Insmed Reports Third Quarter 2019 Financial Results and Provides Business Update

--ARIKAYCE® (amikacin liposome inhalation suspension) Total Revenue of $38.9 Million for the Third Quarter of 2019--
--Company Raises Full-Year 2019 ARIKAYCE Revenue Guidance to Range of $133 Million to $138 Million--
--Martina Flammer, M.D., M.B.A., appointed as Chief Medical Officer--

BRIDGEWATER, N.J., Oct. 30, 2019 /PRNewswire/ -- 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, 2019 and provided a business update.

"We remain very pleased with the continued strong performance of the US launch of ARIKAYCE® (amikacin liposome inhalation suspension). Insmed remains guided by our desire to have a positive impact on patients' lives and is powered by our shared sense of purpose to deliver therapies to small patient populations experiencing big health problems," commented Will Lewis, Chairman and Chief Executive Officer of Insmed. "We are also progressing well with our global expansion efforts including filing for regulatory approval of ARIKAYCE in the EU last quarter and advancing our planned regulatory filings in Japan in first half of 2020. We are also looking forward to the topline results from the WILLOW study which we expect in the first quarter of 2020."

Third Quarter 2019 Financial Results

- Total revenue for the third quarter ended September 30, 2019 was $38.9 million, comprising U.S. net sales of $37.8 million and ex-U.S. net sales of $1.1 million. The ex-U.S. net product sales include $0.9 million from the Temporary Authorization for Use (Autorisation Temporaire d'Utilisation or ATU) program in France and $0.2 million from the named patient program in Germany, both compassionate use programs.
- Cost of product revenues (excluding amortization of intangible assets) was $6.4 million for the third quarter of 2019.
- Research and development expenses were $34.3 million for the third quarter of 2019, compared with $39.5 million for the third quarter of 2018.
- Selling, general and administrative expenses for the third quarter of 2019 were $53.3 million, compared with $44.4 million for the third quarter of 2018. The increase was primarily due to milestone payments and other external expenses related to ARIKAYCE.
- For the third quarter of 2019, Insmed reported a GAAP net loss of $60.7 million, or $0.68 per share, compared with a GAAP net loss of $87.7 million, or $1.14 per share, for the third quarter of 2018.

Recent Corporate Developments & Program Highlights

WILLOW Study

Insmed completed enrollment in the six-month Phase 2 WILLOW study of INS1007 for patients with non-cystic fibrosis (CF) bronchiectasis during the 2nd quarter of 2019 and continues to expect top-line data in the first quarter of 2020.

ARIKAYCE Launch and Lifecycle Management

Insmed continues to advance the post-approval confirmatory clinical trial for ARIKAYCE and the Company has initiated efforts to develop an appropriate patient reported outcome (PRO) tool that will enable the assessment of therapies for the treatment of NTM lung disease. Insmed plans to conduct the confirmatory study of ARIKAYCE in a frontline setting of patients with MAC lung disease as well as a separate study in patients with NTM lung disease caused by *Mycobacterium abscessus*.

Insmed Appoints Chief Medical Officer

Martina Flammer, M.D., M.B.A., has been appointed Chief Medical Officer at Insmed, effective mid-December 2019. Dr. Flammer has more than 17 years of experience in both medical and commercial roles. She has launched global brands and managed pipeline portfolios across therapeutic areas and geographies, including the U.S., Europe, Japan and China. Dr. Flammer was most recently Head of Corporate Division Customer Value, Senior Vice President at Boehringer Ingelheim International. She has previously held various roles at Boehringer Ingelheim, including Vice President Clinical Development & Medical Affairs, Specialty Care Business Unit, and Chief Medical Officer, Vice President of Medicine, Regulatory Affairs & Pharmacovigilance, Boehringer Ingelheim Canada. Prior to that, Dr. Flammer held commercial and medical roles at Pfizer. She holds a medical degree from the University of Vienna Medical School, Austria and a Master of Business Administration degree from New York University Stern School of Business.

Financial Guidance and Balance Sheet

As of September 30, 2019, Insmed had cash and cash equivalents of $535.6 million. The Company's total costs and expenses for the third quarter of 2019 were $95.4 million, compared with total costs and expenses for the third quarter of 2018 of $84.0 million. Cash-based operating expenses, as defined below, for the third quarter of 2019 were $72.6 million, compared with cash-based operating expenses for the third quarter of 2018 of $75.1 million.

