



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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## Public summary of opinion on orphan designation

### Clofazimine for the treatment of nontuberculous mycobacterial lung disease

On 21 August 2019, orphan designation EU/3/19/2200 was granted by the European Commission to Dr Sebastian Canisius, Germany, for clofazimine for the treatment of nontuberculous mycobacterial lung disease.

#### **What is nontuberculous mycobacterial lung disease?**

Nontuberculous mycobacterial lung disease is an infection of the lungs by nontuberculous mycobacteria.

Nontuberculous mycobacteria (also known as environmental mycobacteria) are bacteria found widely in the environment, such as in water and in soil, but unlike other species of mycobacteria that cause tuberculosis and leprosy, they do not usually cause disease in people. When they do cause disease, patients are infected by coming into direct contact with the bacteria in the environment and not from close contact with people who have the infection.

Lung infection with nontuberculous mycobacteria typically occurs in patients who already have lung disease or who have a weakened immune system. Symptoms include long-term coughing, excess sputum (phlegm) and tiredness and, less commonly, coughing up blood, difficulty breathing, fever and weight loss.

Nontuberculous mycobacterial lung disease is debilitating in the long term because of lung damage.

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

At the time of designation, nontuberculous mycobacterial lung disease affected approximately 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 31,000 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).

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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 518,400,000 (Eurostat 2019).



## **What treatments are available?**

At the time of designation, a number of antibiotics were authorised in the EU for treating nontuberculous mycobacterial lung disease.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with nontuberculous mycobacterial lung disease. Studies have shown that adding this medicine to standard treatments could clear the bacteria faster than using only standard treatments. Furthermore, laboratory studies indicate that this medicine, when inhaled, could be more effective than oral medicines containing the same active substance.

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?**

Clofazimine is an antibiotic that acts against mycobacteria. It does so by interfering with the process through which the bacteria produce energy and by causing the release of toxic substances that stop the bacteria from growing.

Clofazimine has been authorised for use by mouth in the treatment of leprosy (a mycobacterial infection) in some EU countries. As this medicine is to be inhaled, it will be delivered directly to the lungs where its effects are needed.

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

The effects of clofazimine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with this medicine in patients with nontuberculous mycobacterial lung disease had been started.

At the time of submission, use of this medicine by inhalation was not authorised anywhere in the EU for the treatment of nontuberculous mycobacterial lung disease. Orphan designation had been granted in the United States for the treatment of 'pulmonary nontuberculous mycobacterial infections'.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 18 July 2019, 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](#).

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	Clofazimine	Treatment of nontuberculous mycobacterial lung disease
Bulgarian	Клофазимин	Лечение на нетуберкуозна микобактериална белодробна болест
Croatian	Klofazimin	Léčba netuberkulózního mykobakteriálního onemocnění plic
Czech	Clofazimin	Liječenje bolesti pluća uzrokovanih netuberkuloznim mikobakterijama
Danish	Clofazimin	Behandling af non-tuberkulose mykobakteriel lungeinfektion
Dutch	Clofazimine	Behandeling van niet-tuberculeuze mycobacteriële longziekte
Estonian	Klofasimiin	Mittetuberkuloosse mükobakteriaalse kopsuhaiguse ravi
Finnish	Klofatsimiini	Ei-tuberkuloottisten mykobakteerien aiheuttaman keuhkosairauden hoito
French	Clofazimine	Traitement de l'infection pulmonaire à mycobactéries non tuberculeuses
German	Clofazimin	Behandlung von durch nichttuberkulöse Mykobakterien hervorgerufenen Lungenerkrankung
Greek	Κλοφαζιμίνη	Θεραπεία της άτυπης πνευμονικής μυκοβακτηρίωσης
Hungarian	Clofazimine	Nem-tuberkulotikus mikobaktérium (NTM) fertőzés okozta tüdőbetegség kezelése
Italian	Clofazimina	Trattamento della pneumopatia micobatterica non tubercolare
Latvian	Klofazimīns	Netuberkulozo mikobaktēriju izraisītas plaušu slimības ārstēšana
Lithuanian	Klofaziminas	Plaučių ligos, sukeltos netuberkuliozinių mikobakterijų, gydymas
Maltese	Clofazimine	Kura ta' mard tal-pulmun minn mikobatterji li ma jikkawżawx it-tuberkulosi
Polish	Klofazymina	Leczenie choroby płuc wywołanej przez prątki niegruźlicze
Portuguese	Clofazimina	Tratamento de doença pulmonar por micobactérias não tuberculosas
Romanian	Clofazimină	Tratamentul infectilor pulmonare cu micobacterii netuberculoase
Slovak	Klofazimín	Liečba netuberkulózneho mykobakteriálneho ochorenia pľúc
Slovenian	Klofazimin	Zdravljenje netuberkulozne mikobakterijske pljučne bolezni
Spanish	Clofazimina	Tratamiento de la enfermedad pulmonar por micobacterias no tuberculosas
Swedish	Klofazimin	Behandling av icke-tuberkulös mykobakteriell lungsjukdom
Norwegian	Klofazimin	Behandling av ikke tuberkuløs mykobakteriell lungesykdom
Icelandic	Clofazimine	Meðferð við mýkóbakteríal lungnasjúkdómi sem stafar ekki af dæmigerðri berklabakteríu

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

