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# Insmmed Announces FDA Acceptance for Filing of New Drug Application for ALIS in NTM Lung Disease Caused by MAC

## PDUFA Action Date Set for September 28, 2018

BRIDGEWATER, N.J., May 16, 2018 (GLOBE NEWSWIRE) -- Insmmed Incorporated (Nasdaq:INSM), a global biopharmaceutical company focused on the unmet needs of patients with rare diseases, announced today that the U.S. Food and Drug Administration (FDA) has notified the company that the New Drug Application (NDA) for ALIS (Amikacin Liposome Inhalation Suspension) for adult patients with nontuberculous mycobacterial (NTM) lung disease caused by *Mycobacterium avium* complex (MAC) has been accepted and will be considered filed on May 27, 2018. The FDA has completed the filing review and determined that the application is sufficiently complete to permit a substantive review. The FDA granted Insmmed's request for Priority Review and has set an action date of September 28, 2018 under the Prescription Drug User Fee Act (PDUFA).

"We are pleased that the FDA has accepted our NDA with Priority Review, which underscores the urgent need for therapies which address NTM lung disease caused by MAC," said Will Lewis, President and Chief Executive Officer of Insmmed. "ALIS has the potential to be the first approved inhaled therapy for the treatment of this serious and debilitating lung infection in the United States, and we look forward to working with the Agency through the review process."

Insmmed continues to expect that the FDA will convene an advisory committee meeting to discuss this application.

Priority Review applies to an application for a drug that treats a serious condition and that, if approved, would provide a significant improvement in safety or effectiveness. FDA has previously designated ALIS as an orphan drug, a breakthrough therapy and a Qualified Infectious Disease Product (QIDP) under the Generating Antibiotic Incentives Now (GAIN) Act.

## About NTM Lung Disease

NTM lung disease is a rare and serious disorder associated with increased rates of morbidity and mortality. There is an increasing prevalence of lung disease caused by NTM, and Insmmed believes it is an emerging public health concern worldwide. Patients with NTM lung disease may experience a multitude of symptoms such as fever, weight loss, cough, lack of appetite, night sweats, blood in the sputum, and fatigue. Patients with NTM lung disease frequently require lengthy hospital stays to manage their condition. Insmmed is not aware of any approved inhaled therapies specifically indicated for refractory NTM lung disease caused by MAC in North America, Japan or Europe. Current guideline-based approaches involve use of multi-drug regimens not approved for the treatment of NTM lung disease, and treatment can be as long as two years or more.

The prevalence of human disease attributable to NTM has increased over the past two decades. In a decade long study (1997 to 2007), researchers found that the prevalence of NTM lung disease in the U.S. was increasing at approximately 8% per year and that NTM patients on Medicare over the age of 65 were 40% more likely to die over the period of the study than those who did not have the disease. In the U.S., Insmmed estimates there will be between 75,000 and 105,000 patients with diagnosed NTM lung disease in 2018, of which the Company expects 40,000 to 50,000 will be treated for NTM lung disease caused by MAC. Insmmed expects that between 10,000 and 15,000 of these patients will be refractory to treatment.

In Japan, Insmmed estimates there will be between 125,000 and 145,000 patients with diagnosed NTM lung disease in 2018, with approximately 60,000 to 70,000 of those patients being treated for NTM lung disease caused by MAC and 15,000 to 18,000 of these treated patients being refractory to treatment. Insmmed also estimates there will be approximately 14,000 patients with diagnosed NTM lung disease in the EU5 (comprised of France, Germany, Italy, Spain and the United Kingdom) in 2018, of which the Company estimates approximately 4,400 will be treated for NTM lung disease caused by MAC and approximately 1,400 of these treated patients will be refractory to treatment.

## About ALIS

ALIS is a novel, inhaled, once-daily formulation of amikacin that is in late-stage clinical development for adult patients with treatment-refractory NTM lung disease caused by MAC. Amikacin solution for parenteral administration is an established drug that has activity against a variety of NTM; however, its use is limited by the need to administer it intravenously and by toxicity to hearing, balance, and kidney function. Insmmed's advanced pulmonary liposome technology uses charge neutral liposomes to deliver amikacin directly to the lung where it is taken up by the lung macrophages where the NTM infection resides. This prolongs the release of amikacin in the lungs while minimizing systemic exposure thereby offering the potential for decreased

systemic toxicities. ALIS's ability to deliver high levels of amikacin directly to the lung distinguishes it from intravenous amikacin. ALIS is administered once daily using an optimized, investigational eFlow® Nebulizer System manufactured by PARI Pharma GmbH (PARI), a portable aerosol delivery system.

## **About Insmed**

Insmed Incorporated is a global biopharmaceutical company focused on the unmet needs of patients with rare diseases. The Company's lead product candidate is ALIS, which is in late-stage development for adult patients with treatment refractory NTM lung disease caused by MAC, which is a rare and often chronic infection that is capable of causing irreversible lung damage and can be fatal. Insmed'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 nanoparticle 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).

## **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: risks that the data from the remainder of the treatment and off-treatment phases of INS-212 will not be consistent with the top-line six-month results of the study; uncertainties in the research and development of the Company's 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 the Company's 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; 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 the Company's product candidates or their delivery devices, such as the eFlow Nebulizer System, including due to insufficient clinical data, selection of endpoints that are not satisfactory to regulators, complexity in the review process for combination products or inadequate or delayed data from a human factors study required for U.S. regulatory approval; failure to maintain regulatory approval for the Company's product candidates, if received, due to a failure to satisfy post-approval regulatory requirements, such as the submission of sufficient data from confirmatory clinical studies; safety and efficacy concerns related to the Company's product candidates; lack of experience in conducting and managing preclinical development activities and clinical trials necessary for regulatory approval, including the regulatory filing and review process; failure to comply with extensive post-approval regulatory requirements or imposition of significant post-approval restrictions on the Company's product candidates by regulators; 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 the Company's product candidates, if approved; inaccuracies in the Company's estimates of the size of the potential markets for the Company's product candidates or limitations by regulators on the proposed treatment population for the Company's product candidates; failure of third parties on which the Company is dependent to conduct the Company's clinical trials, to manufacture sufficient quantities of the Company's product candidates for clinical or commercial needs, including the Company's raw materials suppliers, or to comply with the Company's agreements or laws and regulations that impact the Company's business; inaccurate estimates regarding the Company's future capital requirements, including those necessary to fund the Company's 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 the Company's product candidates; changes in laws and regulations applicable to the Company's business and failure to comply with such laws and regulations; inability to repay the Company's existing indebtedness or to obtain additional capital when needed on desirable terms or at all; failure to obtain, protect and enforce the Company's patents and other intellectual property and costs associated with litigation or other proceedings related to such matters; restrictions imposed on the Company by license agreements that are critical for the Company's product development, including the Company's license agreements with PARI Pharma GmbH and AstraZeneca AB, and failure to comply with the Company's obligations under such agreements; competitive developments affecting the Company's product candidates and potential exclusivity related thereto; the cost and potential reputational damage resulting from litigation to which the Company is or may be a party, loss of key personnel; and lack of experience operating internationally.

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 Annual Report on Form 10-K for the year ended December 31, 2017 and any 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 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.

**Contact:**

Blaine Davis  
Insmmed Incorporated  
(908) 947-2841  
[blaine.davis@insmed.com](mailto:blaine.davis@insmed.com)

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