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# Insmmed Provides Update on ARIKAYCE® (amikacin liposome inhalation suspension) U.S. Launch and Outlines Strategic Priorities for 2019

*-ARIKAYCE Preliminary Unaudited U.S. Net Product Sales Approximately \$9.2 Million for the Fourth Quarter of 2018-*

*-More Than 500 Patients Have Initiated Treatment and Approximately 600 Physicians Have Prescribed ARIKAYCE-*

*-Company to Present at 37th Annual J.P. Morgan Healthcare Conference in San Francisco-*

BRIDGEWATER, N.J., Jan. 4, 2019 /PRNewswire/ -- Insmmed Incorporated (Nasdaq: INSM), a global biopharmaceutical company on a mission to transform the lives of patients with serious and rare diseases, today provided an update on the U.S. launch of ARIKAYCE® (amikacin liposome inhalation suspension), including preliminary unaudited net product sales for the fourth quarter of 2018, and shared the Company's strategic priorities for 2019. ARIKAYCE was granted accelerated approval by the U.S. Food and Drug Administration (FDA) on September 28, 2018, for the treatment of *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen for adult patients who have limited or no alternative treatment options.

These updates will be discussed as part of the Company's presentation at the 37th Annual J.P. Morgan Healthcare Conference in San Francisco on Wednesday, January 9, at 4:00 p.m. PT (7:00 p.m. ET).

"We are very pleased with the U.S. launch progress of ARIKAYCE and the strides we have made in a short period of time to reach patients with refractory MAC lung disease," said Will Lewis, President, Chief Executive Officer, and Chairman of the Board of Insmmed. "Importantly, we've seen steady increases in patients initiating therapy during the first three months of launch and have been pleased with the rates of reimbursement to date from payers. The strong prescription uptake we've seen thus far is complemented by positive anecdotal feedback from treating physicians. As we begin the new year, we are excited to continue this momentum and will provide a further update on the U.S. launch during our fourth quarter earnings call. We also look forward to pursuing a number of corporate and clinical milestones throughout 2019 that we believe will deliver value both to shareholders and to the rare disease community."

### **Preliminary Q4 2018 Net Product Sales (Unaudited) and Additional Launch Metrics**

Based on preliminary unaudited financial information, the Company expects total net product sales of ARIKAYCE to be approximately \$9.8 million for the quarter ended December 31, 2018, comprising U.S. net sales of \$9.2 million and ex-U.S. net sales of \$0.6 million. The ex-U.S. net sales reflect utilization from the Temporary Authorisation for Use (ATU) program in France. As of December 31, 2018, more than 500 patients in the U.S. have initiated treatment with ARIKAYCE and approximately 600 physicians in the U.S. have written at least one prescription for the therapy. The Company commenced commercialization efforts promptly following FDA approval.

Insmmed expects to release final results for the fourth quarter and audited results for the full year of 2018 on Friday, February 22, 2019.

### **Strategic Priorities**

Insmmed has established the following strategic priorities for 2019:

- Continue our efforts to ensure a successful U.S. launch of ARIKAYCE.
- Complete the design and protocol of the confirmatory clinical study during the first half of 2019 required for the full approval of ARIKAYCE by the FDA in the U.S. in a front-line setting of patients with MAC lung disease.
- Accelerate our global expansion to support potential regulatory filings for ARIKAYCE in Europe in mid-2019 and in Japan in the first half of 2020.
- Advance our pipeline, which is intended to bring additional therapies to market for patients with serious

and rare diseases, including completing enrollment in the WILLOW study, our six-month Phase 2 trial of INS1007 in patients with non-cystic fibrosis bronchiectasis, in mid-2019.

### **Presentation at 37th Annual J.P. Morgan Healthcare Conference**

Will Lewis, President, Chief Executive Officer, and Chairman of the Board of Insmmed, will present at the 37th annual J.P. Morgan Healthcare Conference on Wednesday, January 9, 2019, at 4:00 p.m. PT (7:00 p.m. ET). A live audio webcast of the presentation will be available on the Investor Relations section of the Company's website at [www.insmed.com](http://www.insmed.com). A replay will also be archived for 90 days on the Investor Relations section of the site.

