ARIKACE Meets Primary Endpoint of Non-Inferiority to TOBI in Phase 3 Clinical Trial in Europe and Canada to Treat Pseudomonas aeruginosa in Cystic Fibrosis Patients

ARIKACE Receives Qualified Infectious Disease Product and Fast Track Designations from the U.S. FDA for the Treatment of Non-Tuberculous Mycobacteria Lung Infections
Conference Call to be Held Monday, July 1st at 8:00 a.m. Eastern Time

MONMOUTH JUNCTION, N.J.--(BUSINESS WIRE)-- Insmed Incorporated (Nasdaq CM: INSM), a biopharmaceutical company focused on developing and commercializing an inhaled anti-infective to treat patients battling serious lung diseases that are often life-threatening, announces positive developments in both of its clinical development programs for ARIKACE®, or liposomal amikacin for inhalation (LAI).

The Company reports that its Phase 3 study of once-daily ARIKACE to treat Pseudomonas aeruginosa (Pa) in cystic fibrosis (CF) patients conducted at 70 sites in Europe and Canada met its primary endpoint of non-inferiority compared with twice-daily TOBI®* (tobramycin inhalation solution) for relative change in forced expiratory volume in one second (FEV1), measured at the end of the third treatment cycle (24 weeks) as compared to baseline.

The Company also announces that the U.S. Food and Drug Administration (FDA) designated ARIKACE as a Qualified Infectious Disease Product (QIDP) for the treatment of Non-Tuberculous Mycobacteria (NTM) lung infections. Additionally, the FDA granted Fast Track designation to ARIKACE for the treatment of NTM.

Phase 3 Clinical Trial Design for ARIKACE to Treat Pa in CF Patients

The Phase 3 trial was an open-label, multi-center, randomized study designed to assess the comparative safety and efficacy of ARIKACE and TOBI in CF patients with Pa. A total of 302 adult and pediatric CF patients with chronic Pa were randomized to receive 28-days of ARIKACE treatment delivered once-daily via an investigational eFlow® Nebulizer System, or TOBI delivered twice-daily via the PARI LC Plus® Nebulizer System over a 24-week treatment period.

The primary endpoint was relative change in FEV1 measured after three treatment cycles, with each cycle consisting of 28 days "on" treatment and 28 days "off" treatment. The study was designed to demonstrate non-inferiority to TOBI at a 5% non-inferiority margin with 80% power agreed upon by the Company and the European Medicines Agency (EMA). Secondary endpoints measured were relative changes in FEV1 at other time points, time to and number of pulmonary exacerbations, time to antibiotic rescue treatment, change in density of Pa in sputum, respiratory hospitalizations and changes in Patient Reported Outcomes assessing Quality of Life.

Primary Endpoint

The Phase 3 clinical trial of ARIKACE achieved its primary endpoint of non-inferiority to TOBI for relative change in FEV1 from baseline to end of study.

Secondary Endpoints

Overall, secondary endpoints showed comparability of once-daily ARIKACE compared with twice-daily TOBI consistent with the primary endpoint of the study. Complete trial data will be reviewed in greater detail and is

expected to be released at a scientific conference later this year. The Company also intends to pursue publication of trial data in a peer-reviewed journal.

Safety

During all three treatment cycles, the safety profile of ARIKACE was comparable to TOBI, with adverse events consistent with those seen in similar studies and expected in a population of CF patients receiving inhaled antibiotics. There was no difference between arms in the reporting of serious adverse events and there were no unexpected adverse events.

"The Phase 3 study of ARIKACE achieved its primary endpoint and demonstrated that ARIKACE is an effective inhaled antibiotic. These results are consistent with our earlier studies of ARIKACE and we believe further strengthen the clinical data packages Insmed is developing for submission to the EMA and Health Canada for ARIKACE in the treatment of CF," said Renu Gupta, MD, FAAP, Executive Vice President Development and Chief Medical Officer of Insmed. "If approved, we believe ARIKACE, with its once-daily administration, represents a novel alternative for clinicians treating CF patients with chronic lung infection caused by Pseudomonas aeruginosa."

