Insmed Completes Enrollment of Phase 2 Clinical Trial of ARIKACE to Treat Nontuberculous Mycobacteria Lung Disease in U.S. and Canada

MONMOUTH JUNCTION, NJ -- (Marketwired) -- 10/15/13 -- Insmed Incorporated (NASDAQ: INSM), a biopharmaceutical company focused on developing an inhaled anti-infective to treat patients battling serious lung diseases in orphan indications that are often life-threatening, today announced that the Company has completed patient enrollment in its Phase 2 clinical study of ARIKACE®, or liposomal amikacin for inhalation, for patients with recalcitrant nontuberculous mycobacterial (NTM) lung disease in the U.S. and Canada. ARIKACE has received Orphan Drug, Qualified Infectious Disease Product (QIDP) and Fast Track designations from the U.S. Food and Drug Administration (FDA) for the treatment of NTM lung infections.

Separately, Insmed announced that it commenced the Scientific Advice Working Party (SAWP) process with the European Medicines Agency (EMA) and expects to have discussions with the EMA regarding ARIKACE for NTM lung disease during the fourth quarter of 2013. In addition, the Company is in the process of evaluating other international markets, such as Japan, where NTM lung infections are already a significant and growing unmet medical need. Insmed is actively developing a strategy for more broadly commercializing ARIKACE in this globally uncontested orphan disease market.

The Phase 2 clinical study is a randomized, placebo-controlled study of ARIKACE in adult patients with recalcitrant NTM lung disease. Eligibility for the study required patients to have been on the American Thoracic Society/Infectious Disease Society of America (ATS/IDSA) guideline therapy for at least six months prior to screening with persistently positive mycobacterial culture. All patients in the study continued with their antibiotic regimen, and received additionally, either ARIKACE or placebo, delivered once daily via an optimized, investigational eFlow® Nebulizer System (PARI Pharma GmbH).

The primary efficacy endpoint in the study is the semi-quantitative measurement of the change in mycobacterial density on a seven-point scale from baseline (day one) to the end of the randomized portion of the trial on day 84. Mycobacterial density is a measurement currently used in clinical practice to assess the progress or decline of those patients with recalcitrant NTM. Following the randomized portion of the study, all eligible patients may choose to receive ARIKACE once daily for an additional 84 days in an open-label design.

Patients in the trial are stratified for either Mycobacterium avium complex (MAC) infections or Mycobacterium abscessus infections. These pathogens collectively account for approximately 85% percent of all patients with NTM lung disease in the U.S. Additionally, stratification is performed based on patients with cystic fibrosis versus those who do not have cystic fibrosis.

Certain secondary, tertiary and exploratory endpoints being measured include, but are not limited to, time to sputum conversion, change in clinical signs and symptoms, change in patient related outcomes/quality of life and safety.

The Company has completed recruitment with 90 patients in the trial as required per protocol. The Company has concluded that the study was sufficiently powered to achieve statistical significance with 80% power to demonstrate a one-step change in the seven-step scale measuring bacterial density, as specified in the protocol.

"We are very pleased to have completed enrollment in this important clinical trial of ARIKACE to treat recalcitrant NTM lung infections," stated Renu Gupta, M.D., Executive Vice President Development and Chief Medical Officer ofInsmed. "This is the first controlled clinical trial of an antibiotic in patients suffering from NTM lung disease. There are no FDA-approved treatments in this serious and increasingly prevalent infectious disease, and current treatment regimens include multiple antibiotics that must be given for prolonged periods of time, are often poorly tolerated and can be associated with severe toxicities. Sadly, for many patients, these therapies are often inadequate with few alternative treatment options available."

"Completing enrollment of this trial is a major accomplishment for Insmed, particularly given the challenge of enrolling patients who have completed six months on ATS/IDSA guideline therapy. The current tolerability challenges of the guideline therapy limit the practical utility of the ATS/IDSA recommended treatment regimen and are the reason why approximately half of diagnosed NTM patients do not receive or continue to take the recommended off label therapies," stated Will Lewis, President and Chief Executive Officer of Insmed.

"We entered this study with compelling in vitro and in vivo data showing ARIKACE had good activity against the NTM pathogens. In addition, we demonstrated in our Phase 3 clinical study of ARIKACE in cystic fibrosis patients that our liposomal inhalation technology safely and effectively delivers the drug to the site of infection and reduces bacterial burden," Lewis added.

"Importantly, recent U.S. regulatory designations underscore the importance theFDA places on helping to advance critically needed antibiotics for serious infections such as NTM. Pursuant to our QIDP status, we expect to continue our ongoing dialogue with the FDA regarding the regulatory pathway for registration and approval of ARIKACE to treat NTM. We remain on track to review and release the clinical data and related dialogue with the FDA by the end of the first quarter of 2014."

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. The prevalence of NTM disease is reported to be increasing, and according to reports from the American Thoracic Society, is believed to be likely greater than that of tuberculosis in the United States. 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, characterized by bronchiectasis and cavitary disease. NTM infections often require lengthy hospital stays for medical management. Treatment usually involves lengthy multi-drug regimens that can be poorly tolerated and with 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 ARIKACE®

ARIKACE 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. ARIKACE 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.

ARIKACE has Orphan Drug, QIDP and Fast Track designations from theFDA for the treatment of NTM lung infections. ARIKACE has been granted orphan drug designation in the U.S. by the FDA and in Europe by EMA for the treatment of Pseudomonas infections in patients with cystic fibrosis.

About eFlow® Technology and PARI Pharma

ARIKACE is delivered by an investigational eFlow® Nebulizer System developed by PARI Pharma and optimized specifically for ARIKACE. 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 Insmed

Insmed Incorporated is a biopharmaceutical company dedicated to improving the lives of patients battling serious lung diseases. Insmed plans to develop new therapeutics for unmet medical needs at the intersection of pulmonary, infectious and orphan diseases. Insmed's lead drug candidate, ARIKACE®, or liposomal amikacin for inhalation, is engineered to deliver a proven and potent anti-infective directly to the site of lung infections to improve efficacy, safety, durability and compliance for at least two identified patient populations: cystic fibrosis (CF) patients with Pseudomonas aeruginosa lung infections; and patients with nontuberculous mycobacteria lung infections (NTM). For more information, 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, inability to make product candidates commercially successful, changes in anticipated expenses, changes in the Company's financing requirements or ability 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 endedDecember 31, 2012. 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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