The Company now expects full-year 2019 total revenue for ARIKAYCE to be in the range of $133 million to $138 million.

The Company plans to continue to invest in the following key activities in 2019:

(i) support of the U.S. launch and commercialization of ARIKAYCE;
(ii) clinical trials including (a) the development and verification of a PRO for NTM lung disease as a pivotal step toward initiating a confirmatory clinical study of ARIKAYCE, (b) the six-month Phase 2 WILLOW study of INS1007 in patients with non-CF bronchiectasis, and (c) the advancement of other pipeline programs including INS1009 and our earlier-stage research pipeline;

(iii) global expansion in Europe and Japan to support pre-commercial activities in those regions and potential regulatory filings in Japan; and
(iv) buildout of an additional third-party manufacturing facility to increase long-term production capacity for ARIKAYCE and its new corporate headquarters facility.

Insmed continues to expect cash-based operating expenses to be in the range of $140 million to $155 million for the second half of 2019. In addition, the Company continues to expect capital expenditures, including those related to its new corporate headquarters facility as well as payments classified within other assets for the future right-of-use asset related to the buildout of an additional third-party manufacturing facility, to be in the range of $20 million to $30 million for the second half of 2019.

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 (888) 317-6003 (domestic) or (412) 317-6061 (international) and referencing conference ID number 6042526. 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 one hour after its completion through November 6, 2019 by dialing (877) 344-7529 (domestic) or (412) 317-0088 (international) and referencing replay access code 10136134. 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 costs and expenses excluding cost of product revenues, stock-based compensation expense, depreciation, amortization of intangibles and milestone payments related to ARIKAYCE. 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 this non-GAAP information to investors, in addition to the GAAP results, 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 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 liposomal amikacin 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 (PARI).

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.

IMPORTANT SAFETY INFORMATION

WARNING: RISK OF INCREASED RESPIRATORY ADVERSE REACTIONS

ARIKAYCE has been associated with an increased risk of respiratory adverse reactions, including hypersensitivity pneumonitis, hemoptysis, bronchospasm, and exacerbation of underlying pulmonary disease that have led to hospitalizations in some cases.

Hypersensitivity Pneumonitis has been reported with the use of ARIKAYCE in the clinical trials. Hypersensitivity pneumonitis (reported as allergic alveolitis, pneumonitis, interstitial lung disease, allergic reaction to ARIKAYCE) was reported at a higher frequency in patients treated with ARIKAYCE plus background regimen (3.1%) compared to patients treated with a background regimen alone (0%). Most patients with hypersensitivity pneumonitis discontinued treatment with ARIKAYCE and received treatment with corticosteroids. If hypersensitivity pneumonitis occurs, discontinue ARIKAYCE and manage patients as medically appropriate.

Hemoptysis has been reported with the use of ARIKAYCE in the clinical trials. Hemoptysis was reported at a higher frequency in patients treated with ARIKAYCE plus background regimen (17.9%) compared to patients treated with a background regimen alone (12.5%). If hemoptysis occurs, manage patients as medically appropriate.

Bronchospasm has been reported with the use of ARIKAYCE in the clinical trials. Bronchospasm (reported as asthma, bronchial hyperreactivity, bronchospasm, dyspnea, dyspnea exertional, prolonged expiration, throat tightness, wheezing) was reported at a higher frequency in patients treated with ARIKAYCE plus background regimen (28.7%) compared to patients treated with a background regimen alone (10.7%). If bronchospasm occurs during the use of ARIKAYCE, treat patients as medically appropriate.

Exacerbations of underlying pulmonary disease has been reported with the use of ARIKAYCE in the clinical trials. Exacerbations of underlying pulmonary disease (reported as chronic obstructive pulmonary disease (COPD), infective exacerbation of COPD, infective exacerbation of bronchiectasis) have been reported at a higher frequency in patients treated with ARIKAYCE plus background regimen (14.8%) compared to patients treated with background regimen alone (9.8%). If exacerbations of underlying pulmonary disease occur during the use of ARIKAYCE, treat patients as medically appropriate.

