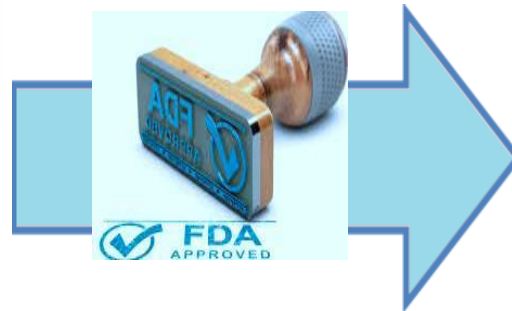


US FDA approved ARIKAYCE (amikacin liposome inhalation suspension) antibacterial drug to treat a serious lung disease approved on 28th Sept 2018



FDA approves a new antibacterial drug to treat a serious lung disease using a novel pathway to spur innovation



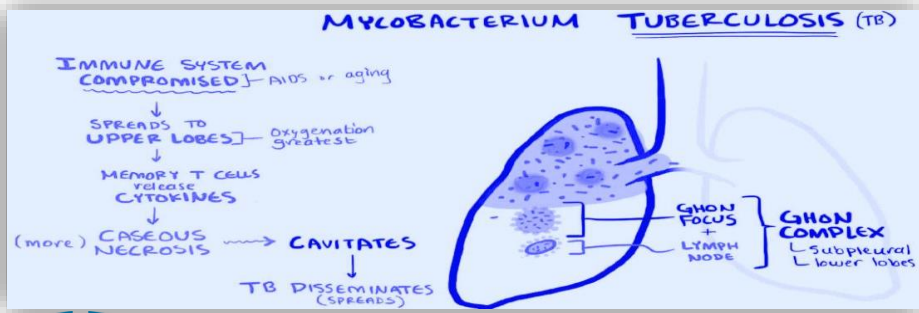
Lung Infection due to MAC Burden

Lung Infection Signs & Symptoms

Symptoms such as: cough, sputum production, weight loss, fever, lethargy, and night sweats

Mycobacterium Avium Infection:

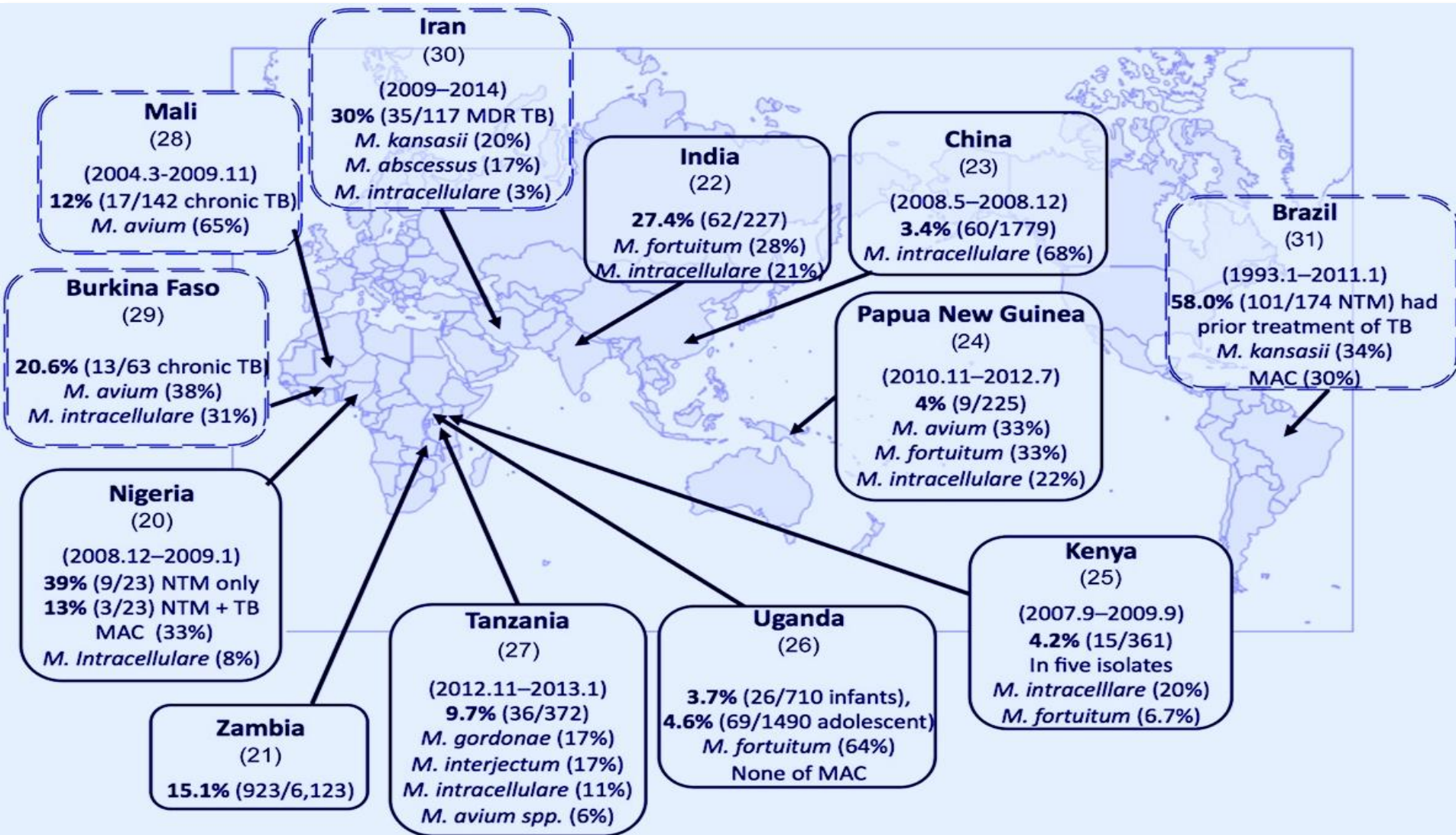
Mycobacterium avium-intracellulare infection (MAI) is an atypical mycobacterial infection, i.e. one with nontuberculous mycobacteria or NTM, caused by Mycobacterium avium complex ("MAC"), which is made of three mycobacteria species, *M. avium*, *M. intracellulare*, and *M. chimaera*.



