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INSMED INC

FORM 8-K
(Current report filing)

Filed 09/05/17 for the Period Ending 09/05/17

Address	10 FINDERNE AVENUE BUILDING 10 BRIDGEWATER, NJ, 08807
Telephone	908-977-9900
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Symbol	INSM
SIC Code	2834 - Pharmaceutical Preparations
Industry	Biotechnology & Medical Research
Sector	Healthcare
Fiscal Year	12/31

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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington D.C. 20549

FORM 8-K

CURRENT REPORT
PURSUANT TO SECTION 13 OR 15(d)
OF THE SECURITIES EXCHANGE ACT OF 1934

Date of Report (Date of Earliest Event Reported): **September 5, 2017**

INSMED INCORPORATED

(Exact name of registrant as specified in its charter)

Virginia	**000-30739**	**54-1972729**
(State or other jurisdiction of incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

10 Finderne Avenue, Building 10 **Bridgewater, NJ**	**08807**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: (**908) 977-9900**

Not Applicable
(Former name or former address, if changed since last report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

ITEM 7.01 — Regulation FD Disclosure.

On September 5, 2017, Insmed Incorporated (the "Company") issued a press release announcing top-line results from the Phase 3 CONVERT study of ALIS (Amikacin Liposome Inhalation Suspension) in adult patients with treatment-refractory nontuberculous mycobacterial (NTM) lung disease caused by *Mycobacterium avium* complex. The press release is attached hereto as Exhibit 99.1 and incorporated herein by reference.

The Company will host a conference call to discuss the top-line study results on the date hereof at 8:30 AM Eastern Time, and a live webcast of the call will be available through the investor relations section of the Company's website. The slide presentation to be used by the Company during the call is attached hereto as Exhibit 99.2 and incorporated herein by reference.

ITEM 9.01 - Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release issued by Insmed Incorporated on September 5, 2017.
99.2	Insmed Incorporated CONVERT Phase 3 Top-Line Results Data Presentation, dated September 5, 2017.

EXHIBIT INDEX

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Dated: September 5, 2017

INSMED INCORPORATED
By: /s/ Christine Pellizzari
Name: Christine Pellizzari
Title: General Counsel and Corporate Secretary

Insmed Announces Positive Top-Line Results from Phase 3 CONVERT Study of ALIS (Amikacin Liposome Inhalation Suspension) in Adult Patients with Treatment-Refractory Nontuberculous Mycobacterial (NTM) Lung Disease Caused by Mycobacterium Avium Complex (MAC)

- ***Study met primary endpoint of culture conversion (p< 0.0001)***
- ***Positive top-line results from global Phase 3 controlled study in patients with NTM, a rare, progressive, destructive lung infection***
- ***Company plans to pursue accelerated approval and request priority review***
- ***Company to host conference call today at 8:30 am EDT***

BRIDGEWATER, NJ, September 5, 2017 — Insmed Incorporated (Nasdaq: INSM), a global biopharmaceutical company focused on the unmet needs of patients with rare diseases, today announced top-line data from its Phase 3 CONVERT study.

Top-Line Efficacy Data

The global CONVERT study met its primary endpoint of culture conversion by Month 6 with statistical and clinical significance. The study demonstrated that the addition of ALIS to guideline-based therapy (GBT) eliminated evidence of NTM lung disease caused by MAC in sputum by Month 6 in 29% of patients, compared to 9% of patients on GBT alone ($p < 0.0001$). The trial was powered to detect a treatment effect of 15% between the two treatment groups. The CONVERT study enrolled 336 adult patients with NTM lung disease caused by MAC who were refractory to at least six months of GBT. Patients were randomized 2:1 to receive ALIS plus GBT versus GBT alone. The primary endpoint was the proportion of patients achieving culture conversion by Month 6.

