Policy and Procedure	
PHARMACY PRIOR AUTHORIZATION POLICY AND CRITERIA ORPTCINF034.1225	**ANTI-INFECTIVE AGENTS** **ARIKAYCE®** (amikacin liposome inhalation suspension) 590 mg/8.4 ml
Effective Date: 2/1/2026	**Review/Revised Date**: 01/19, 10/19, 10/20, 11/21, 11/22, 11/23, 10/24, 10/25 (MTW)
Original Effective Date: 04/19	**P&T Committee Meeting Date**: 02/19, 12/19, 10/20, 12/21, 12/22, 12/23, 12/24, 12/25
Approved by: Oregon Region Pharmacy and Therapeutics Committee	

SCOPE:
Providence Health Plan and Providence Health Assurance as applicable (referred to individually as "Company" and collectively as "Companies").

APPLIES TO:
Commercial
Medicaid

POLICY CRITERIA:

COVERED USES:
All Food and Drug Administration (FDA) approved indications not otherwise excluded from the benefit.

Coverage for Medicaid is limited to a condition that has been designated a covered line item number by the Oregon Health Services Commission listed on the Prioritized List of Health Care Services when all applicable indication-specific criteria below are met or if the Early and Periodic Screening, Diagnostic and Treatment (EPSDT) benefit applies.

REQUIRED MEDICAL INFORMATION:
Initial authorization requires all the following:

1. Documentation of a confirmed diagnosis of *Mycobacterium avium* complex (MAC) lung infection by MAC-positive sputum or bronchoscopy cultures
   AND
2. Documentation that the patient is unable to achieve negative sputum cultures after a minimum of six consecutive months of a standard guideline-based therapy (GBT). Guideline-based therapy is a three-drug oral antibiotic regimen composed of a macrolide (clarithromycin or azithromycin), ethambutol and a rifamycin (rifabutin)
   AND
3. Documentation that organism is susceptible to amikacin

Reauthorization requires documentation of negative sputum cultures

EXCLUSION CRITERIA: N/A

AGE RESTRICTIONS:
Approved for patients 18 years of age and older

PRESCRIBER RESTRICTIONS:
Must be prescribed by, or in consultation with, an infectious disease specialist or pulmonologist

COVERAGE DURATION:
Initial authorization and reauthorization will be approved for six months

QUANTITY LIMIT:
28 vials per 28 days (8.4 ml/day)

Requests for indications that were approved by the FDA within the previous six (6) months may not have been reviewed by the health plan for safety and effectiveness and inclusion on this policy document. These requests will be reviewed using the New Drug and or Indication Awaiting P&T Review; Prior Authorization Request ORPTCOPS047.

Requests for a non-FDA approved (off-label) indication requires the proposed indication be listed in either the American Hospital Formulary System (AHFS), Drugdex, or the National Comprehensive Cancer Network (NCCN) and is considered subject to evaluation of the prescriber's medical rationale, formulary alternatives, the available published evidence-based research and whether the proposed use is determined to be experimental/investigational.

Coverage for Medicaid is limited to a condition that has been designated a covered line item number by the Oregon Health Services Commission listed on the Prioritized List of Health Care Services.

Coverage decisions are made on the basis of individualized determinations of medical necessity and the experimental or investigational character of the treatment in the individual case.

INTRODUCTION:
Amikacin liposomal inhalation suspension (Arikayce®) is an inhaled antibiotic medication to be given as an add-on therapy in adult patients with serious lung infection caused by *Mycobacterium avium* complex (MAC) that have not responded to standard treatment alone.

FDA APPROVED INDICATIONS:
LIMITED POPULATION: Treatment of *Mycobacterium avium* complex (MAC) lung disease in adults who have limited or no alternative treatment options, 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 a multidrug background regimen therapy.

POSITION STATEMENT:

