MEDICAL MUTUAL®

Drug Policy

Policy:	Arikayce (amikacin liposome inhalation suspension for oral inhalation)	Annual Review Date: 10/19/2023 Last Revised Date: 10/19/2023

OVERVIEW

Arikayce is indicated for the treatment of ***Mycobacterium avium* complex (MAC) lung disease**, in adults who have limited or no alternative treatment options, as part of a combination antibacterial regimen in patients who do not achieve negative sputum cultures after at least six consecutive months of a background multidrug regimen (MDR) therapy. As only limited clinical safety and efficacy data is available, reserve Arikayce for adults with limited or no other treatment options. This indication is approved under accelerated approval based on achieving sputum culture conversion (defined as three consecutive negative monthly sputum cultures) by Month 6.

Limitation of Use: Arikayce has only been studied in patients with refractory MAC lung disease defined as not achieving culture negativity after at least 6 months of background MDR treatment.[1] Arikayce is not recommended in patients with non-refractory MAC lung disease.

POLICY STATEMENT

This policy involves the use of Arikayce. Prior authorization is recommended for pharmacy benefit coverage of Arikayce. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Arikayce as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Arikayce be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Arikayce is recommended in those who meet the following criteria:

FDA-Approved Indications

1. ***Mycobacterium avium* Complex (MAC) Lung Disease.** Approve for the duration noted if the patient meets ONE of the following criteria (A or B):

A) Initial Therapy. Approve for 1 year of the patient meets the following criteria (i, ii, iii, iv, and v):
- **i.** Patient is ≥ 18 years of age; AND
- **ii.** Patient has completed ≥ 6 consecutive months of a background multidrug regimen; AND
  Note: A multidrug regimen typically includes a macrolide (azithromycin or clarithromycin), ethambutol, and a rifamycin (rifampin or rifabutin).
- **iii.** Patient has a positive sputum culture for *Mycobacterium avium* complex; AND
- **iv.** The culture meets BOTH of the following criteria (a and b):
  - **a)** Culture was collected within the past 3 months; AND
  - **b)** Culture was collected AFTER the patient has completed ≥ 6 consecutive months of a background multidrug regimen; AND
- **v.** The *Mycobacterium avium* complex isolate is susceptible to amikacin with a minimum inhibitor concentration (MIC) of ≤ 64 µg/mL; AND
- **vi.** Arikayce will be used in conjunction with a background multidrug regimen; AND
  Note: A multidrug regimen typically includes a macrolide (azithromycin or clarithromycin), ethambutol, and a rifamycin (rifampin or rifabutin).
- **vii.** The medication is prescribed by a pulmonologist, infectious diseases physician or a physician who specializes in the treatment of *Mycobacterium avium* complex lung infections.

B) Patient is Currently Receiving Arikayce. Patient meets both of the following criteria (i or ii):
- **i.** Arikayce will be used in conjunction with a background multidrug regimen; AND
  Note: A multidrug regimen typically includes a macrolide (azithromycin or clarithromycin), ethambutol, and a rifamycin (rifampin or rifabutin).
- **ii.** Patient meets ONE of the following criteria (a or b):
  - a) Approve for 1 year if patient has not achieved negative sputum cultures for *Mycobacterium avium* complex; OR
  - b) Approve for 1 year (total) if patient has achieved negative sputum cultures for *Mycobacterium avium* complex for less than 12 months; AND
    Note: Approve enough Arikayce to complete 12 months of therapy following a negative sputum culture for *Mycobacterium avium* complex.

Other Uses with Supportive Evidence

2. Cystic Fibrosis. Approve for 1 year if the patient meets the following criteria (A and B):
- **A)** Patient has *Pseudomonas aeruginosa* in culture of the airway (e.g., sputum culture, oropharyngeal culture, bronchoalveolar lavage culture); AND
- **B)** The medication is prescribed by or in consultation with a pulmonologist or a physician who specializes in the treatment of cystic fibrosis.

Initial Approval/ Extended Approval.
A) *Initial Approval:* 365 days
B) *Extended Approval:* 365 days

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Arikayce has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

1. Arikayce [prescribing information]. Bridgewater, NJ: Insmed; March 2020.
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4. Daley CL. Mycobacterial avium Complex Disease. *Microbiol Spectrum*. 2017;5: TNMI7-0045-2017. doi:10.1128/microbiolspec.TNMI7-0045-2017.
5. Prevots DR, Marras TK. Epidemiology of Human Pulmonary Infection with Non-Tuberculous Mycobacteria: A Review. *Clin Chest Med*. 2015;36:13-34.
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10. Okusanya OO, Bhavnani SM, Hammel JP, et al. Evaluation of the Pharmacokinetics and Pharmacodynamics of Liposomal Amikacin for Inhalation in Cystic Fibrosis Patients with Chronic Pseudomonal Infections Using Data from Two Phase 2 Clinical Studies. *Antimicrob Agents Chemother*. 2014;58:5005-5015.