Insmed plans to pursue accelerated approval of ALIS under subpart H based on the data from the CONVERT study, which will be reviewed by the Division of Anti-Infective Products. FDA previously granted this product breakthrough therapy designation and fast track status and designated ALIS as a qualified infectious disease product (QIDP) under the Generating Antibiotic Incentives Now (GAIN) Act.

“We consider these compelling top-line data to be a remarkable accomplishment in a rare disease state with no currently approved therapies,” said Will Lewis, President and Chief Executive Officer of Insmed. “We are particularly encouraged by the consistency of these data when compared with our Phase 2 study results, and look forward to additional data as the CONVERT study continues over the next two years. We want to thank all the patients who participated in this trial around the world as well as the physicians who supported them. Treatment of this

serious and potentially debilitating disease is an unmet medical need, and we expect these important data will enable us to submit for accelerated approval."

The Company also reported top-line data for several secondary endpoints as of the 6-month timepoint. Top-line data for the 6-minute walk test indicates no statistically significant difference between patients in the two arms. However, an analysis of these data (per a pre-specified endpoint) shows that patients who achieved culture conversion in either arm demonstrated an improvement in 6-minute walk distance when compared to patients who did not culture convert ($p=0.0108$). Top-line data for the secondary endpoint of time to conversion demonstrated that patients on GBT took approximately 30% longer to convert when compared to patients on ALIS plus GBT ($p<0.0001$). The Company is continuing its analysis of the impact of conversion on a variety of other clinical measures.

"I am extremely pleased with and impressed by the culture conversion results that ALIS demonstrated in treatment-refractory patients with NTM lung disease caused by MAC. The eradication of MAC is the first and most important goal for treatment of patients with MAC lung disease,"said David Griffith, M.D., Professor of Medicine, W.A and E.B. Moncrief Distinguished Professor at The University of Texas Health Sciences Center and Principal Investigator in the CONVERT study. "I am not only encouraged by the higher conversion rate, but also by the faster time to conversion and safety profile of patients in the ALIS arm of the study. Although it is not a parameter routinely used in clinical practice, I also find it encouraging that patients who achieved culture conversion showed improvement in 6-minute walk distance, a quality of life parameter, as was seen in a prior ALIS study."

"Today marks an important advance in our quest to bring a safe and effective treatment to patients who suffer from NTM lung disease caused by MAC. This represents the first ever global Phase 3 controlled study in patients with NTM, a rare, progressive and destructive infection that is associated with irreversible lung damage and increased rates of mortality," said Paul Streck, M.D., Chief Medical Officer of Insmed. "The current guideline-based therapy to which we were compared in this study is not approved for the treatment of this disease, but is generally regarded as the best available option for these patients. Our drug candidate, ALIS, delivers high levels of an aminoglycoside directly to the lung macrophages and pulmonary tissue where the infection resides, and we believe this accounts for the significant impact on conversion that the drug demonstrated in these trial results."

Safety and Tolerability

In the study, serious treatment emergent adverse events were similar between treatment arms. There were no distinctions between treatment arms due to hearing loss or renal impairment, side effects commonly associated with intravenous use of amikacin. The overall dropout rate was 16.1%, with an 8.9% dropout rate in the GBT arm and a 19.6% rate in the ALIS plus GBT arm. Overall the rate of reported adverse events in the ALIS plus GBT arm was higher and these

events were predominately mild or moderate in nature and generally declined after the second month of treatment. These findings are consistent with the Phase 2 study results and demonstrate adverse events similar to those seen in other clinical studies of inhaled antibiotics.

