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Insmed Completes Enrollment of Adult Patients in Phase 3 ASPEN Study of Brensocatib in Bronchiectasis

—Topline Data Remains on Track to be Reported in Q2 of 2024—

—More than 1,700 Adult Patients Enrolled in Global, Registrational Study of First-in-Class Treatment Candidate—

BRIDGEWATER, N.J., March 31, 2023 /[PRNewswire](#)/ -- Insmed Incorporated (Nasdaq:INSM), a global biopharmaceutical company on a mission to transform the lives of patients with serious and rare diseases, announced that adult patient enrollment is complete as of today in its pivotal ASPEN study of brensocatib.

ASPEN is a global, randomized, double-blind, placebo-controlled Phase 3 study to assess the efficacy, safety, and tolerability of brensocatib in patients ages 12 to 85 years with non-cystic fibrosis bronchiectasis. More than 1,700 adult patients have been enrolled and randomized (1:1:1) to receive brensocatib 10 mg, brensocatib 25 mg, or placebo once daily for 52 weeks, followed by 4 weeks off treatment. The primary endpoint is the rate of pulmonary exacerbations over the 52-week treatment period. Insmed anticipates reporting topline data from adults in the ASPEN trial in the second quarter of 2024.

"We are thrilled to have completed adult patient enrollment on time in the ASPEN study, a critical milestone in our efforts to develop a potentially life-transforming therapy for people living with bronchiectasis," said Martina Flammer, M.D., MBA, Chief Medical Officer of Insmed. "ASPEN is the largest single Phase 3 study ever conducted in bronchiectasis, a serious pulmonary disease that today has no approved treatments. We believe the pace of enrollment and enthusiasm of the treating community reflect the significant need for a therapy specifically developed for this disease. We are grateful to the patients, their families, and the clinical trial investigators for their support and commitment to advancing this important work."

The ASPEN study is being conducted at more than 460 sites in nearly 40 countries. The enrollment of adolescent patients ages 12 through <18 is ongoing and does not have an impact on planned submission timelines with regulatory authorities. More information on the ASPEN study is available at [clinicaltrials.gov \(NCT04594369\)](https://clinicaltrials.gov/NCT04594369).

Brensocatib has received Breakthrough Therapy Designation from the U.S. Food and Drug Administration as well as Priority Medicines (PRIME) designation from the European Medicines Agency for patients with bronchiectasis. Insmed is also advancing the development of brensocatib in additional neutrophil-driven inflammatory diseases with significant health burdens and unmet treatment needs.

About Brensocatib

Brensocatib is a small molecule, oral, reversible inhibitor of dipeptidyl peptidase 1 (DPP1) being developed by Insmed for the treatment of patients with bronchiectasis and other neutrophil-mediated diseases. DPP1 is an enzyme responsible for activating neutrophil serine proteases (NSPs), such as neutrophil elastase, 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. In chronic inflammatory lung diseases, neutrophils accumulate in the airways and result in excessive active NSPs that cause lung destruction and inflammation. Brensocatib may decrease the damaging effects of inflammatory diseases such as bronchiectasis by inhibiting DPP1 and its activation of NSPs. Brensocatib is an investigational drug product that has not been approved for any indication in any jurisdiction.

About Bronchiectasis

Bronchiectasis is a severe, chronic pulmonary disorder in which the bronchi become permanently dilated due to

a cycle of infection, inflammation, and lung tissue damage. The condition is marked by frequent pulmonary exacerbations requiring antibiotic therapy and/or hospitalizations. Symptoms include chronic cough, excessive sputum production, shortness of breath, and repeated respiratory infections, which can worsen the underlying condition. Bronchiectasis affects approximately 340,000 to 520,000 patients in the U.S. Today, there are no approved therapies specifically targeting bronchiectasis in the U.S., Europe, or Japan.

About Insmmed

Insmmed Incorporated is a global biopharmaceutical company on a mission to transform the lives of patients with serious and rare diseases. Insmmed's first commercial product is a first-in-disease therapy approved in the United States, Europe, and Japan to treat a chronic, debilitating lung disease. The Company is also progressing a robust pipeline of investigational therapies targeting areas of serious unmet need, including neutrophil-mediated inflammatory diseases and rare pulmonary disorders. Insmmed is headquartered in Bridgewater, New Jersey, with a footprint across Europe and in Japan. For more information, visit www.insmed.com.

Forward-looking Statements

This press release contains forward-looking statements that involve substantial risks and uncertainties. "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) may identify forward-looking statements.

