Insmed Reports Third Quarter 2017 Financial Results and Provides Business Update

During the third quarter, the Company announced positive top-line results from its Phase 3 CONVERT Study of ALIS (amikacin liposome inhalation suspension) in patients with NTM lung disease caused by MAC, a rare, progressive, destructive lung infection
Study met primary endpoint of culture conversion ($p < 0.0001$)
Insmed plans to pursue accelerated approval and request priority review
Company enhanced cash position by $378 million through public offering of stock

BRIDGEWATER, N.J, Nov. 02, 2017 (GLOBE NEWSWIRE) -- Insmed Incorporated (Nasdaq:INSM), a global biopharmaceutical company focused on the unmet needs of patients with rare diseases, today reported financial results for the third quarter ended September 30, 2017 and provided a business update.

"The positive top-line results from CONVERT we announced during the third quarter represent a significant step forward in fulling our mission of transforming the lives of patients with rare diseases. Our top priority is to complete the NDA for a U.S. regulatory filing with the FDA, under subpart H," said Will Lewis, President and Chief Executive Officer of Insmed. "We are rapidly expanding our commercial team and enhancing our market access efforts for the U.S., and we've commenced our planning for expansion in Japan. We also remain committed to executing on life cycle management opportunities for ALIS and the advancement of our phase 2 study of INS1007 in non-cystic fibrosis (non-CF) bronchiectasis with patient screening underway. We also intend to transform our INS1009 inhaled treprostinil product candidate into an inhaled dry powder formulation which we believe will create a more compelling product profile. Following our successful public offering completed in the third quarter, we are in a solid financial position to fund these activities."

CONVERT Study

- CONVERT study met its primary endpoint of culture conversion by Month 6 with statistical and clinical significance.
- Study demonstrated that the addition of ALIS to guideline-based therapy (GBT) eliminated evidence of nontuberculous mycobacteria (NTM) lung disease caused by Mycobacterium avium complex (MAC) in sputum by Month 6 in 29% of patients, compared to 9% of patients on GBT alone ($p < 0.0001$).
- Insmed plans to pursue accelerated approval for ALIS under subpart H based on the data from the CONVERT study, which will be reviewed by the Division of Anti-Infective Products.
- FDA previously granted product Breakthrough Therapy designation and fast track status and designated ALIS as a qualified infectious disease product (QIDP) under the Generating Antibiotic Incentives Now (GAIN) Act.

Safety and Tolerability

- Serious treatment emergent adverse events were similar between treatment arms.
- Overall dropout rate was 16.1%, with an 8.9% dropout rate in the GBT arm and a 19.6% rate in the ALIS plus GBT arm.

Third Quarter Financial Results

For the third quarter of 2017, Insmed reported a net loss of $45.2 million, or $0.69 per share, compared with a net loss of $37.8 million, or $0.61 per share, for the third quarter of 2016.

Research and development expenses were $26.7 million for the third quarter of 2017, compared with $23.4 million for the third quarter of 2016. The increase was primarily due to higher expenses related to INS1007 as compared to the prior year period.

General and administrative expenses for the third quarter of 2017 were $17.4 million, compared with $13.7 million for the third quarter of 2016. The increase was primarily due to higher expenses related to our pre-commercial planning activities for ALIS and higher compensation and related expenses due to an increase in headcount, as compared to the prior year period.

Balance Sheet and Other Financial Highlights

As of September 30, 2017, Insmed had cash and cash equivalents of approximately $431 million. The cash position reflects net proceeds of $378 million received from the public offering of Insmed common stock completed on September 11, 2017. The Company's operating expenses for the third quarter of 2017 were approximately $44 million, and its cash-based operating expenses for the third quarter of 2017 were approximately $39 million. Insmed ended the third quarter of 2017 with approximately $55 million in debt.

Conference Call

Insmed will host a conference call beginning today at 8:30 AM 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 5699819. 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 November 9, 2017 by dialing (855) 859-2056 (domestic) or (404) 537-3406 (international) and referencing conference ID number 5699819. 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.

Non-GAAP Financial Measures

In addition to the United States generally accepted accounting principles (GAAP) results, this earnings release includes cash-based operating expenses, a non-GAAP financial measure, which Insmed defines as total operating expenses excluding stock-based compensation expense and depreciation expense. A reconciliation of this non-GAAP financial measure to its most directly comparable GAAP financial measure is presented in the table attached to this press release.

Management believes that this non-GAAP financial measure is useful to both management and investors in analyzing our ongoing business and operating performance. Management believes that providing non-GAAP information to investors, in addition to the GAAP presentation, allows investors to view our financial results in the way that management views financial results. Management does not intend the presentation of this non-GAAP financial measure to be considered in isolation or as a substitute for results prepared in accordance with GAAP. In addition, this non-GAAP financial measure may differ from similarly named measures used by other companies.

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 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

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 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 INS-312 study; lack of safety and efficacy of our product candidates; failure to develop, or to license for development, additional product candidates, including a failure to attract experienced third-party collaborators; failure to obtain, or delays in obtaining, regulatory approval from the United States Food and Drug Administration, Japan's Ministry of Health, Labour and Welfare, the European Medicines Agency, and other regulatory authorities for our product candidates or their delivery devices, including due to insufficient clinical data or 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; 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 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, or to comply with our agreements or laws and regulations that impact our business; failure to comply with license agreements that are critical for our product development, including our license agreements with PARI Pharma GmbH and AstraZeneca AB; inaccuracies in our estimate of the size of the potential markets for our product candidates; 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 trials; uncertainties in the rate and degree of market acceptance of product candidates, if approved; uncertainties in the timing, scope and rate of reimbursement for our product candidates; competitive developments affecting our product candidates; 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; inability to repay our existing indebtedness or to obtain additional financing when needed; failure to obtain, protect and enforce our patents and other intellectual property; 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; 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; failure to comply with the laws and regulations that impact our business; loss of key personnel; and changes in laws and regulations applicable to our business, including those related to pricing and reimbursement of our product candidates.

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.

Financial Statements and Reconciliation to Follow

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