国立保健医療科学院　保健医療経済評価研究センター
Center for Outcomes Research and Economic Evaluation for Health (C2H), National Institute of Public Health (NIPH) | URL:http://c2h.niph.go.jp

2023 年 4 月 4 日

[C2H2105] Summary of cost-effectiveness evaluation of amikacin sulfate (ARIKAYCE®)

1. Indications

Nontuberculous mycobacterial (NTM) lung infections caused by Mycobacterium avium complex (MAC)

2. Price of the drug

Amikacin sulfate (ARIKAYCE®) has been reimbursed from May 2021 at JPY 42408.40 (as of January 2022). The price is calculated using a cost calculation method with a usefulness premium (II) of 10%. This product s designated as a H1 cost-effectiveness evaluation item.

3. Scope of cost–effectiveness evaluation

This product is indicated for the treatment of non-tuberculous mycobacterial (NTM) lung infections caused by Mycobacterium avium complex (MAC) as part of a combination antibacterial drug regimen in patients who do not achieve negative sputum cultures after a minimum of 6 consecutive months of multidrug background regimen therapy*. The scope of evaluation agreed upon at the first session of the Expert Committee of Cost-Effectiveness Evaluation (ECCEE) is described below.

Population	Patients with MAC lung disease after a minimum of 6 consecutive months of a multidrug background regimen therapy
Comparator	Multidrug regimen (Selected technology： ARIKAYCE® + Multidrug regimen)

*3-drug regimen including rifamycin, ethambutol, and clarithromycin

4. Evaluation of additional benefits

In this systematic review, the randomized INS-212 trial was detected. The INS-212 trial is a Phase 3 trial involving patients with refractory MAC lung disease(n=336). Patients were randomized to receive either ARIKAYCE plus a background regimen (n=223) or a background regimen alone (n=112). The primary endpoint of sputum culture conversion was significantly greater for ARIKAYCE plus a background regimen than for a background regimen alone (29.0% vs. 8.9%; adjusted odds ratio, 4.22; 95% CI, 2.08–8.57; P < 0.001). In addition, the number of participants who achieved sustained culture conversion at the end of treatment was greater in the ARIKAYCE plus background regimen arm.

Consequently, the academic group concluded that ARIKAYCE has additional benefits for the comparator.

5. Results of the cost-effectiveness analysis

The manufacturer estimated the cost effectiveness using the simulation model. For QOL values, the manufacturer evaluated the presented health scenario (MAC-negative/MAC-positive) for the general population using the TTO (time-trade off) method. The Guideline for Preparing Cost-Effectiveness Evaluation to the Central Social Insurance Medical Council states that "it is difficult to directly collect QOL scores from subjects, it is acceptable for general people to evaluate the presented health scenario by direct methods." However, because QOL values were measured using the EQ-5D-3L in the INS-212 trial, the academic group concluded that it was not difficult to measure QOL values for patients with MAC lung disease. In addition, the Guideline for preparing Cost-Effectiveness Evaluation state that "When the QOL score is assessed by PBM, the subjects' own QOL responses should be used." Thus, the academic group applied the results of the INS-212 trial in which QOL values were measured using the EQ-5D-3L.

The academic group modified the quality of life (QOL) score and recurrence rate in patients with MAC lung disease. The ECCEE accepted the following criteria:

Population	Comparator	ICER (JPY/QALY)
Patients with MAC lung disease after a minimum of 6 consecutive months of multidrug background regimen therapy	Multidrug regimen	11,135,395