Ototoxicity has been reported with the use of ARIKAYCE in the clinical trials. Ototoxicity (including deafness, dizziness, presyncope, tinnitus, and vertigo) were reported with a higher frequency in patients treated with ARIKAYCE plus background regimen (17%) compared to patients

treated with background regimen alone (9.8%). This was primarily driven by tinnitus (7.6% in ARIKAYCE plus background regimen vs 0.9% in the background regimen alone arm) and dizziness (6.3% in ARIKAYCE plus background regimen vs 2.7% in the background regimen alone arm). Closely monitor patients with known or suspected auditory or vestibular dysfunction during treatment with ARIKAYCE. If ototoxicity occurs, manage patients as medically appropriate, including potentially discontinuing ARIKAYCE.

Nephrotoxicity was observed during the clinical trials of ARIKAYCE in patients with MAC lung disease but not at a higher frequency than background regimen alone. Nephrotoxicity has been associated with the aminoglycosides. Close monitoring of patients with known or suspected renal dysfunction may be needed when prescribing ARIKAYCE.

Neuromuscular Blockade: Patients with neuromuscular disorders were not enrolled in ARIKAYCE clinical trials. Patients with known or suspected neuromuscular disorders, such as myasthenia gravis, should be closely monitored since aminoglycosides may aggravate muscle weakness by blocking the release of acetylcholine at neuromuscular junctions.

Embryo-Fetal Toxicity: Aminoglycosides can cause fetal harm when administered to a pregnant woman. Aminoglycosides, including ARIKAYCE, may be associated with total, irreversible, bilateral congenital deafness in pediatric patients exposed *in utero*. Patients who use ARIKAYCE during pregnancy, or become pregnant while taking ARIKAYCE, should be apprised of the potential hazard to the fetus.

Contraindications: ARIKAYCE is contraindicated in patients with known hypersensitivity to any aminoglycoside.

Most Common Adverse Reactions: The most common adverse reactions in Trial 1 at an incidence ≥5% for patients using ARIKAYCE plus background regimen compared to patients treated with background regimen alone were dysphonia (47% vs 1%), cough (39% vs 17%), bronchospasm (29% vs 11%), hemoptysis (18% vs 13%), ototoxicity (17% vs 10%), upper airway irritation (17% vs 2%), musculoskeletal pain (17% vs 8%), fatigue and asthenia (16% vs 10%), exacerbation of underlying pulmonary disease (15% vs 10%), diarrhea (13% vs 5%), nausea (12% vs 4%), pneumonia (10% vs 8%), headache (10% vs 5%), pyrexia (7% vs 5%), vomiting (7% vs 4%), rash (6% vs 2%), decreased weight (6% vs 1%), change in sputum (5% vs 1%), and chest discomfort (5% vs 3%).

Drug Interactions: Avoid concomitant use of ARIKAYCE with medications associated with neurotoxicity, nephrotoxicity, and ototoxicity. Some diuretics can enhance aminoglycoside toxicity by altering aminoglycoside concentrations in serum and tissue. Avoid concomitant use of ARIKAYCE with ethacrynic acid, furosemide, urea, or intravenous mannitol.

Overdosage: Adverse reactions specifically associated with overdose of ARIKAYCE have not been identified. Acute toxicity should be treated with immediate withdrawal of ARIKAYCE, and baseline tests of renal function should be undertaken. Hemodialysis may be helpful in removing amikacin from the body. In all cases of suspected overdosage, physicians should contact the Regional Poison Control Center for information about effective treatment.

U.S. INDICATION

LIMITED POPULATION: ARIKAYCE® is indicated in adults, who have limited or no alternative treatment options, for the treatment of *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen in patients who do not achieve negative sputum cultures after a minimum of 6 consecutive months of a multidrug background regimen therapy. As only limited clinical safety and effectiveness data for ARIKAYCE are currently available, reserve ARIKAYCE for use in adults who have limited or no alternative treatment options. This drug is indicated for use in a limited and specific population of patients.

This indication is approved under accelerated approval based on achieving sputum culture conversion (defined as 3 consecutive negative monthly sputum cultures) by Month 6. Clinical benefit has not yet been established. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

Limitation of Use: ARIKAYCE has only been studied in patients with refractory MAC lung disease defined as patients who did not achieve negative sputum cultures after a minimum of 6 consecutive months of a multidrug background regimen therapy. The use of ARIKAYCE is not recommended for patients with non-refractory MAC lung disease.

Patients are encouraged to report negative side effects of prescription drugs to the FDA. Visit **www.fda.gov/medwatch**, or call 1-800-FDA-1088. You can also call the Company at 1-844-4-INSMED.

