Insmed Reports 2014 Fourth Quarter and Full Year Financial Results

Marketing Authorization Application for ARIKAYCETM Validated by the European Medicines Agency

BRIDGEWATER, N.J.--(BUSINESS WIRE)-- Insmed Incorporated (Nasdaq: INSM) today reported financial results for the fourth quarter and year ended December 31, 2014.

Highlights of the fourth quarter of 2014 and recent weeks include:

- Received validation of the Company's Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for ARIKAYCE following the approval of the Pediatric Investigation Plan (PIP) by the EMA's Pediatric Committee;
- Advanced progress of the Company's phase 3 global trial (the "212" trial) for patients with treatment resistant nontuberculous mycobacteria (NTM) lung infections including the activation of multiple clinical trial sites;
- Appointed Myrtle Potter to its Board of Directors;
- Appointed Dr. Olaf Bartsch as Vice President - Europe, General Manager - Germany and Dr. Francois Cornu as Vice President - Europe, General Manager - France to commence the build-out of operations in Europe. Both Drs. Bartsch and Cornu will report to Will Lewis, President and Chief Executive Officer;
- Launched a comprehensive NTM disease state awareness website www.ntmfakten.de for the education of healthcare providers in Germany;
- Entered into a partnership with the European Respiratory Society (ERS) that established an inaugural award in NTM research entitled, ERS Research Award: Innovation in Non-Tuberculous Mycobacteria Science and Medicine, which is designed to advance the science and understanding of NTM lung disease; and
- Company to host an Analyst and Investor Day in New York City on Tuesday, March 24.

"We remain focused on our primary goal of advancing the 212 trial, our global phase 3 study in NTM patients who failed prior standard-of-care treatment. We have activated multiple sites and we continue to expect preliminary top-line clinical results in mid-2016," said Mr. Lewis. "We are also pleased with our progress in Europe on several fronts. The validation of our MAA filing with the EMA starts the formal review process for ARIKAYCE, the hiring of Drs. Bartsch and Cornu to lead our operations and the partnership with ERS show our commitment to bringing ARIKAYCE to patients with NTM lung disease and to CF patients with pseudomonas lung infections. Finally, we are encouraged by the progress we have made with INS1009 treprostinil prodrug for the treatment of pulmonary arterial hypertension (PAH), which remains on track for an investigational new drug (IND) filing and the initiation of a phase 1 clinical trial later this year."

Fourth Quarter 2014 Financial Results

For the fourth quarter of 2014, Insmed posted a net loss of $17.6 million, or ($0.36) per share, compared with a net loss of $16.2 million, or ($0.41) per share, for the fourth quarter of 2013. The increase in net loss was primarily due to increases in personnel costs, manufacturing expenses and pre-commercial activities, offset in part by a benefit from income taxes resulting from the sale of a portion of the Company's New Jersey Net Operating Losses (NOLs) under the State of New Jersey's Technology Business Tax Certificate Transfer Program.

Research and development expense for the fourth quarter of 2014 increased to $14.8 million from $9.6 million in the fourth quarter of 2013. The increase in research and development expense was primarily due to the build-out of additional third-party manufacturing capacity for the production of ARIKAYCE and higher compensation and personnel-related expenses.

General and administrative expense for the fourth quarter of 2014 was $8.3 million compared with $6.0 million in the fourth quarter of 2013. The increase in general and administrative expense primarily resulted from greater personnel costs due to an increase in headcount and an increase in pre-commercial activities.

Full Year 2014 Financial Results

For 2014, Insmed posted a net loss of $79.2 million, or ($1.84) per share, compared with a net loss of $56.1 million, or ($1.60) per share, for the 2013 fiscal year. The increase in net loss was primarily due to $11.5 million in other revenue received in 2013 related to a one-time payment for the sale of the Company's right to receive future royalties under its license agreement with Shire. Also contributing to the net loss in the current year were increases in personnel costs, manufacturing expenses and pre-commercial activities. The impact of these items was offset in part by a benefit from income taxes resulting from the sale of a portion of the Company's New Jersey NOLs under the State of New Jersey's Technology Business Tax Certificate Transfer Program. The $10.4 million benefit from income taxes for the year ended December 31, 2014 represents two sales of NOLs in 2014 as compared to only one sale in the year ended December 31, 2013.

