Insmed Highlights Progress Across Portfolio and Outlines Growth Strategy at R&D Day

- Initiated Phase 1 study of treprostinil palmitil inhalation powder (TPIP); Phase 2a study in pulmonary arterial hypertension planned for 2021 -
- Expects to initiate registrational Phase 3 ASPEN trial of brensocatib in patients with non-cystic fibrosis bronchiectasis (NCFBE) in fourth quarter of 2020; expansion into additional neutrophil-mediated diseases planned in 2021 -
- Expanding commercial opportunity for ARIKAYCE with planned European launch by end of 2020 and potential Japanese launch in 2021, if approved, in refractory MAC lung disease; expects to initiate registrational program in front-line NTM in 2020 -
- Cash runway of over three years based on currently planned development programs -

BRIDGEWATER, N.J., Sept. 30, 2020 /PRNewswire/ -- Insmed Incorporated (Nasdaq: INSM), a global biopharmaceutical company on a mission to transform the lives of patients with serious and rare diseases, today announced progress across its portfolio of therapies and pipeline candidates, which will be discussed in further detail today at the Company's virtual Research & Development (R&D) Day. The event will feature presentations from the Insmed management team and Ronald J. Oudiz, M.D., FACP, FACC, FCCP, Professor of Medicine at the David Geffen School of Medicine at the University of California Los Angeles (UCLA), the Interim Chief, Division of Cardiology, and a Director at the Liu Center for Pulmonary Hypertension at the Lundquist Institute for Biomedical Innovation at Harbor-UCLA Medical Center, and the lead investigator in the Company's planned Phase 2a trial for treprostinil palmitil inhalation powder (TPIP).

"Our success in building a commercial company with a global footprint and strong pipeline sets the stage for Insmed's next chapter of growth," said Will Lewis, Chair and Chief Executive Officer of Insmed. "With ARIKAYCE, our global expansion efforts and upcoming study in front-line NTM patients has the potential to establish this first-in-disease program as the standard of care in NTM lung disease worldwide. We believe brensocatib, our DPP1 inhibitor, offers an entirely new approach to address a wide array of neutrophil-mediated diseases. We plan to build upon our early leadership position in this space with our anticipated near-term registrational study in NCFBE, followed by a new development program in cystic fibrosis and plans to explore other opportunities across the neutrophil-mediated disease landscape. We are also pleased to announce the initiation of a Phase 1 trial of TPIP, which represents an exciting opportunity to more fully harness the potential of the prostanoid pathway for the treatment of pulmonary arterial hypertension and may offer a disease-modifying effect. We are well capitalized to execute against our strategic objectives during this next phase of growth for Insmed."

Select R&D Day Highlights

Initiated Phase 1 Trial for Treprostinil Palmitil Inhalation Powder

The Company announced today that it has initiated dosing of the first subjects in the Phase 1 healthy volunteer trial of TPIP in the United States. The objective of this first-in-human single ascending dose and multiple ascending dose study is to assess the pharmacokinetics and tolerability profile of TPIP. Top-line data are expected in the first quarter of 2021. The Company also announced plans to initiate a Phase 2a study in patients with pulmonary arterial hypertension (PAH) in early 2021.

Today, Dr. Oudiz will provide a review of the PAH treatment landscape and the remaining unmet medical need. Additionally, the Company will provide an update on TPIP and review key highlights of preclinical data supporting the product candidate's potentially differentiated product profile that could prove to be an advantageous addition to the therapeutic arsenal for the treatment of patients with PAH.

Treprostinil palmitil is a prodrug consisting of treprostinil linked by an ester bond to a 16-carbon chain. The prodrug becomes active when esterases cleave off the 16-carbon chain, resulting in the active molecule

treprostinil. TPIP is formulated as a dry powder for administration in a capsule-based inhalation device.

Building a Leading Neutrophil-Mediated Portfolio with Brensocatib

Insmed will outline its plans to leverage the potential of brensocatib, its small molecule, oral, reversible inhibitor of dipeptidyl peptidase I (DPP1), in an array of neutrophil-mediated diseases, beginning with NCFBE. The Company will discuss the study design for its registrational Phase 3 trial of brensocatib for the treatment of NCFBE. The trial, known as ASPEN, was developed with input from both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). Insmed remains on track to launch the ASPEN trial in the fourth quarter of 2020.

The Company also announced plans to advance a clinical development program for brensocatib in cystic fibrosis (CF) and to discuss this program with health authorities in the first half of 2021.