"The study achieved its primary endpoint and suggests that once-daily ARIKACE treatment was comparable to twice-daily TOBI treatment, the current gold standard of care for cystic fibrosis patients with Pseudomonas aeruginosa lung infections," stated Felix Ratjen, MD, PhD, FRCPC Head, Division of Respiratory Medicine, Sellers Chair of Cystic Fibrosis, Professor, University of Toronto Hospitalfor Sick Children and Co-Chair of the study Steering Committee. "These results provide hope that an inhaled aminoglycoside that requires only once-daily dosing may be added to the toolbox of available therapies for physicians to treat cystic fibrosis patients who suffer from chronic Pseudomonas aeruginosa lung infections."

"This early review of data is encouraging. ARIKACE has the potential to be the first once-daily treatment, as compared with the current treatment regimens that require multiple dosing each day. Once-daily treatment may significantly enhance patient convenience and compliance," stated Bonnie Ramsey, MD, Seattle Children's Hospital, Director, Cystic Fibrosis Therapeutics, Development Network Coordinating Center and Chair of the study Steering Committee.

"Cystic fibrosis patients chronically infected with Pseudomonas aeruginosa are among the most heavily burdened patients when it comes to the number, frequency and types of treatments they need to take on a daily basis. The data from this study suggest that once-daily treatment with ARIKACE may provide a safe, effective and more convenient alternative to twice-daily TOBI treatment," stated Diana Bilton, MD, Director of Adult CF Centre at the Royal Brompton Hospital in London, and the Principal Investigator of the study. "The hope of a once-daily treatment offers encouragement to cystic fibrosis patients suffering with Pseudomonas aeruginosa lung infections."

"We are very pleased with the results ARIKACE achieved in our Phase 3 trial and our attention now turns to preparing for regulatory filings with the EMA and Health Canada, which we expect to make during the first half of 2014," said Will Lewis, President and Chief Executive Officer of Insmed.

"We appreciate the support of the Cystic Fibrosis Foundation and the European Cystic Fibrosis Society, and thank our principal investigators and patients for their commitment to this study. We look forward to the opportunity to present the complete data set at an upcoming medical conference and to having it published in a peer-reviewed journal. More importantly, if approved by the EMA and Health Canada, we look forward to bringing ARIKACE to market in order to benefit the thousands of CF patients in need of an effective and convenient treatment for these chronic and often life-threatening Pseudomonas aeruginosa lung infections," he added.

Two-Year Extension Study

Eligible patients from this Phase 3 trial were given the option to participate in a two-year, open-label safety study. Approximately 75% of eligible patients have consented to participate in this study. The Company expects to use data from this study as part of the regulatory submissions to the European and Canadian authorities.

ARIKACE for the Treatment of NTM

Insmed is currently enrolling patients in the U.S. and Canada in a Phase 2 clinical trial of LAI to treat NTM lung infections. Of the targeted enrollment of 100 patients, 81 patients have been enrolled in this study to date. The second regularly scheduled Data Safety Monitoring Board (DSMB) meeting was held on June 28th, 2013 and the DSMB again recommended proceeding with the study without modifications.

"NTM lung infection is a disease that is being recognized and diagnosed with increasing frequency in the U.S.

and worldwide. The risk for NTM lung disease is especially high in women as they age beyond the sixth decade of life. Pulmonary and Infectious Disease physicians are seeing these patients in ever growing numbers. While therapy can be reasonably effective for some patients, it routinely entails multiple, potentially toxic and expensive medications that must be given over long periods of time. Sadly, for many other patients, current therapies are inadequate with few alternative treatment strategies available," said Dr. David Griffith, Professor of Medicine, W.A and E. B. Moncrief Distinguished Professor The University of Texas Health Sciences Center and a co-Principal Investigator on the U.S. NTM study.