### **About MAC Lung Disease**

*Mycobacterium avium* complex (MAC) lung disease is a rare and serious disorder that can significantly increase morbidity and mortality. Patients with MAC lung disease can experience a range of symptoms that often worsen over time, including chronic cough, dyspnea, fatigue, fever, weight loss, and chest pain. In some cases, MAC lung disease can cause severe, even permanent damage to the lungs, and can be fatal.

MAC lung disease is an emerging public health concern worldwide with significant unmet needs. Current guideline-based treatment involves the use of multi-drug regimens that are not specifically approved for MAC lung disease. The course of treatment is often two years or more and is inadequate in treating the disease in many patients.

### **About ARIKAYCE® (amikacin liposome inhalation suspension)**

ARIKAYCE is the first and only FDA-approved therapy indicated for the treatment of *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen for adult patients with limited or no alternative treatment options. ARIKAYCE is a novel, inhaled, once-daily formulation of amikacin, an established antibiotic that was historically administered intravenously and associated with severe toxicity to hearing, balance, and kidney function. Insmmed's proprietary PULMOVANCE™ liposomal technology enables the delivery of amikacin directly to the lungs, where it is taken up by lung macrophages where the infection resides. This approach prolongs the release of amikacin in the lungs while limiting systemic exposure. ARIKAYCE is administered once daily using the Lamira™ Nebulizer System manufactured by PARI Pharma GmbH (PARI).

### **About PARI Pharma and the Lamira™ Nebulizer System**

ARIKAYCE® (amikacin liposome inhalation suspension) is delivered by a novel inhalation device, the Lamira™ Nebulizer System, developed by PARI. Lamira™ is a quiet, portable nebulizer that enables efficient aerosolization of liquid medications, including liposomal formulations such as ARIKAYCE, via a vibrating, perforated membrane. Based on PARI's 100-year history working with aerosols, PARI is dedicated to advancing inhalation therapies by developing innovative delivery platforms and new pharmaceutical formulations that work together to improve patient care.

### **About Insmmed**

Insmmed Incorporated is a global biopharmaceutical company on a mission to transform the lives of patients with serious and rare diseases. Insmmed's first commercial product is ARIKAYCE® (amikacin liposome inhalation suspension), which is approved in the United States for the treatment of *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen for adult patients with limited or no alternative treatment options. MAC lung disease is a rare and often chronic infection that can cause irreversible lung damage and can be fatal. Insmmed's earlier-stage clinical pipeline includes INS1007, a novel oral reversible inhibitor of dipeptidyl peptidase 1 with therapeutic potential in non-cystic fibrosis bronchiectasis and other inflammatory diseases, and INS1009, an inhaled formulation of a treprostinil prodrug that may offer a differentiated product profile for rare pulmonary disorders, including pulmonary arterial hypertension. For more information, visit [www.insmed.com](http://www.insmed.com).

### **IMPORTANT SAFETY INFORMATION**

#### **WARNING: RISK OF INCREASED RESPIRATORY ADVERSE REACTIONS**

**ARIKAYCE has been associated with an increased risk of respiratory adverse reactions, including hypersensitivity pneumonitis, hemoptysis, bronchospasm, and exacerbation of underlying pulmonary disease that have led to hospitalizations in some cases.**

**Hypersensitivity Pneumonitis** has been reported with the use of ARIKAYCE in the clinical trials. Hypersensitivity pneumonitis (reported as allergic alveolitis, pneumonitis, interstitial lung disease, allergic reaction to ARIKAYCE) was reported at a higher frequency in patients treated with ARIKAYCE plus background regimen (3.1%) compared to patients treated with a background

regimen alone (0%). Most patients with hypersensitivity pneumonitis discontinued treatment with ARIKAYCE and received treatment with corticosteroids. If hypersensitivity pneumonitis occurs, discontinue ARIKAYCE and manage patients as medically appropriate.

**Hemoptysis** has been reported with the use of ARIKAYCE in the clinical trials. Hemoptysis was reported at a higher frequency in patients treated with ARIKAYCE plus background regimen (17.9%) compared to patients treated with a background regimen alone (12.5%). If hemoptysis occurs, manage patients as medically appropriate.