- Nontuberculous mycobacteria (NTM) are widespread environmental organisms that cause lung disease primarily in individuals with chronic underlying pulmonary pathologies, such as bronchiectasis and chronic obstructive pulmonary disease. *Mycobacterium avium* complex (MAC) bacteria are the most common cause of NTM lung disease in North America.
- Practice guidelines issued by the American Thoracic Society, the European Respiratory Society, the European Society of Clinical Microbiology and Infectious Diseases and the Infectious Diseases Society of America (ATS/ERS/ESCMID/IDSA) recommend the following for MAC lung disease:[8]
    - Initial guideline-based therapy (GBT) includes a three-drug antibiotic regimen composed of a macrolide, ethambutol, and a rifamycin.
    - Parenteral amikacin or streptomycin for two to three months of therapy should be considered for cavitary or advanced/severe bronchiectatic or macrolide-resistant MAC pulmonary disease as part of the initial treatment regimen. Major side effects/toxicity associated with amikacin or streptomycin are vestibular/auditory toxicity (dizziness, vertigo, ataxia, tinnitus and hearing loss).
    - Inhaled amikacin should not be used as part of the initial treatment regimen.
    - Amikacin liposome inhalation suspension is recommended to be added to the treatment regimen after failure of at least six months of guideline-based therapy. Inhaled parenteral amikacin can be an alternative if liposome inhalation suspension is not available.
    - The goals of therapy include symptomatic, radiographic, and microbiologic improvement.
    - The microbiologic goal of treatment is 12 months of negative sputum cultures while on GBT.
- Amikacin liposomal inhalation suspension (Arikayce®) was approved under accelerated approval based on a phase 3, randomized, open-label clinical trial demonstrating sputum culture conversion (defined as three consecutive negative monthly sputum cultures) by Month six in adult patients with refractory MAC. *Clinical benefit has not yet been established.* Post-marketing clinical trials are pending to confirm clinical benefit.[1,2]
- Although inhaled amikacin liposomal formulation allows direct localized drug delivery and potentially minimize systemic toxicity associated with IV aminoglycosides, there have not been clinical trials assessing the efficacy and

safety of the liposomal formulation compared with the injectable formulation of amikacin, either inhaled or parenterally administered.

- Amikacin liposomal inhalation suspension (Arikayce®) carries a boxed warning of *risk of increased respiratory adverse reactions*. There were six reports (2.7%) of death from treatment-emergent adverse events in the phase 3 clinical trial – respiratory failure (n=2), COPD exacerbation (n=1), pulmonary embolism (n=1), lung infection (n=1) and cachexia (n=1).[1]
- Due to limited clinical safety and effectiveness data, amikacin liposomal inhalation suspension (Arikayce®) should only be used in adults who have limited or no alternative treatment options. This medication is not recommended in patients with non-refractory MAC lung disease or other types of lung infections (e.g., *Pseudomonas aeruginosa* in patients with cystic fibrosis).
- The recommended dosage in adults is one 590 mg/8.4 ml vial once daily via oral inhalation and only with the Lamira Nebulizer System. The device is distributed by the specialty pharmacy with the first medication dispense. No separate billing is required on the device.[1]

Early and Periodic Screening Diagnostic and Treatment (EPSDT) Review

The Early and Periodic Screening, Diagnostic and Treatment (EPSDT) benefit includes comprehensive preventative health care services for Medicaid members until they turn age 21 and for members with qualifying special health care needs (Youth with Special Healthcare Needs (YSHCN)) as they turn 21. This benefit applies when a condition is determined to impact the ability to grow, develop or participate in school and the applicable criteria above are met.

REFERENCE/RESOURCES:

1. Arikayce® (amikacin liposome inhalation suspension) package insert.
2. Griffith DE, Eagle G, Thomson R et al, Amikacin Liposome Inhalation Suspension for Treatment-Refractory Lung Disease Caused by Mycobacterium avium Complex (CONVERT): A Prospective, Open-Label, Randomized Study. Am J Respir Crit Care Med. 2018 Sep 14. doi: 10.1164/rccm.201807-1318OC. (PMID: 30216086)
3. Olivier KN, Griffith DE, Eagle G et al. Randomized Trial of Liposomal Amikacin for Inhalation in Nontuberculous Mycobacterial Lung Disease. Am J Respir Crit Care Med. 2017 Mar 15;195(6):814-823. (PMID: 27748623)
4. Study to Evaluate Arikayce™ in CF Patients With Chronic Pseudomonas Aeruginosa Infections. (2015). Retrieved from https://clinicaltrials.gov/ct2/show/NCT01315678?term=01315678&rank=1 (Identification No. NCT01315678)
5. Extension Study of Arikayce in Cystic Fibrosis (CF) Patients With Chronic

Pseudomonas Aeruginosa Infection. (2016) Retrieved from https://clinicaltrials.gov/ct2/show/NCT01316276?term=01316276&rank=1 (Identification No. NCT01316276)

6. Griffith DE, Aksamit T, Brown-Elliott BA et al, An official ATS/IDSA statement: diagnosis, treatment, and prevention of nontuberculous mycobacterial diseases. Am J Respir Crit Care Med. 2007 Feb 15;175(4):367-416.
7. Shannon Kasperbauer, MDCharles L Daley, MD (2024). Treatment of Mycobacterium avium complex lung infection in adults. In J. A. Melin (Ed.), UpToDate. Retrieved October 15, 2025, from https://www.uptodate.com/contents/treatment-of-mycobacterium-avium-complex-lung-infection-in-adults
8. Daley CL, Iaccarino JM, Lange C *et al*. Treatment of Nontuberculous Mycobacterial Pulmonary Disease: An Official ATS/ERS/ESCMID/IDSA Clinical Practice Guideline. *Clinical Infectious Diseases*. 2020;71(4):e1-e36.