

SEPTEMBER 28, 2018

# ARIKAYCE U.S. FDA Approval

 insmed<sup>®</sup>



# Forward Looking Statements

This presentation contains forward-looking statements. "Forward-looking statements," as that term is defined in the Private Securities Litigation Reform Act of 1995, are statements that are not historical facts and involve a number of risks and uncertainties. Words herein such as "may," "will," "should," "could," "would," "expects," "plans," "anticipates," "believes," "estimates," "projects," "predicts," "intends," "potential," "continues," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) may identify forward-looking statements. The forward-looking statements in this presentation are based upon our current expectations and beliefs, and involve known and unknown risks, uncertainties and other factors, which may cause our actual results, performance and achievements and the timing of certain events to differ materially from the results, performance, achievements or timing discussed, projected, anticipated or indicated in any forward-looking statements. Such factors include, among others: risks that the remainder of the data from the treatment and off-treatment phases of INS-212 will not be consistent with the six-month results of the study; uncertainties in the research and development of our existing product candidates, including due to delays in data readouts, such as the full data from the INS-212 study, patient enrollment and retention or failure of our preclinical studies or clinical trials to satisfy pre-established endpoints, including secondary endpoints in the INS-212 study and endpoints in the INS-212 extension study (the INS-312 study); risks that subsequent data from the INS-312 study will not be consistent with the interim results; imposition of significant post-approval regulatory requirements on our product candidates, including a requirement for a post-approval confirmatory clinical study, or failure to maintain or obtain full regulatory approval for our product candidates, if received, due to a failure to satisfy post-approval regulatory requirements, such as the submission of sufficient data from a confirmatory clinical study; safety and efficacy concerns related to our product candidates; uncertainties in the rate and degree of market acceptance of product candidates, if approved; inability to create an effective direct sales and marketing infrastructure or to partner with third parties that offer such an infrastructure for distribution of our product candidates, if approved; failure to obtain, or delays in obtaining, regulatory approval from the U.S. Food and Drug Administration, Japan's Ministry of Health, Labour and Welfare, Japan's Pharmaceuticals and Medical Devices Agency, the European Medicines Agency, and other regulatory authorities for our product candidates or their delivery devices, including due to insufficient clinical data, selection of endpoints that are not satisfactory to regulators or complexity in the review process for combination products; lack of experience in conducting and managing preclinical development activities and clinical trials necessary for regulatory approval, including the regulatory filing and review process; inaccuracies in our estimates of the size of the potential markets for our product candidates or limitations by regulators on the proposed treatment population for our product candidates; failure of third parties on which we are dependent to conduct our clinical trials, to manufacture sufficient quantities of our product candidates for clinical or commercial needs, including our raw materials suppliers, or to comply with our agreements or laws and regulations that impact our business; inaccurate estimates regarding our future capital requirements, including those necessary to fund our ongoing clinical development, regulatory and commercialization efforts as well as milestone payments or royalties owed to third parties; failure to develop, or to license for development, additional product candidates, including a failure to attract experienced third-party collaborators; uncertainties in the timing, scope and rate of reimbursement for our product candidates; changes in laws and regulations applicable to our business and failure to comply with such laws and regulations; inability to repay our existing indebtedness or to obtain additional capital when needed on desirable terms or at all; failure to obtain, protect and enforce our patents and other intellectual property and costs associated with litigation or other proceedings related to such matters; restrictions imposed on us by license agreements that are critical for our product development, including our license agreements with PARI Pharma GmbH and AstraZeneca AB, and failure to comply with our obligations under such agreements; competitive developments affecting our product candidates and potential exclusivity related thereto; the cost and potential reputational damage resulting from litigation to which we are or may become a party; loss of key personnel; and lack of experience operating internationally. For additional information about the risks and uncertainties that may affect our business, please see the factors discussed in Item 1A, "Risk Factors," in our Annual Report on Form 10-K for the year ended December 31, 2017 and any of our subsequent filings with the Securities and Exchange Commission. The Company cautions readers not to place undue reliance on any such forward-looking statements, which speak only as of the date of this presentation. The Company disclaims any obligation, except as specifically required by law and the rules of the Securities and Exchange Commission, to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. This presentation includes information related to market opportunity as well as cost and other estimates obtained from various internal analyses and calculations and external sources, including publicly available information, market research and data from a variety of claims databases. The externally sourced information has been obtained from sources we believe to be reliable, but we cannot assure the accuracy and completeness of such information. Similarly, our internal analyses and calculations are based upon management's understanding of market and industry conditions and has not been verified by independent sources. Forward-looking information presented from each of these sources is subject to the same qualifications and uncertainties as the other forward-looking statements in this presentation.

# Agenda

1 Introduction

2 ARIKAYCE Overview and Label

3 Commercial Launch

4 Next Steps

5 Q&A



# Our Mission

To transform the lives of people with serious and rare diseases



# Now Approved\*



# ARIKAYCE<sup>®</sup>

(amikacin liposome  
inhalation suspension)

# What is MAC Lung Disease?



*Mycobacterium avium* complex (MAC) lung disease is a rare, progressive, and chronic condition that can cause severe, permanent damage to the lungs.

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The disease is caused by bacteria in the environment and is more likely to affect those with a history of lung conditions, like bronchiectasis, chronic obstructive pulmonary disease (COPD), or asthma.

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Prior to the approval of ARIKAYCE, there were no inhaled therapies approved specifically for the treatment of patients with MAC lung disease.

