Insmed Announces Withdrawal of Marketing Authorization Application for ARIKAYCE™ in Europe

BRIDGEWATER, N.J., June 08, 2016 (GLOBE NEWSWIRE) -- Insmed Incorporated (Nasdaq:INSM), a global biopharmaceutical company focused on the unmet needs of patients with rare diseases, today announced that it has withdrawn its Marketing Authorization Application (MAA) from the European Medicines Agency (EMA) for ARIKAYCE™ for the treatment of nontuberculous mycobacteria (NTM) lung disease.

The MAA filing was based on data from the company's completed phase 2 study. During the May 2016 Committee for Medicinal Products for Human Use (CHMP) meeting, the CHMP indicated that the phase 2 study did not provide a sufficient amount of evidence to support an approval. Insmed intends to resubmit its MAA when clinical data from its ongoing global phase 3 study are available.

"We remain on track to complete patient enrollment later this year in our landmark global phase 3 CONVERT study," said Will Lewis, president and chief executive officer of Insmed. "ARIKAYCE has the potential to make a significant difference to patients whose NTM lung disease persists despite long-term courses of multi-drug regimens. We are committed to achieving our ultimate goal of making ARIKAYCE available to patients in the US, Europe, and Asia who are living with the devastating effects of this disease."

The company will continue to support numerous compassionate use programs in Europe for certain patients whose physicians believe they may benefit from ARIKAYCE but are unable to participate in the clinical study. Compassionate use programs are generally intended to make products available on a named-patient basis before they are approved or commercially available in accordance with local regulations. The company is currently supporting the compassionate use of ARIKAYCE in France, Germany, and the Netherlands. The company also expects to support a compassionate use program in Italy.

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) in nontuberculous mycobacteria (NTM) lung disease, a rare and often chronic infection that is capable of causing irreversible lung damage and can be fatal. There are currently no products indicated for the treatment of NTM lung disease in the United States or European Union (EU). Insmed's earlier-stage clinical pipeline includes INS1009, a nebulized prodrug formulation of treprostinil that the company believes may offer a differentiated product profile with therapeutic potential in rare pulmonary disorders such as pulmonary arterial hypertension (PAH), idiopathic pulmonary fibrosis (IPF) sarcoidosis, and severe refractory asthma. To complement its internal research, Insmed actively seeks in-licensing opportunities for a broad range of rare diseases. For more information, visit www.insmed.com.

About Nontuberculous Mycobacteria Lung Disease

NTM is a rare and serious disorder associated with increased morbidity and mortality. There is an increasing rate of lung disease caused by NTM and this is an emerging public health concern worldwide. Patients with NTM lung disease may experience a multitude of symptoms such as fever, weight loss, cough, lack of appetite, night sweats, blood in the sputum, and fatigue. Patients with NTM lung disease frequently require lengthy hospital stays to manage their condition. There are no products specifically indicated for the treatment of NTM lung disease in the US, Europe and Canada. Current guideline-based approaches involve multi-drug regimens that may cause severe side effects and treatment can be as long as two years or more.

The prevalence of human disease attributable to NTM has increased over the past two decades. In a decade long study (1997 to 2007), researchers found that the prevalence of NTM in the US is increasing at approximately 8% per year and that NTM patients on Medicare over the age of 65 are 40% more likely to die over the period of the study than those who did not have the disease. A 2015 publication from co-authors from several US government departments projected 181,037 national annual cases in 2014 costing the US healthcare system approximately $1.7 billion.

For more information about NTM lung disease, visit NTMfacts.com.

About ARIKAYCE

ARIKAYCE, or liposomal amikacin for inhalation, is a novel, once daily formulation of amikacin that is in late stage clinical development for patients with NTM lung disease. Amikacin solution for parenteral administration is an established drug that is effective against a variety of NTM; however, its use is limited by the need to administer it intravenously and by toxicity to hearing, balance, and kidney function. Insmed's advanced pulmonary liposome technology uses charge neutral liposomes to deliver amikacin directly to the lung where it is taken up by the lung macrophages where the NTM infection resides. This prolongs the release of amikacin in the lungs while minimizing systemic exposure thereby offering the potential for decreased systemic toxicities. ARIKAYCE's ability to deliver high levels of amikacin directly to the lung distinguishes it from intravenous amikacin. 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.

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, 2015 and subsequent quarterly reports on Form 10-Q, and the following: the ability to complete development of, receive regulatory approval for, and successfully commercialize ARIKAYCE, or liposomal amikacin for inhalation (LAI), and INS1009, nebulized treprostinil prodrug; estimates of expenses and future revenues and profitability; plans to develop and market new products and the timing of these development programs; status, timing, and the results of preclinical studies and clinical trials and preclinical and clinical data described herein; the timing of responses to information and data requests from the US Food and Drug Administration, the European Medicines Agency, and other regulatory authorities; clinical development of product candidates; ability to obtain and maintain regulatory approval for product candidates; expectation as to the timing of regulatory review and approval; estimates regarding our capital requirements and the needs for additional financing; estimates of the size of the potential markets for product candidates; selection and licensing of product candidates; 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; 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 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.