The forward-looking statements in this press release are based upon the Company's current expectations and beliefs, and involve known and unknown risks, uncertainties and other factors, which may cause the Company's actual results, performance and achievements and the timing of certain events to differ materially from the results, performance, achievements or timings discussed, projected, anticipated or indicated in any forward-looking statements. Such risks, uncertainties and other factors include, among others, the following: failure to obtain, or delays in obtaining, regulatory approvals for ARIKAYCE[®] (amikacin liposome inhalation suspension) outside the U.S., Europe or Japan, or for the Company's product candidates in the U.S., Europe, Japan or other markets, including separate regulatory approval for the Lamira[®] Nebulizer System in each market and for each usage; failure to successfully commercialize ARIKAYCE, the Company's only approved product, in the U.S., Europe or Japan (amikacin liposome inhalation suspension, Liposomal 590 mg Nebuliser Dispersion, and amikacin sulfate inhalation drug product, respectively), or to maintain U.S., European or Japanese approval for ARIKAYCE; business or economic disruptions due to catastrophes or other events, including natural disasters or public health crises; impact of the COVID-19 pandemic and efforts to reduce its spread on the Company's business, employees, including key personnel, patients, partners and suppliers; risk that brensocatib or TPIP does not prove to be effective or safe for patients in ongoing and future clinical studies, including, for brensocatib, the ASPEN study; uncertainties in the degree of market acceptance of ARIKAYCE by physicians, patients, third-party payors and others in the healthcare community; the Company's inability to obtain full approval of ARIKAYCE from the U.S. Food and Drug Administration, including the risk that the Company will not successfully or in a timely manner complete the study to validate a patient reported outcome tool and the confirmatory post-marketing clinical trial required for full approval of ARIKAYCE; inability of the Company, PARI or the Company's other third-party manufacturers to comply with regulatory requirements related to ARIKAYCE or the Lamira[®] Nebulizer System; the Company's inability to obtain adequate reimbursement from government or third-party payors for ARIKAYCE or acceptable prices for ARIKAYCE; development of unexpected safety or efficacy concerns related to ARIKAYCE, brensocatib, TPIP or the Company's other product candidates; inaccuracies in the Company's estimates of the size of the potential markets for ARIKAYCE, brensocatib, TPIP or the Company's other product candidates or in data the Company has used to identify physicians, expected rates of patient uptake, duration of expected treatment, or expected patient adherence or discontinuation rates; the risks and uncertainties associated with, and the perceived benefits of, the Company's secured senior loan with certain funds managed by Pharmakon Advisors, LP and the Company's royalty financing with OrbiMed Royalty & Credit Opportunities IV, LP, including our ability to maintain compliance with the covenants in the agreements for the senior secured loan and royalty financing and the perceived impact of the restrictions on the Company's operations under these agreements; the Company's inability to create an effective direct sales and marketing infrastructure or to partner with third parties that offer such an infrastructure for distribution of ARIKAYCE or any of the Company's product candidates that are approved in the future; failure to obtain regulatory approval to expand ARIKAYCE's indication to a broader patient population; risk that the Company's competitors may obtain orphan drug exclusivity for a product that is essentially the same as a product the Company is developing for a particular indication; failure to successfully predict the time and cost of development, regulatory approval and commercialization for novel gene therapy products; failure to successfully conduct future clinical trials for ARIKAYCE, brensocatib, TPIP and the Company's other product candidates due to the Company's limited experience in conducting preclinical development activities and clinical trials necessary for regulatory approval

and its potential inability to enroll or retain sufficient patients to conduct and complete the trials or generate data necessary for regulatory approval, among other things; risks that the Company's clinical studies will be delayed or that serious side effects will be identified during drug development; failure of third parties on which the Company is dependent to manufacture sufficient quantities of ARIKAYCE or the Company's product candidates for commercial or clinical needs, to conduct the Company's clinical trials, or to comply with the Company's agreements or laws and regulations that impact the Company's business or agreements with the Company; the Company's inability to attract and retain key personnel or to effectively manage the Company's growth; the Company's inability to successfully integrate its recent acquisitions and appropriately manage the amount of management's time and attention devoted to integration activities; risks that the Company's acquired technologies, products and product candidates are not commercially successful; the Company's inability to adapt to its highly competitive and changing environment; risk that the Company is unable to maintain its significant customers; risk that government healthcare reform materially increases the Company's costs and damages its financial condition; deterioration in general economic conditions in the U.S., Europe, Japan and globally, including the effect of prolonged periods of inflation, affecting the Company, its suppliers, third-party service providers and potential partners; the Company's inability to adequately protect its intellectual property rights or prevent disclosure of its trade secrets and other proprietary information and costs associated with litigation or other proceedings related to such matters; restrictions or other obligations imposed on the Company by agreements related to ARIKAYCE or the Company's product candidates, including its license agreements with PARI and AstraZeneca AB, and failure of the Company to comply with its obligations under such agreements; the cost and potential reputational damage resulting from litigation to which the Company is or may become a party, including product liability claims; risk that the Company's operations are subject to a material disruption in the event of a cybersecurity attack or issue; business disruptions or expenses related to the upgrade to the Company's enterprise resource planning system; the Company's limited experience operating internationally; changes in laws and regulations applicable to the Company's business, including any pricing reform, and failure to comply with such laws and regulations; the Company's history of operating losses, and the possibility that the Company may never achieve or maintain profitability; goodwill impairment charges affecting the Company's results of operations and financial condition; inability to repay the Company's existing indebtedness and uncertainties with respect to the Company's ability to access future capital; and delays in the execution of plans to build out an additional third-party manufacturing facility approved by the appropriate regulatory authorities and unexpected expenses associated with those plans.

The Company may not actually achieve the results, plans, intentions or expectations indicated by the Company's forward-looking statements because, by their nature, forward-looking statements involve risks and uncertainties because they relate to events and depend on circumstances that may or may not occur in the future. For additional information about the risks and uncertainties that may affect the Company's business, please see the factors discussed in Item 1A, "Risk Factors," in the Company's Annual Report on Form 10-K for the year ended December 31, 2022 and any subsequent Company filings with the Securities and Exchange Commission (SEC).

The Company cautions readers not to place undue reliance on any such forward-looking statements, which speak only as of the date of this press release. The Company disclaims any obligation, except as specifically required by law and the rules of the SEC, 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.

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