Insmed Reports Fourth Quarter 2016 Financial Results and Provides Business Update

On Track to Report Top-Line Results from CONVERT Study in 2H 2017
Conference call today at 8:30 AM ET

BRIDGEWATER, N.J., Feb. 23, 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 fourth quarter and year ended December 31, 2016 and provided a business update.

Business Update

- **Achieved enrollment objective in global phase 3 study of ARIKAYCE™.** In November 2016, the company announced that it has achieved its patient enrollment objective in the phase 3 study of ARIKAYCE (liposomal amikacin for inhalation). The study, which is known as CONVERT or INS-212, is evaluating ARIKAYCE in treatment refractory nontuberculous mycobacteria (NTM) lung disease caused by Mycobacterium avium complex (MAC). The primary efficacy endpoint is the proportion of subjects who achieve culture conversion at Month 6 in the ARIKAYCE plus multi-drug regimen arm compared to the multi-drug regimen without ARIKAYCE arm. The Company expects to report the top-line data in the second half of 2017.
- **Granted U.S. Patent for ARIKAYCE, extending patent protection by more than five years into 2034.** U.S. Patent 9,566,234 was issued to Insmed on February 14, 2017. The claims of the patent relate in part to systems and methods for treating pulmonary infections, including NTM infections, comprised of an aqueous dispersion of liposomal complexed aminoglycoside, which can be amikacin sulfate, with a nebulizer.
- **Advancing INS1007 toward Phase 2 trial.** In October, the company exclusively licensed global rights to INS1007 (previously AZD7986) from AstraZeneca. INS1007 is a small molecule, oral reversible inhibitor of dipeptidyl peptidase I (DPP1), an enzyme responsible for activating neutrophil serine proteases (NSPs) in neutrophils when they are formed in the bone marrow. In chronic inflammatory lung diseases, neutrophils accumulate in the airways and result in excessive active NSPs that cause lung destruction and inflammation. The company expects to begin a phase 2 dose-ranging study of INS1007 in non-cystic fibrosis (non-CF) bronchiectasis in 2017. Non-CF bronchiectasis is a rare, progressive, neutrophil-driven pulmonary disorder with no approved therapies. Insmed is also evaluating the potential of INS1007 in other indications and expects to announce plans for phase 2 studies in additional disease states by the end of 2017.

"2017 represents a pivotal year for Insmed, with the potential to set the company on a new growth trajectory supported by a portfolio of novel products that address rare diseases," said Will Lewis, president and chief executive officer of Insmed. "We anticipate announcing top-line results from our ongoing phase 3 CONVERT study of ARIKAYCE for the treatment of NTM lung disease which, if positive, may provide the basis for accelerated approval in the U.S. and potentially other parts of the world. We are also focused on advancing our pre-regulatory activities to support a timely filing and continuing to build NTM lung disease awareness among physicians. We plan to manage our clinical, pre-commercial and operational priorities while maintaining our commitment to efficient use of capital."

Fourth Quarter Financial Results

For the fourth quarter of 2016, Insmed posted a net loss of $68.4 million, or $1.10 per share, compared with a net loss of $31.2 million, or $0.51 per share, for the fourth quarter of 2015. The fourth quarter 2016 results include $30.0 million of expense related to an upfront payment to AstraZeneca for INS1007.

Research and development expenses were $54.9 million for the fourth quarter of 2016, compared with $19.6 million for the fourth quarter of 2015. The increase was primarily due to the $30.0 million upfront payment related to INS1007, as well as the advancement of the company's global phase 3 CONVERT study of ARIKAYCE in NTM lung disease and an increase in headcount and related expenses.

General and administrative expenses for the fourth quarter of 2016 were $12.2 million, compared with $12.9 million for the fourth quarter of 2015. The expenses in the fourth quarter of 2016 were relatively flat compared to the prior year and included $3.7 million of expenses related to pre-commercial activities for ARIKAYCE.