		2:1 Randomization	
Patients Reporting Serious Treatment Emergent Adverse Events >3% in Either Arm		**ALIS + GBT (n=223)**	**GBT (n=112)**
Patients Reporting At Least One Serious Treatment Emergent Adverse Event		20.2% (45)	17.9% (20)
System Organ Class	**Preferred Term**		
Respiratory, Thoracic, Mediastinal Disorders		11.7% (26)	9.8% (11)
	Hemoptysis	2.7% (6)	4.5% (5)
	COPD (exacerbation)	3.1% (7)	0.9% (1)
Infections and Infestations		9.0% (20)	5.4% (6)
	Pneumonia	3.6% (8)	1.8% (2)
Cardiac Disorders		0.4% (1)	4.5% (5)
Patient Deaths		2.7% (6)	4.5% (5)

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 (844) 707-0669 (domestic) or (703) 639-1223 (international) and referencing conference ID number 79817122. The call will also be webcast live on the company's website.

To access the live webcast, or the subsequent archived recording, visit the Investors section of the Insmed website at www.insmed.com. The webcast will be available for replay on Insmed's website for two weeks following the call.

About NTM Lung Disease

NTM is a rare and serious disorder associated with increased rates of morbidity and mortality. There is an increasing prevalence of lung disease caused by NTM, and we believe it is an emerging public health concern worldwide. Patients with NTM lung disease may experience a multitude of symptoms such as fever, weight loss, cough, lack of appetite, night sweats, blood in the sputum, and fatigue. Patients with NTM lung disease frequently require lengthy hospital stays to manage their condition. We are not aware of any approved inhaled therapies specifically indicated for refractory NTM lung disease caused by MAC in North America, Japan or Europe. Current guideline-based approaches involve use of multi-drug regimens not approved for the treatment of NTM lung disease, and treatment can be as long as two years or more.

The prevalence of human disease attributable to NTM has increased over the past two decades. In a decade long study (1997 to 2007), researchers found that the prevalence of NTM in the US was increasing at approximately 8% per year and that NTM patients on Medicare over the age of 65 were 40% more likely to die over the period of the study than those who did not have the disease. In the US, we estimate there will be between 75,000 and 105,000 patients with diagnosed NTM lung disease in 2018, of which we expect 40,000 to 50,000 will be treated for NTM lung disease caused by MAC. We expect that between 10,000 and 15,000 of these patients will be refractory to treatment. In Japan, we estimate there will be between 125,000 and 145,000 patients with diagnosed NTM lung disease in 2018, with approximately 60,000 to 70,000 of those patients being treated for NTM lung disease caused by MAC and 15,000 to 18,000 of these treated patients being refractory to treatment. We also estimate there will be approximately 14,000 patients with diagnosed NTM lung disease in the EU5 (comprised of France, Germany, Italy, Spain and the United Kingdom) in 2018, of which we estimate approximately 4,400 will be treated for NTM lung disease caused by MAC and approximately 1,400 of these treated patients will be refractory to treatment.

About ALIS

ALIS is a novel, inhaled, once daily formulation of amikacin that is in late-stage clinical development for adult patients with treatment-refractory NTM lung disease caused by MAC. Amikacin solution for parenteral administration is an established drug that has activity against a variety of NTM; however, its use is limited by the need to administer it intravenously and by toxicity to hearing, balance, and kidney function. Insmed's advanced pulmonary liposome technology uses charge neutral liposomes to deliver amikacin directly to the lung where it is taken up by the lung macrophages where the NTM infection resides. This prolongs the release of amikacin in the lungs while minimizing systemic exposure thereby offering the potential for decreased systemic toxicities. ALIS's ability to deliver high levels of amikacin directly to the lung distinguishes it from intravenous amikacin. ALIS is administered once daily using an optimized, investigational eFlow® Nebulizer System manufactured by PARI Pharma GmbH (PARI), a portable aerosol delivery system.

About CONVERT

CONVERT is a randomized, open-label, global Phase 3 trial designed to confirm the culture conversion results seen in Insmed's Phase 2 clinical trial of ALIS in patients with refractory NTM lung disease caused by MAC. CONVERT is being conducted in 18 countries at more than 125 sites. The primary efficacy endpoint is the proportion of patients who achieve culture conversion at Month 6 in the ALIS plus GBT arm compared to the GBT-only arm. Patients who achieve culture conversion by Month 6 will continue in the CONVERT study for an additional 12 months of treatment following the first monthly negative sputum culture. Patients who do not culture convert have the option of enrolling in our INS-312 study. INS-312 is a single-arm open-label study where patients will receive ALIS plus GBT for 12 months.

