóylað fitupeptíð, afleitt frá PreS-svæðinu í lifrabólgu B veiru	Meðferð við sýkingu af völdum delta-lifrabólguveiru