Research and development expense for 2014 increased to $56.3 million from $44.3 million in 2013. The increase in research and development expense was primarily due to the build-out of additional third-party manufacturing capacity, the completion of certain process improvement projects at a third party manufacturing partner and the manufacture of ARIKAYCE for clinical supply. In addition, there was an increase in internal expenses, specifically compensation and personnel-related expenses, including non-cash stock compensation.

General and administrative expense for 2014 was $31.1 million compared with $22.2 million in 2013. The increase in general and administrative expense primarily resulted from an increase in pre-commercial activities and greater personnel costs due to an increase in headcount and expenses related to our new headquarters and laboratory facility in Bridgewater, New Jersey.

Balance Sheet Highlights and Cash Guidance

As of December 31, 2014, the Company had cash and cash equivalents of $159.2 million. The cash balance reflects the fourth quarter sale of NJ NOLs in the amount of $5.0 million, a modification of existing debt which generated proceeds of $5.0 million, and the sale of equity securities which generated proceeds of $1.0 million. In addition, during the fourth quarter of 2014 the Company paid $1.5 million for capital expenditures, primarily related to the build-out of its laboratory facilities in Bridgewater, New Jersey. Excluding depreciation and non-cash stock compensation

expense, the Company's cash operating expenses for the quarter ended December 31, 2014 was $20.0 million. The Company ended 2014 with $25.0 million in long term debt and had working capital of $145.1 million.

In 2015, the Company plans to continue to fund further clinical development of ARIKAYCE and INS-1009, fund investment in third-party manufacturing capacity, support efforts to obtain regulatory approvals and prepare for ARIKAYCE commercialization in Europe. As a result, Insmed estimates that its cash operating expenses for the first half of 2015 will be in the range of $46 million to $56 million. The Company will discuss its 2015 cash operating expenses at the Company's upcoming Analyst and Investor Day in March 2015. The Company expects current cash balances will be sufficient to fund operations into 2016.

Analyst and Investor Day

Insmed will host an Analyst and Investor Day on Tuesday, March 24, 2015 at 8:30 a.m. in New York City. Members of Insmed's senior management team will provide a business update and an overview of the Company's strategies to drive long-term growth. The event will be webcast live beginning at 8:30 a.m. Eastern Time. The live webcast and an archived presentation following the event will be available on the Investor Relations section of Insmed's corporate website at www.insmed.com.

About ARIKAYCE

ARIKAYCE is a form of the antibiotic amikacin, which is enclosed in nanocapsules of lipid called liposomes. This advanced pulmonary liposome technology prolongs the release of amikacin in the lungs while minimizing systemic exposure. The treatment uses biocompatible lipids endogenous to the lung that are formulated into small (0.3 micron), charge-neutral liposomes. ARIKAYCE is administered once-daily using an optimized, investigational eFlow® Nebulizer System manufactured by PARI Pharma GmbH, a novel, highly efficient and portable aerosol delivery system.

About eFlow® Technology and PARI Pharma

ARIKAYCE is delivered by an investigational eFlow® Nebulizer System developed by PARI Pharma and optimized specifically for ARIKAYCE. The optimized device uses eFlow Technology to enable highly efficient aerosolization of medication including liposomal formulations via a vibrating, perforated membrane that includes thousands of laser-drilled holes. Compared with other nebulization technologies, eFlow Technology produces aerosols with a very high density of active drug, a precisely defined droplet size and a high proportion of respirable droplets delivered in the shortest possible period of time. eFlow Technology is not an ultrasonic nebulizer technology and is not a general purpose electronic aerosol generator nebulizer technology. Combined with its quiet mode of operation, small size, light weight and battery use, eFlow Technology reduces the burden of taking daily, inhaled treatments.

