Insmed Reports Second Quarter 2018 Financial Results and Provides Business Update

August 2, 2018

- ***PDUFA (Prescription Drug User Fee Act) Action Date for ALIS (Amikacin Liposome Inhalation Suspension) Set for September 28, 2018***
- ***Actively Preparing for Potential Product Launch in Early Q4 2018***

BRIDGEWATER, N.J., Aug. 02, 2018 (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 second quarter ended June 30, 2018 and provided a business update.

"We are approaching a key inflection point for the business as we prepare for the potential approval and launch of our first commercial product, ALIS, which we studied in adult patients with nontuberculous mycobacterial (NTM) lung disease caused by Mycobacterium avium complex (MAC)," commented Will Lewis, President and Chief Executive Officer of Insmed. "We look forward to the U.S. Food and Drug Administration (FDA) Advisory Committee meeting next week to discuss our New Drug Application for ALIS and the significant unmet need in this orphan disease, for which there are currently no approved inhaled therapies in the U.S. Our commercial team is executing our strategy in an effort to support a potential U.S. launch early in the fourth quarter of this year, and we continue to lay the groundwork for long-term growth, with efforts ongoing to support regulatory submissions in Japan and Europe and plans for additional studies to support life cycle management for ALIS."

Recent Corporate Developments

New Drug Application (NDA) Accepted for Priority Review with PDUFA Action Date of September 28, 2018; FDA Advisory Committee Set for August 7, 2018

In May, Insmed reported that the FDA granted Insmed's request for Priority Review of its NDA for ALIS for adult patients with NTM lung disease caused by MAC and set a PDUFA action date of September 28, 2018. The Division of Antimicrobial Products of the FDA has scheduled an advisory committee meeting to review data supporting the NDA on August 7, 2018. The FDA previously designated ALIS an orphan drug, a breakthrough therapy, and a Qualified Infectious Disease Product (QIDP) under the Generating Antibiotic Incentives Now (GAIN) Act.

ALIS Data Presented at the American Thoracic Society (ATS) 2018 International Conference

In late May, Insmed presented detailed data from its ongoing Phase 3 CONVERT study of ALIS in adult patients with treatment refractory NTM lung disease caused by MAC at the ATS 2018International Conference. The global CONVERT study met its primary endpoint of culture conversion by Month 6 with statistical significance ($p < 0.0001$). In the study, the addition of ALIS to guideline-based therapy (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 alone.

Strengthening Expertise in Japan

In mid-May, Insmed also appointed Leo Lee to its Board of Directors. Mr. Lee has deep global commercial leadership experience, with more than 21 years of his career in the pharmaceutical industry spent in Japan, most recently at Merck KGaA, a global pharmaceutical company, where he served as President, Japan. Prior to his role at Merck KGaA, Mr. Lee served as President, Japan of Allergan plc, a global pharmaceutical company, from 2011 to 2015.

During the second quarter, Insmed hired Yuji Orihara to the position of General Manager, Insmed Asia Pacific, to advance our efforts toward potential commercialization of ALIS in Japan. Mr. Orihara joins Insmed from Gilead Sciences, Inc., where he was most recently the President of Gilead Sciences, Japan.

Second Quarter Financial Results

For the second quarter of 2018, Insmed reported a net loss of $76.4 million, or $1.00 per share, compared with a net loss of $44.7 million, or $0.72 per share, for the second quarter of 2017.

Research and development expenses were $35.7 million for the second quarter of 2018, compared with $26.9 million for the second quarter of 2017. The increase as compared to the second quarter of 2017 was primarily due to an increase in external manufacturing expenses for ALIS production-related activities and higher compensation and related expenses due to an increase in headcount.

General and administrative expenses for the second quarter of 2018 were $37.2 million, compared with $16.6 million for the second quarter of 2017. The increase was primarily due to higher compensation and related expenses due to an increase in headcount, including the hiring of our field force, and higher consulting expenses related to our pre-commercial planning activities for ALIS.

Balance Sheet and Cash Guidance

As of June 30, 2018, Insmed had cash and cash equivalents of $634.3 million. The Company's operating expenses for the second quarter of 2018 were $72.9 million. The cash-based operating expenses for the second quarter of 2018 were $65.3 million.

