Insmed Provides End of Year Regulatory and Clinical Update

BRIDGEWATER, N.J.--(BUSINESS WIRE)-- Insmed Incorporated (Nasdaq:INSM) today provides a regulatory and clinical update on the Company's development programs.

- Insmed has submitted its Marketing Authorization Application (MAA) to theEuropean Medicines Agency (EMA) and is awaiting its validation. The Company has been informed by the EMA that the remaining item to be addressed prior to validation is the approval of the Pediatric Investigation Plan (PIP) by the EMA's Pediatric Committee, which is expected to be completed in the first quarter of 2015.
- Insmed's phase 3 pivotal trial for patients with treatment resistant nontuberculous mycobacteria (NTM) lung infections (the "212" trial) is on track. The protocol has been agreed upon following dialogue with the U.S. Food and Drug Administration (FDA) and the U.S. central Institutional Review Board (IRB) submission is complete. The Company expects to begin screening patients no later than January 2015, as previously indicated.
- Insmed has completed a pre-investigational new drug (IND) meeting with theFDA for INS-1009, its novel formulation of a proven prostacyclin therapy for the treatment of Pulmonary Arterial Hypertension (PAH) and has clarified that, subject to final review of the pre-clinical data, it would be eligible for a 505(b)(2) approval pathway.

About ARIKAYCETM

ARIKAYCETM 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 INS-1009

INS-1009 (treprostinil), the Company's novel inhalation formulation of a proven prostacyclin 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 INS-1009 with the goal of addressing current limitations of inhaled prostacyclin therapies in the treatment of PAH. INS-1009 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 Peripheral 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 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 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 ARIKAYCETM, 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. 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, 2013 and its subsequent quarterly reports on Form 10-Q. 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.

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