Insmed Reports Second Quarter 2015 Financial Results and Provides Business Update

BRIDGEWATER, N.J., Aug. 06, 2015 (GLOBE NEWSWIRE) -- Insmed Incorporated (Nasdaq:INSM), a global biopharmaceutical company focused on the needs of patients with rare diseases, today reported financial results for the second quarter and six months ended June 30, 2015.

Second Quarter and Recent Highlights

- **Global phase 3 study expanding.** Patient enrollment is advancing in the company's global phase 3 study of ARIKAYCE™ (liposomal amikacin for inhalation or LAI) in nontuberculous mycobacteria (NTM) lung disease (the "212" or CONVERT™ study). The company has secured health authority clearance in 10 countries with CONVERT study sites. In addition to the U.S., patient recruitment is underway in Australia, Canada, Europe, and New Zealand. With strong interest among clinical investigators, Insmed is expanding the total number of CONVERT clinical sites to more than 100.
- **Preliminary long-term data from phase 2 clinical study (112 study) support durability of ARIKAYCE treatment effect.** One-year post-ARIKAYCE treatment follow-up data is now available for 14 of 23 patients who achieved culture conversion during the 196-day 112 study. Twelve of the 14 patients remained culture negative for NTM at their one-year follow-up visit. Cultures from the remaining two patients grew in liquid medium only and not on solid medium, which may represent contamination (false-positive) or a new infection rather than a relapse of the original infecting strain.
- **EMA regulatory review progressing.** The European Medicine Agency's (EMA) review of the company's marketing authorization application (MAA) for ARIKAYCE is ongoing. Insmedhas received the EMA's 120-day questions and anticipates responding before the end of 2015.
- **ATU approval secured in France and first product shipped.** The French National Agency for Medicines and Health Products Safety (ANSM) granted LAI a nominative Temporary Authorization for Use (Autorisation Temporaire d'Utilisation or ATU). Pursuant to this program, in July the company shipped product after receiving a request from a physician for a patient in France. Expanded access programs are generally intended to make products available to patients before they are approved or commercially available in accordance with local regulations.
- **INS1009 advancing toward clinical development.** The company is completing a number of IND-enabling preclinical studies and expects to submit its Investigational New Drug (IND) Application and initiate a phase 1 clinical study of INS1009 in the fourth quarter of 2015. INS1009 is the company's nebulized treprostinil prodrug that is being developed for pulmonary arterial hypertension (PAH).
- **Pre-commercial and manufacturing activities advancing.** NTM disease awareness campaigns are advancing in Europe and the U.S. In addition, the build-out of additional third-party commercial-scale manufacturing capacity is proceeding according to plan and the company is on track to have two sources of ARIKAYCE supply established by year-end.
- **Significantly strengthened balance sheet.** Insmed completed a successful equity offering in April raising net proceeds of $222.9 million and finished the second quarter with $335 million in cash. The company's cash operating expenses for the six months ended June 30, 2015 were approximately $46 million.
- **Management team enhancements.** The company continues to make key additions to its leadership team, including the recent additions of Ela Bochenek, vice president global compliance and George Georges, MD, vice president global medical affairs. Ms. Bochenek's prior experience includes NPS Pharmaceuticals, C.R. Bard, Schering-Plough, and Bristol-Myers Squibb. Dr. Georges previously served in numerous U.S. and global medical affairs leadership roles at InterMune, GlaxoSmithKline, and Sanofi.

"Throughout the first half of the year we made considerable progress advancing our stated corporate objectives each of which brings us closer to attaining our vision of building a self-sustaining biopharmaceutical company focused on rare diseases," said Will Lewis, president and chief executive officer of Insmed. "We anticipate the remainder of the year to also be marked by strong execution. Importantly, we are in a solid financial position to support completion of all of our 2015 goals."

Financial Results

For the second quarter of 2015, Insmed posted a net loss of $28.6 million, or ($0.47) per share, compared with a net loss of $23.2 million, or ($0.59) per share, for the second quarter of 2014.

Research and development expenses were $18.2 million for the second quarter of 2015, compared with $14.9 million for the second quarter of 2014. The increase was primarily due to the advancement of the company's global phase 3 CONVERT study of ARIKAYCE in NTM lung disease.

General and administrative expenses for the second quarter of 2015 were $9.7 million, compared with $7.9 million for the second quarter of 2014. The increase was primarily related to non-cash stock-based compensation expense, greater legal and administrative expenses related to the establishment of an improved tax structure, and pre-commercial activities in Europe.

Balance Sheet Highlights and Cash Guidance

As of June 30, 2015, Insmed had cash and cash equivalents of $335.0 million, including net proceeds from a public offering in April 2015 of $222.9 million. Excluding depreciation and non-cash stock compensation expense, the company's cash operating expenses for the six months ended June 30, 2015 were approximately $46 million, which was at the low end of the company's previously guided range of $46 million to $56 million. Insmed ended the second quarter of 2015 with $25.1 million in short-term debt and working capital of $299.0 million.

As previously reported, the company will continue to invest in the following activities: (i) clinical development of ARIKAYCE, (ii) third-party manufacturing capacity, (iii) regulatory and pre-commercial initiatives for ARIKAYCE, and (iv) filing an Investigational New Drug Application and initiating a phase 1 clinical study of INS1009. As a result, Insmed estimates that its cash operating expenses for the second half of 2015 will be in the range of $50 million to $60 million.

Conference Call

Insmed will host a conference call beginning today at 8:30 AM Eastern Time. Shareholders and other interested parties may participate in the conference call by dialing 877-698-3991 (domestic) or 817-522-1636 (international) and referencing conference ID number 92042219. The call

will also be webcast live on the internet on the Company's website at www.insmed.com.

A replay of the conference call will be accessible two hours after its completion through August 20, 2015 by dialing 855-859-2056 (domestic) or 404-537-3406 (international) and referencing conference ID number 92042219. A webcast of the call will also be archived for 90 days under the Investor Relations section of the Company's website at www.insmed.com.

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.

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

Nontuberculous mycobacteria 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 in the United States or European Union. 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 INS1009

INS1009 is the company's nebulized treprostinil prodrug 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 INS1009 with the goal of addressing current limitations of inhaled prostacyclin therapies in the treatment of PAH. INS1009 is expected to be delivered once-daily via nebulization. It is designed to provide consistent, effective drug levels and may also reduce the acute systemic effects of current treatment options.

About Pulmonary Arterial Hypertension

Pulmonary arterial hypertension 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 existing 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 global biopharmaceutical company focused on the 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 can lead to progressive inflammation and lung damage. There are no currently approved treatments for NTM in the United States or European Union (EU). In the EU, the company has filed a marketing authorization application seeking approval of ARIKAYCE for use in patients with NTM, as well as in cystic fibrosis patients with Pseudomonas aeruginosa lung infections. Insmed's earlier-stage pipeline includes INS1009, a nebulized prodrug formulation of treprostinil that the company is developing 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. 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.

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, market conditions, 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 the 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, 2014and 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.

Financial Statements to Follow