**Bronchospasm** has been reported with the use of ARIKAYCE in the clinical trials. Bronchospasm (reported as asthma, bronchial hyperreactivity, bronchospasm, dyspnea, dyspnea exertional, prolonged expiration, throat tightness, wheezing) was reported at a higher frequency in patients treated with ARIKAYCE plus background regimen (28.7%) compared to patients treated with a background regimen alone (10.7%). If bronchospasm occurs during the use of ARIKAYCE, treat patients as medically appropriate.

**Exacerbations of underlying pulmonary disease** has been reported with the use of ARIKAYCE in the clinical trials. Exacerbations of underlying pulmonary disease (reported as chronic obstructive pulmonary disease (COPD), infective exacerbation of COPD, infective exacerbation of bronchiectasis) have been reported at a higher frequency in patients treated with ARIKAYCE plus background regimen (14.8%) compared to patients treated with background regimen alone (9.8%). If exacerbations of underlying pulmonary disease occur during the use of ARIKAYCE, treat patients as medically appropriate.

**Ototoxicity** has been reported with the use of ARIKAYCE in the clinical trials. Ototoxicity (including deafness, dizziness, presyncope, tinnitus, and vertigo) were reported with a higher frequency in patients treated with ARIKAYCE plus background regimen (17%) compared to patients treated with background regimen alone (9.8%). This was primarily driven by tinnitus (7.6% in ARIKAYCE plus background regimen vs 0.9% in the background regimen alone arm) and dizziness (6.3% in ARIKAYCE plus background regimen vs 2.7% in the background regimen alone arm). Closely monitor patients with known or suspected auditory or vestibular dysfunction during treatment with ARIKAYCE. If ototoxicity occurs, manage patients as medically appropriate, including potentially discontinuing ARIKAYCE.

**Nephrotoxicity** was observed during the clinical trials of ARIKAYCE in patients with MAC lung disease but not at a higher frequency than background regimen alone. Nephrotoxicity has been associated with the aminoglycosides. Close monitoring of patients with known or suspected renal dysfunction may be needed when prescribing ARIKAYCE.

**Neuromuscular Blockade:** Patients with neuromuscular disorders were not enrolled in ARIKAYCE clinical trials. Patients with known or suspected neuromuscular disorders, such as myasthenia gravis, should be closely monitored since aminoglycosides may aggravate muscle weakness by blocking the release of acetylcholine at neuromuscular junctions.

**Embryo-Fetal Toxicity:** Aminoglycosides can cause fetal harm when administered to a pregnant woman. Aminoglycosides, including ARIKAYCE, may be associated with total, irreversible, bilateral congenital deafness in pediatric patients exposed *in utero*. Patients who use ARIKAYCE during pregnancy, or become pregnant while taking ARIKAYCE should be apprised of the potential hazard to the fetus.

**Contraindications:** ARIKAYCE is contraindicated in patients with known hypersensitivity to any aminoglycoside.

**Most Common Adverse Reactions:** The most common adverse reactions in Trial 1 at an incidence  $\geq 5\%$  for patients using ARIKAYCE plus background regimen compared to patients treated with background regimen alone were dysphonia (47% vs 1%), cough (39% vs 17%), bronchospasm (29% vs 11%), hemoptysis (18% vs 13%), ototoxicity (17% vs 10%), upper airway irritation (17% vs 2%), musculoskeletal pain (17% vs 8%), fatigue and asthenia (16% vs 10%), exacerbation of underlying pulmonary disease (15% vs 10%), diarrhea (13% vs 5%), nausea (12% vs 4%), pneumonia (10% vs 8%), headache (10% vs 5%), pyrexia (7% vs 5%), vomiting (7% vs 4%), rash (6% vs 2%), decreased weight (6% vs 1%), change in sputum (5% vs 1%), and chest discomfort (5% vs 3%).

**Drug Interactions:** Avoid concomitant use of ARIKAYCE with medications associated with neurotoxicity, nephrotoxicity, and ototoxicity. Some diuretics can enhance aminoglycoside toxicity by altering aminoglycoside concentrations in serum and tissue. Avoid concomitant use of ARIKAYCE with ethacrynic acid, furosemide, urea, or intravenous mannitol.

**Overdosage:** Adverse reactions specifically associated with overdose of ARIKAYCE have not been identified. Acute toxicity should be treated with immediate withdrawal of ARIKAYCE, and baseline tests of renal function should be undertaken. Hemodialysis may be helpful in removing amikacin from the body. In all cases of suspected overdosage, physicians should contact the Regional Poison Control Center for information about effective treatment.