Global Expansion for ARIKAYCE® (Amikacin Liposome Inhalation Suspension)

ARIKAYCE is the first and only FDA-approved therapy for the treatment of *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen for adult patients with limited or no alternative treatment options. Today the Company will provide an update on its global expansion efforts in Europe and Japan. Insmed recently received a positive opinion from the Committee for Medicinal Products for Human Use of the EMA for the treatment of nontuberculous mycobacterial (NTM) lung infections caused by MAC in adults with limited treatment options who do not have CF. The Company anticipates receiving Marketing Authorization in the European Union by the end of October, with a planned first European launch by end of 2020. In addition, Insmed filed a Japanese New Drug Application (JNDA) with the Japanese Ministry of Health, Labour and Welfare in March of this year. Following an anticipated 12-month review, if ARIKAYCE is approved, the Company would anticipate launching ARIKAYCE in Japan in the middle of 2021.

Additionally, the Company will provide details around its planned registration program to pursue regulatory approval of ARIKAYCE as a front-line therapy designed to establish a new standard of care for patients suffering from NTM lung disease. The clinical study design includes two separate but inter-related clinical trials to be conducted in parallel. ENCORE is the pivotal study intended to fulfill the post-marketing requirement to allow full approval of ARIKAYCE in the United States. The ARISE trial is an interventional study designed to validate the patient-reported outcome (PRO) tool that will be used to measure efficacy in ENCORE. The Company plans to initiate both trials globally in the fourth quarter of 2020.

Financial Update

As of June 30, 2020, Insmed had cash and cash equivalents of $641.9M. As of today, the Company expects its cash runway to extend over three years based on currently planned development programs.

Conference Call

The R&D Day is scheduled to take place today from 10:00 a.m. to 12:30 p.m. ET in a virtual format. The call and accompanying slides will be webcast live on the Company's website at https://investor.insmed.com/events. To listen to the conference call live, please dial (888) 317-6003 (domestic) or (412) 317-6061 (international) and reference conference ID number 0587949.

A replay of the conference call will be accessible approximately on hour after its completion through October 14, 2020 by dialing (877) 344-7529 (domestic) or (412) 317-0088 (international) and referencing replay access code 10147009. A webcast of the call will also be archived for 90 days under the Investor Relations section of the Company's website at https://investor.insmed.com/events.

About Insmed

Insmed Incorporated is a global biopharmaceutical company on a mission to transform the lives of patients with serious and rare diseases. Insmed's first commercial product, ARIKAYCE® (amikacin liposome inhalation suspension), is the first and only therapy approved in the United States for the treatment of refractory *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen for adult patients with limited or no alternative treatment options. MAC lung disease is a chronic, debilitating condition that can cause severe and permanent lung damage. Insmed's earlier-stage clinical pipeline includes brensocatib, a novel oral reversible inhibitor of dipeptidyl peptidase 1 with therapeutic potential in non-cystic fibrosis bronchiectasis and other inflammatory diseases, and treprostinil palmitil inhalation powder, a treprostinil prodrug formulated as a dry powder for inhalation, which may offer a differentiated product profile for rare pulmonary disorders, including pulmonary arterial hypertension. 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 timing 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 outside the U.S. or for the Company's product candidates in the U.S., Europe, Japan or other markets, including the United Kingdom as a result of its recent exit from the European Union; failure to successfully commercialize or maintain U.S. approval for ARIKAYCE, the Company's only approved product; business or economic disruptions due to catastrophes or other events, including natural disasters or public health crises; impact of the novel coronavirus (COVID-19) pandemic and efforts to reduce its spread on the Company's business, employees, including key personnel, patients, partners and suppliers; the risk that brensocatib does not prove effective or safe for patients in future clinical studies, including the ASPEN and STOP-COVID19 studies; 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 FDA, including the risk that the Company will not timely and successfully complete the study to validate a PRO tool and complete the confirmatory post-marketing study required for full approval of ARIKAYCE; inability of the Company, PARI Pharma GmbH (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; inaccuracies in the Company's estimates of the size of the potential markets for ARIKAYCE or its 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 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 is approved in the future; failure to obtain regulatory approval to expand ARIKAYCE's indication to a broader patient population; failure to successfully conduct future clinical trials for ARIKAYCE, brensocatib and the Company's other product candidates, including due to the Company's limited experience in conducting preclinical development activities and clinical trials necessary for regulatory approval and its inability to enroll or retain sufficient patients to conduct and complete the trials or generate data necessary for regulatory approval; risks that our 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 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 adapt to its highly competitive and changing environment; 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 its 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; 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; 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 FDA-approved third-party manufacturing facility and unexpected expenses associated with those plan.

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, 2019, the Company's Quarterly Reports on Form 10-Q for the quarters

ended March 31, 2020 and June 30, 2020 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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