January 3, 2018

Insmed Announces Additional Data from ALIS (Amikacin Liposome Inhalation Suspension) Phase 3 Clinical Program for Adult Patients with Treatment Refractory NTM Lung Disease Caused by MAC and Reports Progress with Commercial Preparations

- ***Interim results from INS-312 show that 28% of patients who previously received 6 months of guideline based therapy (GBT) alone achieved culture conversion following addition of ALIS to therapy, consistent with data from the INS-212 study***
- ***Continued treatment of culture positive patients with ALIS + GBT beyond 6 months results in additional patients achieving culture conversion***
- ***Long-term data from the INS-212 study show that durability of culture conversion is substantially higher among patients treated with ALIS + GBT***
- ***Company targeting submission of NDA before the end of March***
- ***Regulatory activities and pre-commercial expansion ongoing to support an anticipated US commercial launch of ALIS in late 2018 to be followed by a regulatory filing in Japan***
- ***Conference call today at 5:00 pm EST***

BRIDGEWATER, N.J., Jan. 03, 2018 (GLOBE NEWSWIRE) -- Insmed Incorporated (Nasdaq:INSM), a global biopharmaceutical company focused on the unmet needs of patients with rare diseases, today announced new data from its Phase 3 clinical program for adult patients with treatment refractory NTM lung disease. This program is made up of both the INS-212 and INS-312 studies, both of which are evaluating ALIS (Amikacin Liposome Inhalation Suspension) in adult patients with Nontuberculous Mycobacterial (NTM) lung disease caused by *Mycobacterium avium* Complex (MAC). The Company also provided an update on its preparations to support a potential commercial launch of ALIS in the United States in late 2018, followed by a potential regulatory filing in Japan.

"The interim results announced today from our ongoing INS-212 and INS-312 studies provide confirmation of the initial positive clinical data released in September 2017. These data validate the initial and continued treatment effect of ALIS in combination with GBT and also demonstrate the durability of this effect in this challenging patient population," remarked Dr. Paul Streck, Chief Medical Officer of Insmed. "Specifically, culture conversion rates across the two studies are consistent and show a benefit for patients who use ALIS in combination with GBT. With the addition of ALIS, patients who had continued on GBT alone are now converting in INS-312 at similar rates as patients adding ALIS to GBT in INS-212. Moreover, a number of patients who continued to use ALIS plus GBT beyond six months have now achieved culture conversion. We have also observed greater durability of response among converting patients who continued treatment with add-on ALIS, compared to the GBT alone."

The company previously announced that the global Phase 3 INS-212 study met its primary endpoint of culture conversion by Month 6 with statistical significance. Those results demonstrated that the addition of ALIS to GBT eliminated evidence of NTM lung disease caused by MAC in sputum by Month 6 in 29% of patients, compared to 9% of patients on GBT only ($p < 0.0001$). Serious treatment emergent adverse events (TEAEs) were similar between treatment arms.

Previously Disclosed INS-212 Topline Results from September 2017		
	Patients in INS-212 (n=336)	**Percent Achieving Sputum Culture Conversion by Month 6**
ALIS + GBT	224	29% (n=65/224)
GBT only	112	9% (n=10/112)

Patients that achieved culture conversion by Month 6 in either arm of INS-212 continue in the INS-212 study for an additional 12 months of treatment plus a follow-up period off all therapy to assess durability. Patients in either arm of the INS-212 study who did not achieve culture conversion by Month 6 had the option to enroll in INS-312 at month 8, an extension study that is evaluating treatment with ALIS plus GBT for 12 months.

Interim Data from INS-312 Extension Study for Non-Converters from INS-212 Study

INS-312 is a 12-month extension study for patients who completed six months of treatment in the INS-212 study, but did not demonstrate culture conversion by Month 6. The trial is designed to evaluate: 1) the impact of longer term treatment with ALIS plus GBT and 2) the impact of the addition of ALIS to background GBT therapy. Under the study protocol, patients in the ALIS plus GBT arm of the INS-212 study receive an additional 12 months of ALIS plus GBT. Patients who crossed over from the GBT only arm of the INS-212 study receive 12 months of treatment of ALIS plus GBT.

As of December 2017, of the 163 patients enrolled in INS-312, 124 patients were evaluable for sputum culture conversion. Interim culture conversion data as of December 2017 for these 124 patients is detailed below.

Impact of treating patients with ALIS + GBT		
	Number of Patients Completing Six Months of Treatment in INS-312 as of December 2017	**Percent Achieving Sputum Culture Conversion by Month 6 in INS-312**
Patients who received GBT only in INS-212 and crossed over to receiving six months of treatment with ALIS + GBT (n=90)	67	28.4% (19/67)
Patients who received ALIS + GBT in INS-212 and continued treatment in INS-312, for a combined total of 14 months of ALIS + GBT treatment in both studies (n=73)	57	12.3% (7/57)

In the INS-312 study, serious treatment emergent adverse events were similar to the previous findings announced in September of 2017. At this point in time, the dropout rate in INS-312 is 24% (n=39/163).

The Company plans to continue to monitor and evaluate patients throughout the duration of the INS-312 study.