Please see Full Prescribing Information.

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, ARIKAYCE® (amikacin liposome inhalation suspension), is the first and only therapy approved in the United States for the treatment of refractory *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 chronic, debilitating condition that can cause severe and permanent lung damage. 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 U.S. 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 develop and validate the PRO tool and complete the confirmatory post-marketing study required for full approval; inability of the Company, PARI or the Company's other 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 or in data the Company has used to identify physicians; expected rates of patient uptake, duration of expected treatment, or expected patient adherence or discontinuation rates; 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 conduct and 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, or delays in obtaining, regulatory approvals for ARIKAYCE outside the U.S. or for the Company's product candidates in the U.S., Europe, Japan or other markets, including as a result of the United Kingdom's planned exit from the European Union; 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 or other obligations imposed on the Company by its agreements related to ARIKAYCE or the Company's product candidates, 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; the Company's limited experience operating internationally; 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 and uncertainties with respect to the Company's ability to access future capital; and delays in the execution of plans to build out an additional third-party manufacturing facility and unexpected expenses associated with those plans and with the move into the Company's new headquarters.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Revenues, net	$ 38,885	$ -	$ 90,759	$ -
Costs and expenses:				
Cost of product revenues (excluding amortization of intangible assets)	6,437	-	15,506	-
Research and development	34,340	39,538	99,081	105,358
Selling, general and administrative	53,347	44,445	160,590	114,258
Amortization of intangible assets	1,249	-	3,745	-
Total costs and expenses	95,373	83,983	278,922	219,616
Operating loss	(56,488)	(83,983)	(188,163)	(219,616)
Investment income	2,885	2,741	7,879	7,510
Interest expense	(6,846)	(6,675)	(20,357)	(18,805)
Loss on extinguishment of debt	-	-	-	(2,209)
Other (expense) income, net	(85)	220	(255)	550
Loss before income taxes	(60,534)	(87,697)	(200,896)	(232,570)
Provision for income taxes	148	46	453	134
Net loss	$ (60,682)	$ (87,743)	$ (201,349)	$ (232,704)
Basic and diluted net loss per share	$ (0.68)	$ (1.14)	$ (2.43)	$ (3.03)
Weighted average basic and diluted common shares outstanding	89,245	77,066	82,907	76,819

INSMED INCORPORATED
Consolidated Balance Sheets

(in thousands, except par value and share data)

	As of September 30, 2019 (unaudited)	As of December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 535,632	$ 495,072
Accounts receivable	15,346	5,515
Inventory	23,313	7,032
Prepaid expenses and other current assets	21,313	11,327
Total current assets	595,604	518,946
Intangibles, net	54,930	58,675
Fixed assets, net	52,991	22,636
Operating lease right-of-use assets	39,949	-
Other assets	19,643	4,299
Total assets	$ 763,117	$ 604,556
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$ 20,415	$ 17,741
Accrued expenses	38,908	38,254
Accrued compensation	13,954	22,208
Lease liabilities	11,985	-
Other current liabilities	130	1,529
Total current liabilities	85,392	79,732
Debt, long-term	331,003	316,558
Long-term lease liabilities	31,417	-
Other long-term liabilities	11,264	-
Total liabilities	459,076	396,290
Shareholders' equity:		
Common stock, $0.01 par value; 500,000,000 authorized authorized shares, 89,310,684 and 77,307,521 issued and outstanding shares at September 30, 2019 and December 31, 2018, respectively	893	773
Additional paid-in capital	1,786,667	1,489,664
Accumulated deficit	(1,483,511)	(1,282,162)
Accumulated other comprehensive loss	(8)	(9)
Total shareholders' equity	304,041	208,266
Total liabilities and shareholders' equity	$ 763,117	$ 604,556

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Total costs and expenses - GAAP	$ 95,373	$ 83,983	$ 278,922	$ 219,616
Cost of product revenues (excluding amortization of intangible assets)	(6,437)	-	(15,506)	-
Stock-based compensation expense	(6,794)	(7,902)	(21,083)	(20,205)
Depreciation	(1,004)	(954)	(3,249)	(2,652)
Amortization of intangibles	(1,249)	-	(3,745)	-
Milestone payments related to ARIKAYCE	(7,249)	-	(10,249)	-
Cash-based operating expenses - Non-GAAP	**$ 72,640**	**$ 75,127**	**$ 225,090**	**$ 196,759**

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