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# Insmmed Submits New Drug Application to FDA for ALIS in NTM Lung Disease Caused by MAC

**ALIS previously designated as an orphan drug, breakthrough therapy and Qualified Infectious Disease Product (QIDP)**

BRIDGEWATER, N.J., March 29, 2018 (GLOBE NEWSWIRE) -- Insmmed Incorporated (Nasdaq:INSM), a global biopharmaceutical company focused on the unmet needs of patients with rare diseases, today announced that it has submitted its New Drug Application (NDA) for ALIS (Amikacin Liposome Inhalation Suspension) to the U.S. Food and Drug Administration (FDA) for adult patients with Nontuberculous Mycobacterial (NTM) lung disease caused by Mycobacterium avium complex (MAC).

"We are very excited to announce the completion of this important milestone which begins the formal process of review by the FDA of our application for approval of ALIS to treat patients living with NTM caused by MAC. There are currently no approved therapies in the U.S. to treat this disease. Our submission is supported by our pivotal Phase 3 INS-212 study conducted in subjects with refractory NTM lung disease, the completed Phase 2 study TR02-112, as well as an expanded safety database," remarked Will Lewis, President and Chief Executive Officer of Insmmed. "We look forward to continuing our conversations with the FDA during this process."

The Company anticipates receiving a six-month Priority Review and that the NDA will be reviewed by the Division of Anti-Infective Products. The FDA will have 60 days to review the submission of the NDA to determine if it is complete and acceptable for filing. The 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 we believe 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. We are 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., we estimate there will be between 75,000 and 105,000 patients with diagnosed NTM lung disease in 2018, of which we expect 40,000 to 50,000 will be treated for NTM lung disease caused by MAC. We expect that between 10,000 and 15,000 of these patients will be refractory to treatment. In Japan, we estimate 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. We also estimate 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 we estimate 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 associated with this systemic exposure. Insmed'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 CONVERT (INS-212) and INS-312**

CONVERT is a randomized, open-label, global Phase 3 trial designed to confirm the culture conversion results seen in Insmed's Phase 2 clinical trial of ALIS in patients with refractory NTM lung disease caused by MAC. CONVERT is being conducted in 18 countries at more than 125 sites. The primary efficacy endpoint is the proportion of patients who achieve sputum culture conversion (defined as 3 consecutive negative monthly sputum cultures) by Month 6 in the ALIS plus GBT arm compared to the GBT-only arm. Patients who achieve culture conversion by Month 6 will continue in the CONVERT study for an additional 12 months of treatment following the first monthly negative sputum culture. Patients who do not culture convert have the option of enrolling in our INS-312 study. INS-312 is a single-arm, open-label extension study for patients who completed six months of treatment in the INS-212 study, but did not demonstrate culture conversion by Month 6. Under the study protocol, patients in the ALIS plus GBT arm of the INS-212 study will receive an additional 12 months of ALIS plus GBT. Patients who crossed over from the GBT-only arm of the INS-212 study will receive 12 months of treatment of ALIS + GBT.

## **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-state 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 full six-month data from the INS-212 study or subsequent data from the remainder of the study's treatment and off-treatment phases 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 312 study); risks that subsequent data from the 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, including, without limitation, the class action lawsuit against the Company that recently was dismissed without prejudice; 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.

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