Facts and Figures

- Countries in North America, Europe, and Australia, have suggested a continuing rise in the prevalence of pulmonary NTM isolates and NTM disease in these continents. Studies from some countries and geographical areas in Eastern Asia, such as Japan, South Korea, and Taiwan, have echoed this phenomenon.
- The increasing prevalence of pulmonary disease due to NTM is especially notable among the elderly, particularly in the context of aging populations in many countries. The gender predominance is frequently confounded by the differential prevalence of smoking-associated lung damage between men and women.
- NTM are ubiquitous organisms found in environmental sources that include drinking and natural water, as well as soil and dust. Human subjects can inhale or ingest NTM in water, aerosols, or dust. NTM are quite resistant to water disinfectants in common use, such as chlorine. This resistance likely contributes to reports describing frequent NTM detection in potable water within Australia and the USA. The ability of NTM to persist in urban water supplies may therefore be contributing to the increasing prevalence of pulmonary NTM disease in many countries.

Global Burden



About ARIKAYCE (1)

About the Insmed

Insmed, incorporated on March 21, 2013, is a biopharmaceutical company. The Company operates through development and commercialization of therapies for patients with rare diseases segment. The Company's lead product candidate is ARIKAYCE, or liposomal amikacin for inhalation (LAI). Its earlier-stage pipeline includes preclinical compounds that the Company is evaluating in multiple rare diseases of unmet medical need, including methicillin-resistant staph aureus (MRSA) and nontuberculous mycobacteria (NTM). Its earlier clinical-stage pipeline includes INS1007 and INS1009.

INS1007- INS1007 is an oral, reversible inhibitor of dipeptidyl peptidase 1 (DPP1), an enzyme responsible for activating neutrophil serine proteases, which are implicated in the pathology of chronic inflammatory lung diseases, such as non-cystic fibrosis (non-CF) bronchiectasis. In a Phase I study of healthy volunteers, INS1007 was well tolerated

INS1009- INS1009 is an inhaled nanoparticle formulation of a treprostinil prodrug that may offer a differentiated product profile for rare pulmonary disorders, including pulmonary arterial hypertension (PAH). It has completed a Phase I study of INS1009



Brief on Arikayce (Liposomal Inhalation Suspension)

- **INDICATIONS AND USAGE:** 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.
- **DOSAGE AND ADMINISTRATION:**
 - For oral inhalation use only.
 - Use ARIKAYCE vials only with the Lamira Nebulizer System.
 - The recommended dosage in adults is once daily oral inhalation of the contents of one 590 mg/8.4 mL ARIKAYCE vial.

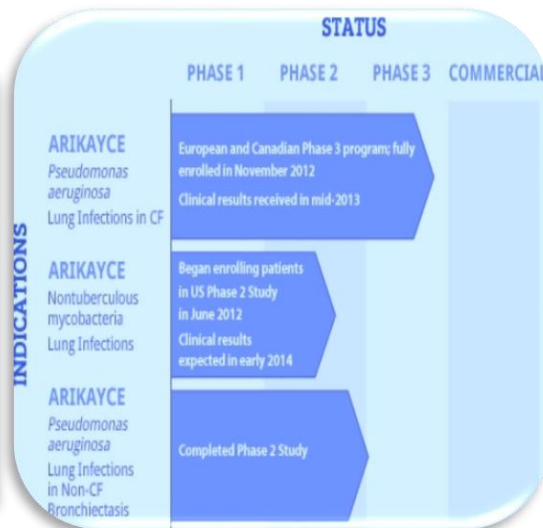


Market Overview - Lung Infection

Current Treatment options

The most common NTM lung infection, treated with a combination of three antibiotics:

1. Either azithromycin and clarithromycin.
2. Ethambutol.
3. Rifampin



Important information

- The LAMIRA device is specifically designed to be used with ARIKAYCE to treat MAC lung disease in patients that didn't respond to conventional antibiotic therapy. Inside the LAMIRA is a vibrating membrane nebulizer that aerosolizes the liposomal suspension of ARIKAYCE, a technology PARI calls "eFlow". It allows designers of nebulizers to optimize them for specific drugs, as to achieve the best penetration and clinical efficacy.
- The approval of the drug is the first under the FDA's new Limited Population Pathway for Antibacterial and Antifungal Drugs, which helps to speed up approvals for certain medications.
- **PARI Pharma**, a German manufacturer of aerosol delivery products, won FDA approval of its LAMIRA nebulizer along with **Insmed's** ARIKAYCE





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