Long-Term Durability Data from INS-212 Study

Insmed has also evaluated the durability of sputum culture conversion, as defined by patients that have completed treatment and continued in the study off all therapy for three months, the endpoint necessary to support full regulatory approval in the United States. As of December 2017, of the 75 patients achieving sputum culture conversion in INS-212, 53 of these patients were evaluable for durability of sputum culture conversion three months after the completion of treatment. Interim data for durability of sputum culture conversion as of December 2017 on these 53 patients are detailed below.

Durability of Culture Conversion 3 Months After all Treatment is Stopped		
	Evaluable Number of Patients as of December 2017 (At Least Three Months Post-Treatment)	**Percent with Durable Sputum Culture Conversion Three Months After Completion of Treatment**
Converters in the ALIS + GBT arm (n=65)	46	60.9% (28/46)
Converters in the GBT only arm (n=10)	7	0.0% (0/7)

At this point in time, the overall dropout rate in INS-212 is 18% (n=60/336).

The Company plans to continue to monitor and evaluate patients throughout the duration of the INS-212 study and expects to report additional data in late 2018 or early 2019 when the INS-312 study has completed its full 12 months and the data has been analyzed pursuant to its statistical analysis plan as agreed with FDA.

"The new data announced today amplify the results previously released from the CONVERT study demonstrating the potential of ALIS to produce sputum conversion in difficult-to-treat MAC lung disease patients which is the first and most important goal for the successful treatment of MAC lung disease. Further, these new data provide evidence of durable MAC eradication in these patients," stated David Griffith, M.D., Professor of Medicine, W.A and E.B. Moncrief Distinguished Professor at The University of Texas Health Sciences Center and Principal Investigator in the CONVERT study. "The sputum conversion data from the CONVERT studies are consistent and quite compelling, and represent an important advance in the treatment of MAC lung disease, a potentially devastating disease for which there are no approved therapies."

Regulatory and Commercial Progress

Insmed also provided an update on its regulatory efforts for ALIS and commercial readiness in its two initial target markets, the United States and Japan.

- The Company remains on track to file its New Drug Application (NDA) for accelerated approval of ALIS with the U.S. Food and Drug Administration (FDA) before the end of March.

- Insmed expects to receive a six-month priority review from the FDA.
- The Company is accelerating its production efforts, including the construction of a larger capacity manufacturing facility at a contract manufacturing organization as well as initiation of manufacturing of commercial product supply from existing facilities.
- The Company announced the hiring of John D. Soriano as Chief Compliance Officer. John most recently served as Senior Vice President, Chief Compliance Officer for Celgene.
- The Company is hiring the field teams that will help to support the potential U.S. product launch in late 2018, as well as senior leadership to direct and advance our Japanese opportunity.
- The Company opened a Japanese subsidiary, a requirement for regulatory approval in Japan, and plans to reconfirm with the Pharmaceuticals and Medical Device Agency (PMDA) the approval path for ALIS in Japan based on the clinical data. Insmed plans to file an application with the PMDA in late 2018 or early 2019.

"We continue to make solid progress in our regulatory and pre-commercial efforts focused on our first two target markets, the United States and Japan, which together represent the vast majority of known NTM patients worldwide," said Will Lewis, President and Chief Executive Officer of Insmed. "We are working diligently to submit a high-quality NDA that includes the data to date from CONVERT and, pending an approval, believe that we could be in a position to launch in the U.S. in late 2018. In Japan, we are building the initial infrastructure to support our regulatory and pre-commercial efforts in that important market. We look forward to providing updates on our progress over the course of 2018."

Conference Call

Insmed will host a conference call beginning today at 5:00 PM Eastern Time. Shareholders and other interested parties may participate in the conference call by dialing (844) 707-0669 (domestic) or (703) 639-1223 (international) and referencing conference ID number 6787825. The call will also be webcast live on the internet on the Company's website at www.insmed.com.

A replay of the conference call will be accessible approximately two hours after its completion through January 10, 2018 by dialing (855) 859-2056 (domestic) or (404) 537-3406 (international) and referencing conference ID number 6787825. A webcast of the call will also be archived for 90 days under the Investor Relations section of the Company's website at www.insmed.com.

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. 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, an important cell in this disease where the NTM can multiply. The liposome 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 culture conversion at 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 study where patients will receive ALIS plus GBT for 12 months.

About Insmed

Insmed Incorporated is a global biopharmaceutical company focused on the unmet needs of patients with rare diseases. Our lead product candidate is ALIS 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. 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. 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 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.

Forward-looking Statements

This press release 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 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: risks that the full six-month data from the CONVERT 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 our existing product candidates, including due to delays in data readouts, such as the full data from the CONVERT study, patient enrollment and retention or failure of our preclinical studies or clinical trials to satisfy pre-established endpoints, including secondary endpoints in the CONVERT study and endpoints in the CONVERT extension study (the 312 study); risks that subsequent data from the 312 study will not be consistent with the interim results of the study; failure to obtain, or delays in obtaining, regulatory approval from the FDA, Japan's Ministry of Health, Labour and Welfare, the European Medicines Agency, and other regulatory authorities for our 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 US regulatory approval; failure to maintain 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 confirmatory clinical studies; safety and efficacy concerns related to our 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 our 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 our product candidates, if approved; 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; 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 a party, including, without limitation, the class action lawsuit pending against us; 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 the Company's Report on Form 10-Q for the quarter ending September 30, 2017.

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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