Insmed Reports Third Quarter 2018 Financial Results and Provides Business Update

—Quarter Highlighted by U.S. FDA Approval of ARIKAYCE® (amikacin liposome inhalation suspension), Followed by Immediate Launch—

BRIDGEWATER, N.J., Oct. 30, 2018 (GLOBE NEWSWIRE) -- Insmed Incorporated (Nasdaq:INSM), a global biopharmaceutical company on a mission to transform the lives of patients with serious and rare diseases, today reported financial results for the third quarter ended September 30, 2018 and provided a business update.

As previously announced, Insmed received accelerated approval from the U.S. Food and Drug Administration (FDA) on September 28, 2018, for ARIKAYCE® (amikacin liposome inhalation suspension) for the treatment of *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen for adult patients who have limited or no alternative treatment options. ARIKAYCE is the first and only therapy approved in the U.S. specifically for patients with MAC lung disease.

"With the FDA approval of ARIKAYCE for the treatment of refractory MAC lung disease, the company has taken a monumental step forward on our journey to transform the lives of patients with serious and rare diseases. We are now focused on executing a successful U.S. launch while laying the groundwork for long-term growth," commented Will Lewis, President and Chief Executive Officer of Insmed. "We look forward to working with the FDA on the design of the post-approval confirmatory study required to support the full approval of ARIKAYCE in a front-line setting; continuing to collaborate with regulators in Europe and Japan to support potential regulatory submissions in those regions; and advancing our pipeline to bring other potential therapies to patients with serious and rare diseases."

Recent Corporate Developments

Commercial Launch of ARIKAYCE Under Way

Following the FDA approval of ARIKAYCE, the Company launched its commercial efforts, with its team of 72 therapeutic specialists conducting outreach to pulmonologists and infectious disease physicians. Insmed also launched the Arikares™ Support Program, which provides dedicated coordinators to help patients navigate the reimbursement process and trainers to provide support to eligible patients in using ARIKAYCE.

***Pivotal Phase 3 CONVERT Study Data Published in* American Journal of Respiratory and Critical Care Medicine**

In September, results from the CONVERT study were published online in the *American Journal of Respiratory and Critical Care Medicine*. As previously reported, the study showed that the addition of ARIKAYCE to guideline-based therapy (GBT) eliminated evidence of nontuberculous mycobacterial (NTM) lung disease caused by MAC in sputum cultures by Month 6 in 29.0% of patients (65/224), compared to 8.9% of patients (10/112) on GBT alone ($p<0.0001$).

New Data Presented at Infectious Disease Week (IDWeek) 2018 and CHEST Annual Meeting

In October, Insmed presented data on MAC lung disease and ARIKAYCE at IDWeek 2018 and at the CHEST Annual Meeting. The presentations included an analysis of the rate and risk factors of all-cause mortality in Medicare beneficiaries with NTM lung disease; an analysis of the incidence and prevalence of NTM lung disease among Medicare beneficiaries; two analyses related to susceptibility in the Phase 3 CONVERT study evaluating the safety and efficacy of ARIKAYCE in adult patients with treatment-refractory NTM lung disease caused by MAC; and interim results from INS-312, the ongoing open-label single-arm extension of the CONVERT study, as of July 2017.

Patent Protection in Japan Extended to 2033

In October, the Japanese Patent Office issued its eighth patent to Insmed for amikacin liposome inhalation suspension, extending exclusivity in Japan by nearly seven and a half years to May 2033. The claims of the patent relate, in part, to systems for treating pulmonary infections.

Third Quarter Financial Results

For the third quarter of 2018, Insmed reported a net loss of $87.7 million, or $1.14 per share, compared with a net loss of $45.2 million, or $0.69 per share, for the third quarter of 2017.

Research and development expenses were $39.5 million for the third quarter of 2018, compared with $26.7 million for the third quarter of 2017. The increase is primarily due to an increase in external manufacturing expenses for ARIKAYCE production-related activities and higher compensation and related expenses due to an increase in headcount.

General and administrative expenses for the third quarter of 2018 were $44.4 million, compared with $17.4 million for the third quarter of 2017. The increase is primarily due to an increase in headcount, including the hiring of our field force, and higher consulting expenses related to pre-commercial planning activities in preparation for the launch of ARIKAYCE.