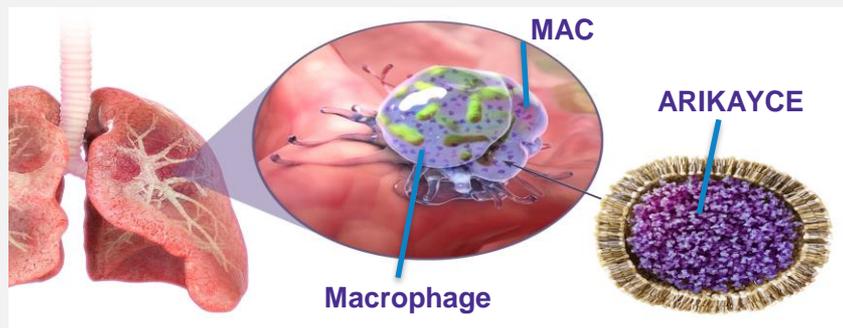
# About ARIKAYCE



# ARIKAYCE Developed to Address Significant Unmet Need in MAC Lung Disease

An inhaled, innovative, once-daily formulation of liposomal amikacin

## Uptake in the Lung Macrophage

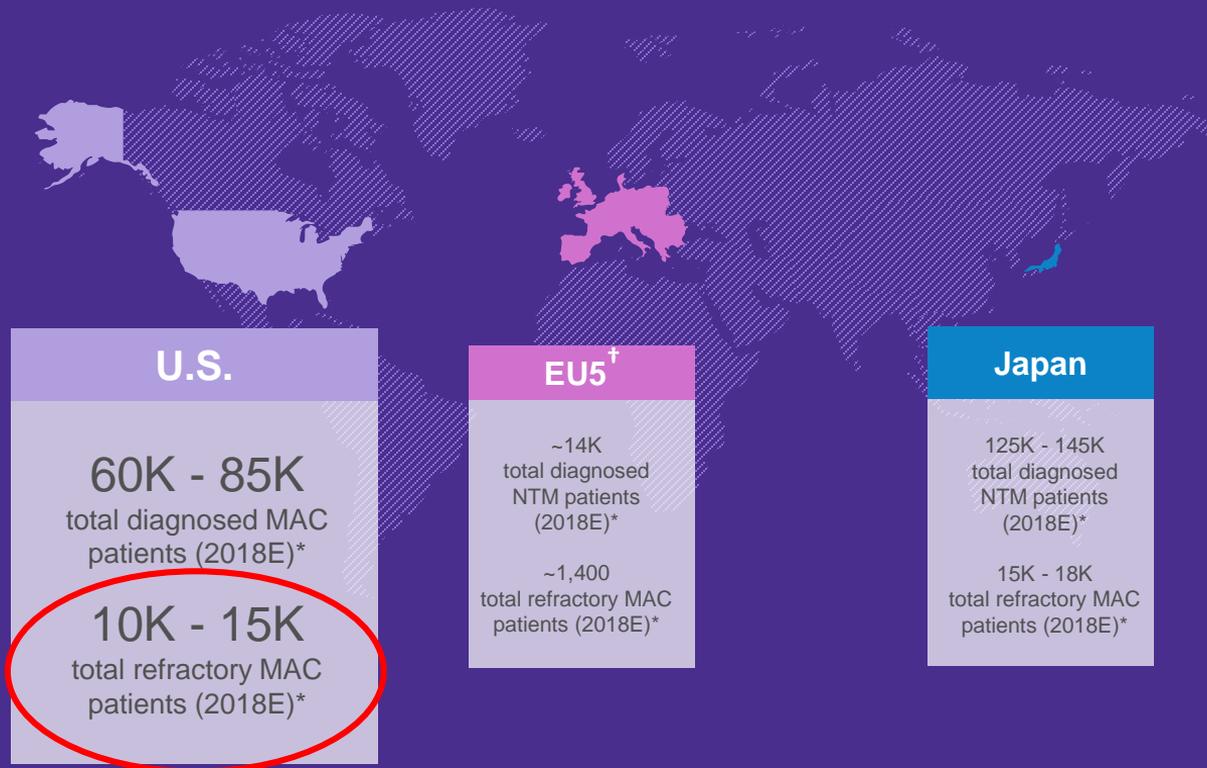


Advanced liposomal technology delivers drug **directly to site of infection**; prolongs release of amikacin in the lungs while **limiting systemic exposure**

# ARIKAYCE Indication and Use

- LIMITED POPULATION: 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. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.
- **Limitation 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.

# Commercial Approach: Near-Term Focus on U.S. Launch; Expansion to EU and Japan



\*Source: Internal analysis of published NTM epidemiology, primary market research with treating HCPs, and anonymized patient level claims data in US

<sup>†</sup> EU5 comprised of France, Germany, Italy, Spain and the United Kingdom

# Laying the Groundwork for a Successful U.S. Launch



## Sales Force Deployment

72 therapeutic specialists fully trained and will now be deployed in U.S. market

Transition from disease awareness efforts to product education immediately



## Awareness & Education

Pre-launch disease awareness-building, HCP engagement, and advocacy group support



## Patient Identification

Ongoing, innovative efforts under way to identify appropriate patients and enhance disease diagnosis



## Access

Payer engagement to enable efficient patient access



## Support

Comprehensive patient support program intended to help with treatment introduction, compliance, and navigating the reimbursement landscape

Program includes 35 Arikares Trainers and 10 Arikares Coordinators

# Go-Forward Strategic Priorities

1

Launch ARIKAYCE in U.S. market for appropriate patients

2

Establish patient access and provide appropriate support services for patients being prescribed ARIKAYCE

3

Maintain commitment to MAC lung disease patients and physicians through additional investment in post marketing studies and life cycle management programs

4

Continue expansion efforts in EU and Japan to support regulatory filings for ARIKAYCE

5

Maintain strong financial position



## Q&A