About Nontuberculous Mycobacteria (NTM)

Nontuberculous mycobacteria (NTM) are organisms found in the soil and water that can cause serious lung disease in susceptible individuals, for which there are currently limited effective treatments and no approved therapies. The prevalence of NTM disease is reported to be increasing, and according to reports from the American Thoracic Society is believed to be greater than that of tuberculosis in the U.S. According to the National Center for Biotechnology Information, epidemiological studies show that presence of NTM infection is increasing in developing countries, perhaps because of the implementation of tap water. Women with characteristic phenotype are believed to be at higher risk of acquiring NTM infection along with patients with defects on cystic fibrosis transmembrane conductance regulators.

NTM lung disease is often a chronic condition that can lead to progressive inflammation and lung damage, and is characterized by bronchiectasis and cavitary disease. NTM infections often require lengthy hospital stays for medical management. Treatment usually involves multi-drug regimens that can be poorly tolerated and have limited effectiveness, especially in patients with severe disease or in those who have failed prior treatment attempts. According to a company-sponsored patient chart study conducted by Clarity Pharma Research, approximately 50,000 patients suffering from NTM lung disease visited physician offices in the U.S. during 2011.

About INS1009

INS1009, the Company's inhaled treprostinil prodrug for the treatment of pulmonary arterial hypertension (PAH), a chronic, life-threatening disorder characterized by abnormally high blood pressure in the arteries between the heart and lungs. Insmed has applied its product design, drug development and sustained-release formulation expertise to advance INS1009 with the goal of addressing current limitations of inhaled prostacyclin therapies in the treatment of PAH. INS1009 is expected to be delivered once-daily via inhalation. It is designed to provide consistent, effective drug levels and may also reduce the acute systemic effects of current treatment options.

About Pulmonary Arterial Hypertension (PAH)

Pulmonary arterial hypertension, or PAH, is a chronic, life-threatening form of pulmonary hypertension that is characterized by abnormally high blood pressure in the arteries between the heart and lungs. Pulmonary arteries carry blood from the heart to the lungs, where it picks up oxygen to be delivered throughout the body. In PAH, the pulmonary arteries constrict abnormally. This forces the heart to pump harder to maintain adequate blood flow, which causes blood pressure within the lungs to rise. Common early symptoms include shortness of breath, fatigue, weakness, chest pain and syncope (fainting), particularly during physical activity. PAH worsens over time and is life-threatening because the pressure in a patient's pulmonary arteries rises to dangerously high levels, which strains the heart and may lead to heart failure. The one-year mortality rate among patients with PAH is 15% despite currently available treatments. The cause of some cases of PAH is unknown and there is no cure. PAH is considered an orphan disease, afflicting 30,000 to 40,000 people in the U.S. and 200,000 people globally.

There are several prescription medications approved by the U.S. Food and Drug Administration (FDA) to treat the symptoms of PAH and patients typically are treated with combination therapy including endothelin receptor antagonists, PDE-5 inhibitors and prostacyclin agonists. With annual sales exceeding $1 billion, prostacyclins are the preferred choice for treating late-stage disease. Prostacyclin formulations used to treat PAH include oral, intravenous, subcutaneous and inhaled formulations. All existing prostacyclin compounds have the limitation of a short half-life in the body and require multiple dosing sessions per day for inhaled and oral formulations, or the invasiveness of continuous infusion for injectable formulations.

About the European Respiratory Society

The European Respiratory Society (ERS) is the leading professional organization in its field in Europe. It is broad-based, with some 10,000 members and counting in over 100 countries. Its scope covers both basic science and clinical medicine. ERS seeks to alleviate suffering from respiratory disease and promote lung health through research, sharing of knowledge and through medical and public education. For further information, visit www.ersnet.org/ntmawa.