Balance Sheet and Cash Guidance

As of September 30, 2018, Insmed had cash and cash equivalents of $567.6 million. The Company's operating expenses for the third quarter of 2018 were $84.0 million. The cash-based operating expenses for the third quarter of 2018 were $75.1 million.

The Company is investing in the following key activities in 2018: (i) the build-out of the commercial organization to support the U.S. launch and potential global expansion activities for ARIKAYCE; (ii) manufacturing of commercial inventory and build-out of an additional third-party manufacturing facility; and (iii) clinical trials for ARIKAYCE and the WILLOW study, our Phase 2 development program for INS1007, along with advancement of other pipeline programs. As a result of these activities, Insmed continues to expect cash-based operating expenses and capital and other cash investments to be toward the low end of 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 2556408. 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 November 6, 2018 by dialing (855) 859-2056 (domestic) or (404) 537-3406 (international) and referencing conference ID number 2556408. 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 U.S. 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 MAC Lung Disease

Mycobacterium avium complex (MAC) lung disease is a rare and serious disorder that can significantly increase morbidity and mortality. Patients with MAC lung disease can experience a range of symptoms that often worsen over time, including chronic cough, dyspnea, fatigue, fever, weight loss, and chest pain. In some cases, MAC lung disease can cause severe, even permanent damage to the lungs, and can be fatal.

MAC lung disease is an emerging public health concern worldwide with significant unmet needs. Current guideline-based treatment involves the use of multi-drug regimens that are not specifically approved for MAC lung disease. The course of treatment is often two years or more and is inadequate in treating the disease in many patients.

About ARIKAYCE® (amikacin liposome inhalation suspension)

ARIKAYCE is the first and only FDA-approved therapy indicated for the treatment of *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen for adult patients with limited or no alternative treatment options. ARIKAYCE is a novel, inhaled, once-daily formulation of amikacin, an established antibiotic that was historically administered intravenously and associated with severe toxicity to hearing, balance, and kidney function. Insmed's proprietary PULMOVANCE™ liposomal technology enables the delivery of amikacin directly to the lungs, where it is taken up by lung macrophages where the infection resides. This approach prolongs the release of amikacin in the lungs while limiting systemic exposure. ARIKAYCE is administered once daily using the Lamira™ Nebulizer System manufactured by PARI Pharma GmbH.

About PARI Pharma and the Lamira™ Nebulizer System

ARIKAYCE® (amikacin liposome inhalation suspension) is delivered by a novel inhalation device, the Lamira™ Nebulizer System, developed by PARI. Lamira™ is a quiet, portable nebulizer that enables efficient aerosolization of liquid medications, including liposomal formulations such as ARIKAYCE, via a vibrating, perforated membrane. Based on PARI's 100-year history working with aerosols, PARI is dedicated to advancing inhalation therapies by developing innovative delivery platforms and new pharmaceutical formulations that work together to improve patient care.

About CONVERT (INS-212) and INS-312

CONVERT is a randomized, open-label, global Phase 3 trial designed to confirm the sputum culture conversion results seen in Insmed's Phase 2 clinical trial of ARIKAYCE 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 sputum culture conversion at Month 6 in the ARIKAYCE plus GBT arm compared to the GBT-only arm. Patients who achieved sputum 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 ARIKAYCE plus GBT arm of the INS-212 study will receive an additional 12 months of ARIKAYCE plus GBT. Patients who crossed over from the GBT-only arm of the INS-212 study will receive 12 months of treatment of ARIKAYCE plus GBT.