"Research in the area of NTM lung disease has been woefully underfunded with no major therapeutic advances in the last 20 years. The availability of a once-a-day product that can help reduce bacterial density in these patients would represent a major step forward in the treatment of this disease," added Dr. Griffith.

On June 28, 2013, ARIKACE was granted QIDP designation and Fast Track designation by the FDA for the "treatment of Non-Tuberculous Mycobacterial lung infections." The QIDP designation for ARIKACE will enable Insmed to benefit from certain incentives for the development of new antibiotics, including potentially more frequent and ongoing dialogue with FDA, priority review, and if ARIKACE is ultimately approved by the FDA, a five-year extension of Hatch-Waxman exclusivity. These incentives are provided under the Generating Antibiotic Incentives Now Act (GAIN Act), which was signed into law in July 2012 as part of the FDA Safety and Innovation Act, the fifth authorization of the Prescription Drug User Fee Act.

The Fast Track designation is intended to expedite the review of new drugs that have the potential to serve unmet medical needs in serious or life-threatening conditions. Under this designation Insmed will have the opportunity to submit completed sections of any future NTM New Drug Application (NDA) for rolling review by FDA, rather than waiting until every section of the application is complete before the entire application can be reviewed. FDA's review clock for the NDA will begin once the final section is submitted.

"QIDP designation for ARIKACE underscores the importance the FDA places on helping to advance critically needed antibiotics for serious infections such as NTM," stated Mr. Lewis. "We believe this is an acknowledgment by the FDA of the potentially important role that ARIKACE may play in the treatment of NTM. QIDP and Fast Track designations may help us bring ARIKACE to patients who face these serious and life-threatening lung infections more quickly. We also believe that QIDP status complements the intellectual property protection provided by our underlying patents."

Pursuant to QIDP status, the Company expects to continue its dialogue with the FDA regarding the regulatory pathway for registration and approval of ARIKACE to treat NTM. Insmed expects to complete the Phase 2 data review and related dialogue with the FDA by the end of the first quarter of 2014.

Conference Call and Webcast

Insmed management will host an investment community conference call today at 8:00 a.m. Eastern time, featuring several experts in the fields of CF and NTM. 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 13711485. Slides containing data from the Phase 3 ARIKACE trial will be posted at 7:15 a.m. Eastern time on July 1, 2013 on the homepage of the Company's website at www.insmed.com. The call will also be webcast live and archived on the Company's website at http://investor.insmed.com/events.cfm.

A replay of the conference call will be accessible two hours after its completion through July 15, 2013 by dialing 855-859-2056 (domestic) or 404-537-3406 (international) and referencing conference ID number 13711485. The call will also be archived for 90 days on the Company's website at www.insmed.com.

About Cystic Fibrosis

According to the Cystic Fibrosis Foundation, CF is an inherited chronic disease that affects the lungs and digestive system of about 30,000 children and adults in the U.S. (70,000 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.

About Non-Tuberculous Mycobacteria

NTM infections are acquired directly from the environment, where they are often present in soil and various water sources. 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.

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. ARIKACEis 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 been granted orphan drug designation in the U.S. by the FDA for the treatment of Pseudomonas infections in patients with CF and for the treatment of NTM lung infections. ARIKACE has also received orphan drug designation in Europe by the EMA for the treatment of Pseudomonas infections in patients with CF.

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. PARI Pharma focuses on the development of aerosol delivery devices and inhalation drug development to advance aerosol therapies where drug and device can be optimized together. For more information, please visit www.paripharma.com.

* TOBI® is a Registered Trademark of Novartis Pharmaceuticals Corporation.

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 ARIKACE®, or liposomal amikacin for inhalation, for at least two identified orphan patient populations: CF patients with Pseudomonas aeruginosa lung infections and patients with NTM lung infections. For more information, please visit www.insmed.com.

Forward-Looking Statements

This release contains forward-looking statements that are made pursuant to provisions of Section 21E of the Securities Exchange Act of 1934. 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 the statements contained in this press release, 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 ended December 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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