About Insmed

Insmed Incorporated is a biopharmaceutical company dedicated to improving the lives of patients battling serious lung diseases. Insmed is

focused on the development and commercialization of ARIKAYCE, or liposomal amikacin for inhalation, for at least two identified orphan patient populations: patients with nontuberculous mycobacteria (NTM) lung infections and cystic fibrosis (CF) patients with Pseudomonas aeruginosa lung infections. Insmed is also focused on the development of INS1009, the Company's inhaled treprostinil prodrug for the treatment of pulmonary arterial hypertension (PAH), a chronic, life-threatening disorder characterized by abnormally high blood pressure in the arteries between the heart and lungs. For more information, please visit http://www.insmed.com.

Forward-looking statements

This release contains forward-looking statements. Words, and variations of words, such as "intend," "expect," "will," "anticipate," "believe," "continue," "propose" and similar expressions are intended to identify forward-looking statements. Investors are cautioned that such statements in this release, including statements relating to the status, results and timing of clinical trials and clinical data, the anticipated benefits of Insmed's products, the anticipated timing of regulatory submissions, and the ability to obtain required regulatory approvals, bring products to market and successfully commercialize products constitute forward-looking statements that involve risks and uncertainties that could cause actual results to differ materially from those in the forward-looking statements. Such risks and uncertainties include, without limitation, failure or delay of European, Canadian, U.S. Food and Drug Administration and other regulatory reviews and approvals, competitive developments affecting the Company's product candidates, delays in product development or clinical trials or other studies, patent disputes and other intellectual property developments relating to the Company's product candidates, unexpected regulatory actions, delays or requests, the failure of clinical trials or other studies or results of clinical trials or other studies that do not meet expectations, the fact that subsequent analyses of clinical trial or study data may lead to different (including less favorable) interpretations of trial or study results or may identify important implications of a trial or study that are not reflected in Company's prior disclosures, and the fact that trial or study results or subsequent analyses may be subject to differing interpretations by regulatory agencies, the inability to successfully develop the Company's product candidates or receive necessary regulatory approvals, the inability to make product candidates commercially successful, changes in anticipated expenses, changes in the Company's financing requirements or ability to raise additional capital, and other risks and challenges detailed in the Company's filings with the U.S. Securities and Exchange Commission, including its Annual Report on Form 10-K for the year ended December 31, 2014. Investors are cautioned not to place undue reliance on any forward-looking statements that speak only as of the date of this news release. The Company undertakes no obligation to update these forward-looking statements to reflect events or circumstances or changes in its expectations.

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

	Three Months Ended December 31,		Years Ended December 31,	
	2014	2013	2014	2013
Other revenue	$ -	$ -	$ -	$ 11,500
Operating expenses:				
Research and development	14,799	9,625	56,292	44,279
General and administrative	8,267	5,969	31,073	22,236
Total operating expenses	23,066	15,594	87,365	66,515
Operating loss	(23,066)	(15,594)	(87,365)	(55,015)
Investment income	17	25	58	166
Interest expense	(620)	(610)	(2,415)	(2,412)
Other income / (expense), net	(11)	(35)	141	(33)
Loss before income taxes	(23,680)	(16,214)	(89,581)	(57,294)
Benefit from income taxes	(6,033)	-	(10,422)	(1,221)
Net loss and comprehensive loss	$ (17,647)	$ (16,214)	$ (79,159)	$ (56,073)
Basic and diluted net loss per share	$ (0.36)	$ (0.41)	$ (1.84)	$ (1.60)
Weighted average basic and diluted common shares outstanding	49,662	39,116	43,095	34,980

Investor Relations Contacts:
LHA
Anne Marie Fields, Senior Vice President
212-838-3777
afields@lhai.com
or

Bruce Voss, Managing Director
310-691-7100
bvoss@lhai.com