Clinical Policy: Amikacin (Arikayce)

Reference Number: CP.PHAR.401
Effective Date: 11.13.18
Last Review Date: 08.25
Line of Business: Commercial, HIM, Medicaid Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

Description

Amikacin (Arikayce®) is a liposomal formulation of amikacin – an aminoglycoside antibiotic.

FDA Approved Indication(s)

Arikayce is indicated in adults who have limited or no alternative treatment options, for the treatment of *Mycobacterium avium* complex (MAC) lung disease 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. As only limited clinical safety and effectiveness data for Arikayce are currently available, reserve Arikayce for use in adults who have limited or no alternative treatment options. This drug is indicated for use in a limited and specific population of patients.

This indication is approved under accelerated approval based on achieving sputum culture conversion (defined as 3 consecutive negative monthly sputum cultures) by Month 6. Clinical benefit has not yet been established.

Limitation(s) of use: Arikayce has only been studied in patients with refractory MAC lung disease defined as patients who did not achieve negative sputum cultures after a minimum of 6 consecutive months of a multidrug background regimen therapy. The use of Arikayce is not recommended for patients with non-refractory MAC lung disease.

Policy/Criteria

It is the policy of health plans affiliated with Centene Corporation® that Arikayce is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Mycobacterium Avium Complex Lung Disease (must meet all)**:**

1. Diagnosis of MAC lung disease;
2. Prescribed by or in consultation with an infectious disease specialist or pulmonologist;
3. Age ≥ 18 years;
4. Failure, as evidenced by positive sputum culture, of at least a 6-month trial of a multidrug background regimen therapy at up to maximally indicated doses *(see Appendix B)*, unless all are contraindicated or clinically significant adverse effects are experienced;
5. Dose does not exceed one vial per day.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy

A. Mycobacterium Avium Complex Lung Disease (must meet all)**:**
1. Currently meets one of the following (a or b):
   a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
   b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B);*
2. Confirmation of at least 3 consecutive negative monthly sputum cultures in the first 6 months of therapy or at least 2 consecutive negative monthly sputum cultures in the last 2 months of therapy;
3. Member has not received more than 12 months of treatment following conversion to negative sputum status;
4. If request is for a dose increase, new dose does not exceed one vial per day.

Approval duration: Up to a total of 12 months of treatment after converting to negative sputum status

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or

b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or

2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

FDA: Food and Drug Administration
MAC: mycobacterium avium complex

Appendix B: Therapeutic Alternatives

This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
clarithromycin (Biaxin®) or azithromycin (Zmax®) + ethambutol (Myambutol®) + rifampin (Rifadin®)	Variable dosing	Combo used for initial therapy for nodular/bronchiectasis disease
clarithromycin (Biaxin®) or azithromycin (Zmax®) + ethambutol (Myambutol®) + rifampin (Rifadin®) + streptomycin or amikacin (Amikin®) or none.	Variable dosing	Combo used for initial therapy for cavitary disease
clarithromycin (Biaxin®) or azithromycin (Zmax®) + ethambutol (Myambutol®) + rifampin (Rifadin®) or rifabutin (Mycobutin®) + streptomycin or amikacin (Amikin®)	Variable dosing	Combo used for advanced (severe) or previously treated disease

Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic.

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): a known hypersensitivity to any aminoglycoside.

- Boxed warning(s): risk of increased respiratory adverse reactions, including, hypersensitivity pneumonitis, hemoptysis, bronchospasm, and exacerbation of underlying pulmonary disease that have led to hospitalization in some cases.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
MAC	Oral inhalation of the contents of one 590 mg/8.4 mL Arikayce vial per day	590 mg/8.4 mL per day

VI. Product Availability

Suspension for oral inhalation, unit-dose vial: 590 mg/8.4 mL

VII. References

1. Arikayce Prescribing Information. Bridgewater, NJ: Insmed; February 2023. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2023/207356s012lbl.pdf. Accessed April 17, 2025.
2. Olivier KN, et al. Randomized Trial of Liposomal Amikacin for Inhalation in Nontuberculous Lung Disease. American Journal of Respiratory and Critical Care Medicine. 195;6. March 15, 2017: 814-823.
3. Griffith DE, et al. Amikacin Liposome Suspension for Treatment-Refractory Lung Disease Caused by Mycobacterium Avium Complex (CONVERT): A Prospective, Open-Label, Randomized Study. American Journal of Respiratory and Critical Care Medicine. September 2018. doi: 10.1164/rccm.201807-1318OC.
4. Arikayce Drug Monograph. Clinical Pharmacology. Available at: www.clinicalkeys.com/pharmacology. Accessed May 13, 2025.
5. Griffith DE, et al. An Official ATS/IDSA Statement: Diagnosis, Treatment, and Prevention of Nontuberculous Mycobacterial Diseases. American Journal of Respiratory and Critical Care Medicine. 2007;175:367-416
6. Haworth CS, Banks J, Capstick T, et al. British Thoracic Society guidelines for the management of non-tuberculous mycobacterial pulmonary disease. Thorax 2017;72:ii1–ii64.
7. 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(15 August): e1-e36.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
1Q 2021 annual review: no significant changes; references to HIM.PHAR.21 revised to HIM.PA.154; references reviewed and updated.	11.24.20	02.21
1Q 2022 annual review: added requirement that member has not received more than 12 months of treatment following conversion to negative sputum status to support existing continued authorization coverage duration requirements; references reviewed and updated.	09.15.21	02.22
Template changes applied to other diagnoses/indications and continued therapy section.	09.23.22	

Reviews, Revisions, and Approvals	Date	P&T Approval Date
1Q 2023 annual review: no significant changes; references reviewed and updated.	10.04.22	02.23
3Q 2023 annual review: no significant change; references reviewed and updated.	04.12.23	08.23
Per March SDC, extended initial authorization approval duration from 6 months to 12 months; revised continued therapy criteria language for negative monthly sputum cultures requirement from "documentation" to "confirmation" (which can be determined by documentation or attestation); removed description of policy/criteria that stated "Provider must submit documentation…"	03.12.24	05.24
3Q 2024 annual review: no significant change; references reviewed and updated.	05.06.24	08.24
3Q 2025 annual review: no significant change; references reviewed and updated.	04.17.25	08.25

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note:
For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.