Balance Sheet Highlights and Cash Guidance

As of December 31, 2016, Insmed had cash and cash equivalents of approximately $163 million. The company's cash operating expenses for the second half of 2016 were $64 million, not including the $30 million upfront payment related to INS1007 and excluding depreciation and stock-based compensation expense. Insmed ended the fourth quarter of 2016 with $55 million in debt.

The company is investing in the following activities in 2017: (i) clinical development of ARIKAYCE, (ii) regulatory and pre-commercial initiatives for ARIKAYCE, and (iii) preclinical and clinical activities for its earlier-stage development candidate INS1007. As a result of these activities, Insmed expects its cash-based operating expenses to be in the range of $67 million to $77 millionfor the first half of 2017. This range primarily reflects spending for the CONVERT study, the follow on 312 study for those NTM patients that do not convert, and continued regulatory and commercial development of ARIKAYCE. The estimates also include expenses related to INS1007, including inventory purchases as well as preclinical and clinical start-up activities.

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 62287854. 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 March 9, 2017 by dialing (855) 859-2056 (domestic) or (404) 537-3406 (international) and referencing conference ID number 62287854. 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.

About Insmed

Insmed Incorporated is a global biopharmaceutical company focused on the unmet needs of patients with rare diseases. The company is advancing a global phase 3 clinical study of ARIKAYCE (liposomal amikacin for inhalation) for adult patients with treatment refractory nontuberculous mycobacteria (NTM) lung disease caused by MAC, a rare and often chronic infection that is capable of causing irreversible lung damage and can be fatal. There are currently no approved inhaled products specifically indicated for the treatment of refractory NTM lung infections caused by MAC in the United States or European Union (EU). Insmed's earlier-stage clinical pipeline includes INS1007, a novel oral reversible inhibitor of DPP1 with therapeutic potential in non-CF 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 (PAH). 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) identify forward-looking statements.

Forward-looking statements are based upon the company's current expectations and beliefs, and involve known and unknown risks, uncertainties and other factors, which may cause 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, the factors discussed in Item 1A "Risk Factors" in the company's Annual Report on Form 10-K for the year ended December 31, 2016, and the following: the ability to complete development of, receive, and maintain regulatory approval for, and successfully commercialize ARIKAYCE, INS1007, and INS1009; the ability to successfully develop INS1007 (previously AZD7986) for the treatment of non-CF bronchiectasis; estimates of expenses and future revenues and profitability; status, timing, and the results of preclinical studies and clinical trials and preclinical and clinical data described herein and the timing of the release of top-line data of the company's global Phase 3 clinical study of ARIKAYCE; the sufficiency of preclinical and clinical data in obtaining regulatory approval for the company's product candidates; the timing of responses to information and data requests from the US Food and Drug Administration, the European Medicines Agency, and other regulatory authorities; expectation as to the timing of regulatory review and approval; estimates regarding capital requirements, including milestone payments and royalty obligations due to AstraZeneca, and the needs for additional financing, the ability to repay our existing indebtedness, estimates of the size of the potential markets for product candidates; selection and licensing of product candidates; the ability to attract third parties with acceptable development, regulatory and commercialization expertise; the benefits to be derived from corporate license agreements and other third party efforts, including those relating to the development and commercialization of product candidates; the degree of protection afforded to the company by its intellectual property portfolio; the safety and efficacy of product candidates; sources of revenues and anticipated revenues, including contributions from license agreements and other third party efforts for the development and commercialization of products; the ability to create an effective direct sales and marketing infrastructure for products the company elects to market and sell directly; the rate and degree of market acceptance of product candidates; the impact of any litigation the company is a party to, including, without limitation, the class action lawsuit filed against the company; the timing, scope and rate of reimbursement for product candidates; the success of other competing therapies that may become available; and the availability of adequate supply and manufacturing capacity and quality for product candidates.

The company cautions readers not to place undue reliance on any such forward-looking statements, which speak only as of the date they are made. Insmed 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.