Insmed Provides Regulatory Update for ARIKAYCE

Filing Marketing Authorization Application with European Medicines Agency for NTM Lung Disease and CF Indications Confirms US Development Plan for NTM Following FDA Guidance
Conference Call Today at 8:30 a.m. Eastern Time

BRIDGEWATER, N.J.--(BUSINESS WIRE)-- Insmed Incorporated (Nasdaq: INSM) today announced that, following discussions with European regulatory authorities, it intends to file by the end of 2014 a Marketing Authorization Application (MAA) with the European Medicines Agency (EMA) for ARIKAYCE™, or liposomal amikacin for inhalation, for the treatment of nontuberculous mycobacteria (NTM) lung infections in treatment refractory patients as well as for Pseudomonas aeruginosa lung infections in cystic fibrosis (CF) patients.

Insmed also announced that it will proceed with its previously planned Phase 3 study of the effectiveness of ARIKAYCE for the treatment of lung infections in the broad NTM population. This decision follows a meeting with the U.S. Food and Drug Administration (FDA) in which the FDA acknowledged that exploration of the effectiveness of ARIKAYCE in a broader population is appropriate, based on the results of the recently conducted Phase 2 trial, which showed statistically significant negative culture conversion in patients refractory to standard therapy.

The Company also plans to initiate a second Phase 3 study which will be designed to confirm, in as short a timeframe as possible, the positive culture conversion results seen in the Phase 2 study. This confirmatory study will primarily investigate ARIKAYCE for use in the treatment refractory population with mycobacterium avium complex (MAC) NTM lung infections. This subgroup of the Phase 2 trial's patients responded particularly strongly to the treatment.

The Company believes this two-trial approach will enable both the rapid confirmation of the previous study results to provide the quickest path to filing, as well as expansion of the potential overall label for approval. Following discussions with the FDA, both trials will focus on culture conversion as the primary measure of efficacy with additional goals of demonstrating sustainability and safety. The Company expects results from the smaller confirmatory study by the first half of 2016 and results for the larger trial in 2017.

"Our planned filing in Europe is a significant step forward in our goal to bring ARIKAYCE to market to benefit the thousands of European NTM patients refractory to standard therapy, as well as cystic fibrosis patients with Pseudomonas aeruginosa lung infections," said Will Lewis, Insmed's President and Chief Executive Officer. "We will continue to resource our clinical, commercial and manufacturing capabilities in order to expedite our regulatory submissions and prepare for commercial launch, initially in Europe."

"We are encouraged by the FDA's support for our larger Phase 3 study. We have already made extensive preparations for both trials, including design of the protocols and identification of CROs (clinical research organizations). Given the encouraging results of the previous trial, clinicians are keen to work with us to enroll patients quickly. We look forward to continuing to work with the FDA on the regulatory review and to sharing interim data from these studies in a timely fashion."

"We are moving forward purposefully to complete our MAA submission for ARIKAYCE by the end of the year and to initiate our U.S. Phase 3 clinical trials in the coming months," stated Peggy Berry, Vice President of Regulatory Affairs for Insmed. "We greatly appreciate the collaborative and supportive interactions we have had with the EMA and European agency reviewers providing clarity around their requirements to complete a timely review of ARIKAYCE. We also appreciate the clarity the FDA has provided with regard to a regulatory pathway, and their detailed guidance and support for this additional pivotal study work."

Conference Call and Webcast

Insmed management will host an investment community conference call today at 8:30 a.m. Eastern time, to discuss the regulatory strategy for ARIKAYCE to treat NTM lung infections. Shareholders and other interested parties may participate in the call by dialing 888-803-5993 (domestic) or 706-634-5454 (international) and referencing conference ID number 76369157. The call will be webcast live and archived at http://investor.insmed.com/events.cfm.

A replay of the conference call will be accessible two hours after its completion through August 10, 2014 by dialing 855-859-2056 (domestic) or 404-537-3406 (international) and referencing conference ID number 76369157. The call will also be archived for 90 days on the Company's website at www.insmed.com.

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. In Europe, it is estimated that there are approximately 30,000 cases of pulmonary disease attributed to NTM lung infections. In addition, according to several studies, approximately 55,000 patients suffer from NTM lung disease in the United States.

About Cystic Fibrosis

According to the Cystic Fibrosis Foundation, CF is an inherited chronic disease that affects the lungs and digestive system of about 35,000 patients in Europe, 30,000 patients in the U.S., and a total of 70,000 patients worldwide. A defective gene and its protein product cause the body to produce unusually thick, sticky mucus that clogs the lungs and leads to life-threatening lung infections, and obstructs the pancreas and stops natural enzymes from helping the body break down and absorb food. More than half of all CF patients have acquired Pseudomonas lung infections by age 18 and receive extensive and often chronic antibiotic treatments. Antibiotics delivered via inhalation have become part of standard treatment for CF patients with Pseudomonas lung infections. However, due to the thick sticky mucous these patients produce in their lungs, CF patients seldom clear the Pseudomonas, and they become chronically infected. This results in a continuous decline in lung function, despite all currently available antibiotic treatments.

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. ARIKAYCE has received Orphan Drug Designation from the EMA for both NTM lung infections and for Pseudomonas aeruginosa lung infections. In addition, ARIKAYCE has received Breakthrough Therapy, Orphan Drug, Qualified Infectious Disease Product (QIDP) and Fast Track designations from the U.S. FDA for the treatment of NTM lung infections.

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. For more information, please visit 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, without limitation, 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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