## INDICATION

LIMITED POPULATION: ARIKAYCE<sup>®</sup> 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.

Patients are encouraged report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch), or call 1-800-FDA-1088. You can also call the Company at 1-844-4-INSMED.

Please see [Full Prescribing Information](#).

### **Preliminary Financial Information**

The fourth quarter 2018 net product sales information and additional launch metrics presented above are preliminary and subject to revision. The preliminary net product sales information may be revised based on the completion of the Company's fourth quarter financial close and reporting process and the year-end audit to be performed by Ernst & Young LLP, the Company's independent registered public accounting firm. During the course of this process, the Company may identify items that would require adjustments to this information. These estimates thus constitute forward-looking statements, and the Company cautions you that they are subject to risks and uncertainties. Ernst & Young LLP has not audited, reviewed, compiled or performed any procedures with respect to these preliminary estimates. Accordingly, Ernst & Young LLP does not express an opinion or any other form of assurance with respect to these preliminary estimates.

### **Forward-looking Statements**

This press release contains forward-looking statements that involve substantial risks and uncertainties. "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 press release are based upon the Company's current expectations and beliefs, and involve known and unknown risks, uncertainties and other factors, which may cause the Company's 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 risks, uncertainties and other factors include, among others, the following: failure to successfully commercialize or maintain US approval for ARIKAYCE, the Company's only approved product; uncertainties in the degree of market acceptance of ARIKAYCE by physicians, patients, third-party payers and others in the healthcare community; the Company's inability to obtain full approval of ARIKAYCE from the FDA, including the risk that the Company will not successfully complete the confirmatory post-marketing study required for full approval; inability of the Company, PARI or the Company's third party manufacturers to comply with regulatory requirements related to ARIKAYCE or the Lamira™ Nebulizer System; the Company's inability to obtain adequate reimbursement from government or third-party payers for ARIKAYCE or acceptable prices for ARIKAYCE; development of unexpected safety or efficacy concerns related to ARIKAYCE; inaccuracies in the Company's estimates of the size of the potential markets for ARIKAYCE; the Company's inability to create an effective direct sales and marketing infrastructure or to partner with third parties that offer such an infrastructure for distribution of ARIKAYCE; failure to obtain regulatory approval to expand ARIKAYCE's indication to a broader patient population; failure to successfully conduct future clinical trials for ARIKAYCE and the Company's product candidates, including due to the Company's limited experience in conducting preclinical development activities and clinical trials necessary for regulatory approval and the Company's inability to enroll or retain sufficient patients to complete the trials or generate data necessary for regulatory approval; risks that the Company's clinical studies will be delayed or that serious side effects will be identified during drug development; failure to obtain regulatory approvals for ARIKAYCE outside the US or for the Company's product candidates in the US, Europe, Japan or other markets; failure of third parties on which the Company is dependent to manufacture sufficient quantities of ARIKAYCE or the Company's product candidates for commercial or clinical needs, to conduct the Company's clinical trials, or to comply with laws and regulations that impact the Company's

business or agreements with the Company; the Company's inability to attract and retain key personnel or to effectively manage the Company's growth; the Company's inability to adapt to its highly competitive and changing environment; the Company's inability to adequately protect its intellectual property rights or prevent disclosure of its trade secrets and other proprietary information and costs associated with litigation or other proceedings related to such matters; restrictions imposed on the Company by its material license agreements, including its license agreements with PARI and AstraZeneca AB, and failure of the Company to comply with its obligations under such agreements; the cost and potential reputational damage resulting from litigation to which the Company is or may become a party, including product liability claims; limited experience operating internationally; changes in laws and regulations applicable to the Company's business and failure to comply with such laws and regulations; and inability to repay the Company's existing indebtedness and uncertainties with respect to the Company's ability to access future capital.

The Company may not actually achieve the results, plans, intentions or expectations indicated by the Company's forward-looking statements because, by their nature, forward-looking statements involve risks and uncertainties because they relate to events and depend on circumstances that may or may not occur in the future. For additional information about the risks and uncertainties that may affect the Company's business, please see the factors discussed in Item 1A, "Risk Factors," in the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2018 and any subsequent Company 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 press release. 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.

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