About Insmed

Insmed Incorporated is a global biopharmaceutical company on a mission to transform the lives of patients with serious and rare diseases. Insmed's first commercial product is ARIKAYCE® (amikacin liposome inhalation suspension), which is approved in the United States for the treatment of *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen for adult patients with limited or no alternative treatment options. MAC lung disease is a rare and often chronic infection that can cause 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 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: failure to successfully commercialize or maintain US approval for ARIKAYCE, the Company's only approved product; uncertainties in the degree of market acceptance of ARIKAYCE by physicians, patients, third-party payers and others in the healthcare community; the Company's inability to obtain full approval of ARIKAYCE from the FDA, including the risk that the Company will not successfully complete the confirmatory post-marketing study required for full approval; inability of the Company, PARI or the Company's third party manufacturers to comply with regulatory requirements related to ARIKAYCE or the Lamira Nebulizer System; the Company's inability to obtain adequate reimbursement from government or third-party payers for ARIKAYCE or acceptable prices for ARIKAYCE; development of unexpected safety or efficacy concerns related to ARIKAYCE; inaccuracies in the Company's estimates of the size of the potential markets for ARIKAYCE; the Company's inability to create an effective direct sales and marketing infrastructure or to partner with third parties that offer such an infrastructure for distribution of ARIKAYCE; failure to obtain regulatory approval to expand ARIKAYCE's indication to a broader patient population; failure to successfully conduct future clinical trials for ARIKAYCE and the Company's product candidates, including due to the Company's limited experience in conducting preclinical development activities and clinical trials necessary for regulatory approval and the Company's inability to enroll or retain sufficient patients to complete the trials or generate data necessary for regulatory approval; risks that the Company's clinical studies will be delayed or that serious side effects will be identified during drug development; failure to obtain regulatory approvals for ARIKAYCE outside the US or for the Company's product candidates in the US, Europe, Japan or other markets; failure of third parties on which the Company is dependent to manufacture sufficient quantities of ARIKAYCE or the Company's product candidates for commercial or clinical needs, to conduct the Company's clinical trials, or to comply with laws and regulations that impact the Company's business or agreements with the Company; the Company's inability to attract and retain key personnel or to effectively manage the Company's growth; the Company's inability to adapt to its highly competitive and changing environment; the Company's inability to adequately protect its intellectual property rights or prevent disclosure of its trade secrets and other proprietary information and costs associated with litigation or other proceedings related to such matters; restrictions imposed on the Company by its material license agreements, including its license agreements with PARI and AstraZeneca AB, and failure of the Company to comply with its obligations under such agreements; the cost and potential reputational damage resulting from litigation to which the Company is or may become a party, including product liability claims; limited experience operating internationally; changes in laws and regulations applicable to the Company's business and failure to comply with such laws and regulations; and inability to repay the Company's existing indebtedness and uncertainties with respect to the Company's ability to access future capital.

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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2018 and any subsequent Company 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 September 30, 2018 (unaudited)
Assets	
Current assets:	
Cash and cash equivalents	$ 567,574
Prepaid expenses and other current assets	9,921
Total current assets	577,495
Intangible assets	59,941
Fixed assets, net	19,526
Other assets	4,551
Total assets	$ 661,513
Liabilities and shareholders' equity	
Current liabilities:	
Accounts payable	$ 16,579
Accrued expenses	40,368
Other current liabilities	472
Total current liabilities	57,419
Long-term debt, net	311,861
Other long-term liabilities	826
Total liabilities	370,106
Shareholders' equity:	

Common stock, $0.01 par value; 500,000,000 authorized shares, 77,085,715 and 76,610,508 issued and outstanding shares at September 30, 2018 and December 31, 2017, respectively	771
Additional paid-in capital	1,481,205
Accumulated deficit	(1,190,589)
Accumulated other comprehensive income (loss)	20
Total shareholders' equity	291,407
Total liabilities and shareholders' equity	$ 661,513

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

	Three Months Ended September 30, 2018	2017	Nine Months Ende 2018
Revenues	$ -	$ -	$ -
Operating expenses:			
Research and development	39,538	26,675	105,358
General and administrative	44,445	17,408	114,258
Total operating expenses	83,983	44,083	219,616
Operating loss	(83,983)	(44,083)	(219,616)
Investment income	2,741	326	7,510
Interest expense	(6,675)	(1,496)	(18,805)
Loss on extingushment of debt	-	-	(2,209)
Other income, net	220	101	550
Loss before income taxes	(87,697)	(45,152)	(232,570)
Provision for income taxes	46	27	134
Net loss	$ (87,743)	$ (45,179)	$ (232,704)
Basic and diluted net loss per share	$ (1.14)	$ (0.69)	$ (3.03)
Weighted average basic and diluted common shares outstanding	77,066	65,312	76,819

INSMED INCORPORATED

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

	Three Months Ended September 30, 2018	2017	Nine Months Ende 2018
Total operating expenses - GAAP	$ 83,983	$ 44,083	$ 219,616
Stock-based compensation expense	(7,902)	(4,741)	(20,205)
Depreciation	(954)	(714)	(2,652)
Cash-based operating expenses - Non-GAAP	**$ 75,127**	**$ 38,628**	**$ 196,759**

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