Generic Name:
Amikacin liposome inhalation suspension
Trade Name:
Arikayce
Company:
Insmed
Notes:

FDA approved amikacin liposome inhalation suspension to treat lung disease caused by a group of bacteria, Mycobacterium avium complex (MAC), in a limited population of patients with the disease who do not respond to conventional treatment. The drug is an inhaled treatment taken through a nebulizer.

MAC is a type of nontuberculous mycobacteria (NTM) commonly found in water and soil. Symptoms of disease in patients with MAC include persistent cough, fatigue, weight loss, night sweats, and occasionally, shortness of breath and coughing up of blood.

It is the first drug to be approved under the Limited Population Pathway for Antibacterial and Antifungal Drugs, or LPAD pathway, established by Congress under the 21st Century Cures Act to advance development and approval of antibacterial and antifungal drugs to treat serious or life-threatening infections in a limited population of patients with unmet need. Approval under the LPAD pathway may be supported by a streamlined clinical development program. These programs may involve smaller, shorter, or fewer clinical trials. As required for drugs approved under the LPAD pathway, the labeling includes certain statements to convey that the drug has been shown to be safe and effective only for use in a limited population.

Approval was based on achieving three consecutive negative monthly sputum cultures by month six of treatment. FDA requires the sponsor to conduct an additional postmarketing study to describe the drug's clinical benefits.

Safety and efficacy were demonstrated in a randomized, controlled clinical trial in which patients were assigned to one of two treatment groups: one group receiving amikacin plus a background multidrug antibacterial regimen, and the other group receiving a background multidrug antibacterial regimen alone.

By the sixth month of treatment, 29% percent of patients treated with amikacin had no growth of mycobacteria in their sputum cultures for three consecutive months, compared with 9% of patients who were not treated with amikacin.

The prescribing information includes a boxed warning about the increased risk of respiratory conditions. Other common adverse effects are difficulty speaking, cough, damaged hearing, upper airway irritation, musculoskeletal pain, fatigue, diarrhea, and nausea.

Medication Monitor Categories:
New Drug Approvals