Content of this Plain Language Summary

The objective of the Plain Language Summary is to help the general public understand EUnetHTA assessments. You can find the link to the full assessment report later in the summary.

What is included in this Plain Language Summary? First, this document explains what EUnetHTA is and what this network does. Second, you will find the summary of the assessment.

What is EUnetHTA?

EUnetHTA is the European Network for Health Technology Assessment (HTA). EUnetHTA was established to create an effective and sustainable network for HTA across Europe. Our partners work together to help develop information to contribute to HTA in European countries. For more information on our goals and how we work, please visit our website and our patient flyer.

EUnetHTA consists of over 80 partners that are all non-profit organisations. All partner organisations either produce or support the production of HTA reports. For more information on HTA, visit EUnetHTA's Frequently Asked Questions.

EUnetHTA does not give any advice on reimbursement of a specific health technology. The reimbursement decision is a national or regional decision. This means that reimbursement of health technologies can also differ between countries in Europe.

What does EUnetHTA do?

EUnetHTA supports national and regional research institutions and health ministries in their decision-making. For this task, EUnetHTA uses specific methods to assess health technologies. Health technologies that may be assessed by EUnetHTA include medicines and other health technologies such as specialist medical care, surgical interventions and diagnostic tests. The purpose of this plain language summary is to help the general public understand the findings from this assessment.

SUMMARY OF THE ASSESSMENT – CEFIDEROCOL, ANTIBIOTIC FOR DIFFICULT TO TREAT INFECTIONS

This section provides a summary of the assessment and was published on 16/06/2020. To get a better understanding of commonly used HTA concepts, we advise you to look at the HTAi glossary.

Why did we conduct this assessment?

The purpose of this EUnetHTA assessment is to give national healthcare systems robust information about the therapy under assessment.

What is the context of this assessment?

Multidrug resistant bacteria (MDR) caused nearly 700,000 infections and 33,000 deaths in the Europe in 2015. This was estimated by the European Centre for Disease Prevention and Control (ECDC). There is a lack of treatment options for this type of infections. Therefore, few guidelines defining the optimal treatment strategies exist. Specific treatment regimens consist often of multiple drug combinations.

Cefiderocol (Fetcroja) is an antibiotic used in adults to treat infections caused by aerobic Gram negative bacteria. This bacteria can lead for example to pneumonia, urinary tract infections and bloodstream infections. This type of infections are difficult to treat. Cefiderocol is used when other treatments may not work.

The current clinical management practice of infections with limited treatment options varies across Europe. This is due to several reasons. Firstly, there are different geographical patterns of treatment resistance. Secondly, there is an assorted availability of drugs at the national level.

Cefiderocol has been granted European Marketing Authorisation on 23/04//2020. It is a treatment given as infusion (drip) into a vein at the hospital.

What did EUnetHTA review?

Through this assessment, EUnetHTA reviewed how well the drug cefiderocol works and how safe it is in patients with difficult to treat infections. This is compared to what is currently used to treat these patients. This assessment included three clinical studies and several pre-clinical studies.

	Scenario 1	Scenario 2
What is the drug under review?	Cefiderocol alone or in combination with other antibiotics	
What is the study group?	Adult patients with a confirmed (antibiotic resistant) infection with limited treatment option.	Adult patients, with life threatening suspected (antibiotic resistant) infection with limited treatment options
What is the drug compared to?	Best available therapy. More information on the different options of best available therapy can be found in the project plan.	
What are the outcomes this review investigates?	Outcomes on effectiveness of the drug: • Cure from infection (at different time-points) • Elimination of bacteria • Combination of these two • Mortality Outcomes on *Safety and side effects* • mild and severe side effects, such as side effects leading to drug discontinuation or death. • Quality of life.	

What are the main findings?

The assessment consists of three randomised clinical trials, two in vitro (*means: grown in a laboratory*) studies, and six clinical pharmacology studies (*this is a study to investigate how the drug works*). The studies were identified through a systematic search of this topic. This search included all studies published up until 19/12/2019. A total of 904 people were included in these studies. Studies were located in both European and non-European countries.

Comparing the clinical studies was challenging. While all participants had a relevant type of infection, the study population, study design, type of antibiotic used and definition of infection varied between studies. Furthermore, researched outcomes were not consistent among the included studies. As a consequence, it was not possible to conduct a complete assessment of cefiderocol with best available therapies.

This assessment may support national decisions for the treatment of patients with a difficult to treat infection regardless the bacteria that causes the (site of) infection. However, current clinical management practice of infections with limited treatment options varies widely across Europe. The authors emphasize the importance of national appraisal of the results of this report. The reasons for this are explained above under *"What is the context of this assessment"*.

Did EUnetHTA involve stakeholders?

EUnetHTA values involvement of stakeholders in the assessments. This ensures the assessments consider/include patient's experiences and improves applicability of the assessments. Patient associations were invited to provide input at the initial stage of this assessment. However, no patient organisation completed the call for their input. Medical specialists were also invited to provide input on this assessment.

Additional information

This report was written by HTA organisations from Norway and The Netherlands. Organizations from France, Spain and Germany have contributed in reviewing roles. The full scientific content is reported in EUnetHTA assessment PTJA11 and can be found here. EUnetHTA has received funding from the European Union's Health Programme (2014-2020). The content of this summary reflects the views of the authoring team. This cannot be considered to reflect the views of the entire EUnetHTA or any body of the European Union. Individuals involved in this assessment were cleared for any potential conflict of interests.

If you have further questions, please contact: eunethta@zinl.nl