October 5, 2016

Insmed Announces Worldwide License Agreement with AstraZeneca for Oral DPP1 Inhibitor

Insmed expects to advance compound into a phase 2 dose-ranging study in non-cystic fibrosis bronchiectasis in 2017

BRIDGEWATER, N.J., Oct. 05, 2016 (GLOBE NEWSWIRE) -- Insmed Incorporated (Nasdaq:INSM), a global biopharmaceutical company focused on the unmet needs of patients with rare diseases, today announced a licensing agreement with AstraZeneca (NYSE:AZN) for global exclusive rights to AZD7986, a novel oral inhibitor of dipeptidyl peptidase I (DPP1, also known as cathepsin C). DPP1 is an enzyme that catalyzes the activation of neutrophil serine proteases (NSPs), which play a key role in pulmonary diseases such as non-cystic fibrosis bronchiectasis (non-CF bronchiectasis).

Insmed has renamed the compound INS1007 and will pursue an initial indication of non-CF bronchiectasis, a rare, progressive, neutrophil-driven pulmonary disorder in which the bronchi become permanently dilated due to chronic inflammation and infection. Symptoms include chronic cough, excessive sputum production, shortness of breath, and repeated respiratory infections, which can worsen the underlying condition. The estimated global prevalence of non-CF bronchiectasis exceeds 2 million, of which at least 110,000 cases are in the United States. There is currently no cure for non-CF bronchiectasis.

Bronchiectasis increases susceptibility to nontuberculous mycobacterial (NTM) lung disease, and up to 50 percent of patients with bronchiectasis may also have an active NTM infection. NTM lung disease is a rare and often chronic infection that is capable of causing irreversible lung damage and can be fatal. Insmed is currently advancing a global phase 3 clinical study of ARIKAYCE (liposomal amikacin for inhalation) in NTM lung disease. Insmed has also completed a phase 2 study of ARIKAYCE for the treatment of chronic *Pseudomonas aeruginosa* infection in non-CF bronchiectasis.

"With this transaction we have added a highly complementary therapy that aligns perfectly with our established expertise in rare pulmonary diseases," said Will Lewis, president and chief executive officer of Insmed. "Because NTM lung disease and bronchiectasis often co-exist, we can readily leverage our existing relationships with physician experts around the world who are eagerly awaiting new treatment options. We continue to expect patient enrollment in our phase 3 study of ARIKAYCE to conclude later this year and to report top line data in 2017. We expect that when approved, ARIKAYCE and INS1007 will allow us to provide great value to the patients who are living with NTM lung disease and bronchiectasis, as well as the physicians who treat them."

"We are pleased to be working with Insmed on this program from our early stage respiratory portfolio, which represents a novel approach to treating bronchiectasis," said Maarten Kraan, head of the Respiratory and Inflammation Innovative Medicines Unit at AstraZeneca. "Insmed has the expertise and experience required to take AZD7986 forward in this important indication and bring about results that we hope will benefit patients in the future."

In a phase 1 study of healthy volunteers AZD7986 was well tolerated and demonstrated inhibition of the activity of the NSP neutrophil elastase in a dose and concentration dependent manner. In preclinical studies, AZD7986 was shown to effectively and reversibly inhibit DPP1 and the activation of NSPs within maturing neutrophils. Insmed is completing its plans for a phase 2 study in non-CF bronchiectasis. The study is expected to begin in 2017.

Under the terms of the agreement, Insmed will pay AstraZeneca an upfront payment of $30 million. AstraZeneca will be eligible to receive future payments totaling $120 million in future clinical, regulatory, and sales-related milestones. AstraZeneca would also be entitled to receive tiered royalties ranging from a high single-digit to mid-teen. In addition, the agreement provides AstraZeneca with the option to negotiate a future agreement with Insmed for commercialization of AZD7986/INS1007 in chronic obstructive pulmonary disease or asthma.

Insmed recently closed a $55 million debt agreement with Hercules Capital, Inc., which refinanced the company's existing debt and will add $30 million of new debt to fund the upfront payment. The company confirms its cash operating expense guidance for the second half of 2016 of $62 to $72 million. Going forward the company remains committed to maintaining a disciplined use of capital that ensures key corporate activities pertaining to its priority ARIKAYCE and INS1007 programs are fully resourced.

About INS1007

INS1007 is a small molecule, 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. Neutrophils are the most common type of white blood cell and play an essential role in pathogen destruction and inflammatory mediation. Neutrophils contain three NSPs (neutrophil elastase, proteinase 3, and cathepsin G) that have been implicated in a variety of inflammatory diseases. In chronic inflammatory lung diseases, neutrophils accumulate in the airways and result in excessive active NSPs that cause lung destruction and inflammation. INS1007 may decrease the damaging effects of inflammatory diseases, such as non-cystic fibrosis bronchiectasis, by inhibiting DPP1 and its activation of NSPs.

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. The company'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 AstraZeneca

AstraZeneca is a global, science-led biopharmaceutical company that focuses on the discovery, development and commercialisation of prescription medicines, primarily for the treatment of diseases in three therapy areas - Respiratory and Autoimmunity, Cardiovascular and Metabolic Diseases, and Oncology. The company is also active in inflammation, infection and neuroscience through numerous collaborations. AstraZeneca operates in over 100 countries and its innovative medicines are used by millions of patients worldwide. For more information please visit: www.astrazeneca.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, 2015 and subsequent quarterly reports on Form 10-Q, and the following: the ability to successfully develop INS1007 (formerly known as AZD7986) for the treatment of non-CF bronchiectasis; the ability to complete development of, receive, and maintain regulatory approval for, and successfully commercialize ARIKAYCE, INS1007 (formerly known as AZD7986), and INS1009; the number of patients enrolled and the timing of patient enrollment in the company's global phase 3 clinical study of ARIKAYCE; 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; 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 and the needs for additional financing, including for payment milestones and royalty obligations under the license agreement; 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 impact of any litigation the company is a party to, including, without limitation, the class action lawsuit recently 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.

Insmed Incorporated:

Susan Mesco

Head of Investor Relations

908-947-4326


Primary Logo

Source: Insmed Incorporated