The Company is investing in the following key activities in 2018: (i) the build-out of the commercial organization to support global expansion activities for ALIS; (ii) manufacturing of commercial inventory and build-out of an additional third-party manufacturing facility; and (iii) clinical activities for ALIS

and the Phase 2 development program for INS1007, along with advancement of other pipeline programs. As a result of these activities, Insmed expects cash-based operating expenses and capital and other cash investments to be in the range of $150 million to $170 million for the second half of 2018.

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 5199733. The call will also be webcast live 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 August 9, 2018 by dialing (855) 859-2056 (domestic) or (404) 537-3406 (international) and referencing conference ID number 5199733. 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 Insmed 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. Insmed 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., Insmed 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. Insmed expects that between 10,000 and 15,000 of these patients will be refractory to treatment. In Japan, Insmed 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. Insmed 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. 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 achieved culture conversion at Month 6 in the ALIS plus GBT arm compared to the GBT-only arm. Patients who achieved culture conversion by Month 6 are continuing in the CONVERT study for an additional 12 months of treatment following the first monthly negative sputum culture. Patients who did not culture convert may have been eligible to enroll 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, non-converting 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 plus 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.

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 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, the outcome of the upcoming advisory committee meeting, extensions of action dates under PDUFA or complexity in the review process for combination products; imposition of significant post-approval regulatory requirements on our product candidates or 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; 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.

Financial Statements and Reconciliation Follow

INSMED INCORPORATED
Consolidated Balance Sheets
(in thousands, except par value and share data)

	As of June 30, 2018 (unaudited)	As of December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 634,329	$ 381,165
Prepaid expenses and other current assets	10,929	8,279
Total current assets	645,258	389,444

In-process research and development	58,200	58,200
Fixed assets, net	17,881	12,432
Other assets	2,905	1,971
Total assets	$ 724,244	$ 462,047
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$ 15,966	$ 14,671
Accrued expenses	29,046	29,339
Other current liabilities	630	646
Total current liabilities	45,642	44,656
Long-term debt, net	307,156	55,567
Other long-term liabilities	805	765
Total liabilities	353,603	100,988
Shareholders' equity:		
Common stock, $0.01 par value; 500,000,000 authorized shares, 77,038,788 and 76,610,508 issued and outstanding shares at June 30, 2018 and December 31, 2017, respectively	770	766
Additional paid-in capital	1,472,699	1,318,181
Accumulated deficit	(1,102,846)	(957,885)
Accumulated other comprehensive income (loss)	18	(3)
Total shareholders' equity	370,641	361,059
Total liabilities and shareholders' equity	$ 724,244	$ 462,047

INSMED INCORPORATED
Consolidated Statements of Net Loss
(in thousands, except per share data)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Revenues	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	35,722	26,871	65,820	49,125
General and administrative	37,160	16,644	69,813	30,359
Total operating expenses	72,882	43,515	135,633	79,484
Operating loss	(72,882)	(43,515)	(135,633)	(79,484)
Investment income	2,729	169	4,769	323
Interest expense	(6,488)	(1,489)	(12,130)	(2,963)
Loss on extingushment of debt	-	-	(2,209)	-
Other income, net	244	200	330	105
Loss before income taxes	(76,397)	(44,635)	(144,873)	(82,019)
Provision for income taxes	40	37	88	67
Net loss	$ (76,437)	$ (44,672)	$ (144,961)	$ (82,086)
Basic and diluted net loss per share	$ (1.00)	$ (0.72)	$ (1.89)	$ (1.32)
Weighted average basic and diluted common shares outstanding	76,767	62,209	76,693	62,126

INSMED INCORPORATED
Reconciliation of GAAP to Non-GAAP Results
(in thousands)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	**2017**	**2018**	**2017**
Total operating expenses - GAAP	$ 72,882	$ 43,515	$ 135,633	$ 79,484
Stock-based compensation expense	(6,629)	(4,559)	(12,303)	(8,591)
Depreciation	(929)	(738)	(1,698)	(1,454)
Cash-based operating expenses - Non-GAAP	**$ 65,324**	**$ 38,218**	**$ 121,632**	**$ 69,439**

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Source: Insmed, Inc.