About Insmed

Insmed Incorporated is a global biopharmaceutical company focused on the unmet needs of patients with rare diseases. Our lead product candidate is ALIS for adult patients with treatment refractory NTM lung disease caused by MAC, which is a rare and often chronic infection that is capable of causing irreversible lung damage and can be fatal. We are not aware of any approved inhaled therapies specifically indicated for refractory NTM lung disease caused by MAC in North America, Japan or Europe. Insmed's earlier-stage clinical pipeline includes INS1007, a novel oral reversible inhibitor of dipeptidyl peptidase 1 with therapeutic potential in non-cystic fibrosis bronchiectasis, and INS1009, an inhaled nanoparticle formulation of a treprostinil prodrug that 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. "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 factors include, among others: risks that the full six-month data from the CONVERT study or subsequent data from the remainder of the study's treatment and off-treatment phases will not be consistent with the top-line six-month results of the study; uncertainties in the research and development of our existing product candidates, including due to delays in data readouts, such as the full data from the CONVERT study, patient enrollment and retention or failure of our

preclinical studies or clinical trials to satisfy pre-established endpoints, including secondary endpoints in the CONVERT study and endpoints in the INS-312 study; lack of safety and efficacy of our product candidates; failure to develop, or to license for development, additional product candidates, including a failure to attract experienced third-party collaborators; failure to obtain, or delays in obtaining, regulatory approval from the United States Food and Drug Administration, Japan's Ministry of Health, Labour and Welfare, the European Medicines Agency, and other regulatory authorities for our product candidates or their delivery devices, including due to insufficient clinical data or selection of endpoints that are not satisfactory to regulators, complexity in the review process for combination products or inadequate or delayed data from a human factors study required for U.S. regulatory approval; lack of experience in conducting and managing preclinical development activities and clinical trials necessary for regulatory approval, including the regulatory filing and review process; failure of third parties on which we are dependent to conduct our clinical trials, to manufacture sufficient quantities of our product candidates for clinical or commercial needs, or to comply with our agreements or laws and regulations that impact our business; failure to comply with license agreements that are critical for our product development, including our license agreements with PARI Pharma GmbH and AstraZeneca AB; inaccuracies in our estimate of the size of the potential markets for our product candidates; failure to maintain regulatory approval for our product candidates, if received, due to a failure to satisfy post-approval regulatory requirements, such as the submission of sufficient data from confirmatory clinical trials; uncertainties in the rate and degree of market acceptance of product candidates, if approved; uncertainties in the timing, scope and rate of reimbursement for our product candidates; competitive developments affecting our product candidates; inaccurate estimates regarding our future capital requirements, including those necessary to fund our ongoing clinical development, regulatory and commercialization efforts as well as milestone payments or royalties owed to third parties; inability to repay our existing indebtedness or to obtain additional financing when needed; failure to obtain, protect and enforce our patents and other intellectual property; inability to create an effective direct sales and marketing infrastructure or to partner with third parties that offer such an infrastructure for distribution of our product candidates, if approved; the cost and potential reputational damage resulting from litigation to which we are a party, including, without limitation, the class action lawsuit pending against us; failure to comply with the laws and regulations that impact our business; loss of key personnel; and changes in laws and regulations applicable to our business, including those related to pricing and reimbursement of our product candidates.

For additional information about the risks and uncertainties that may affect our 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, 2016.

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 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.

Investor Contact:

Blaine Davis
Vice President, Head of Investor Relations
Insmed Incorporated
(908) 947-2841
blaine.davis@insmed.com

Exhibit 99.2

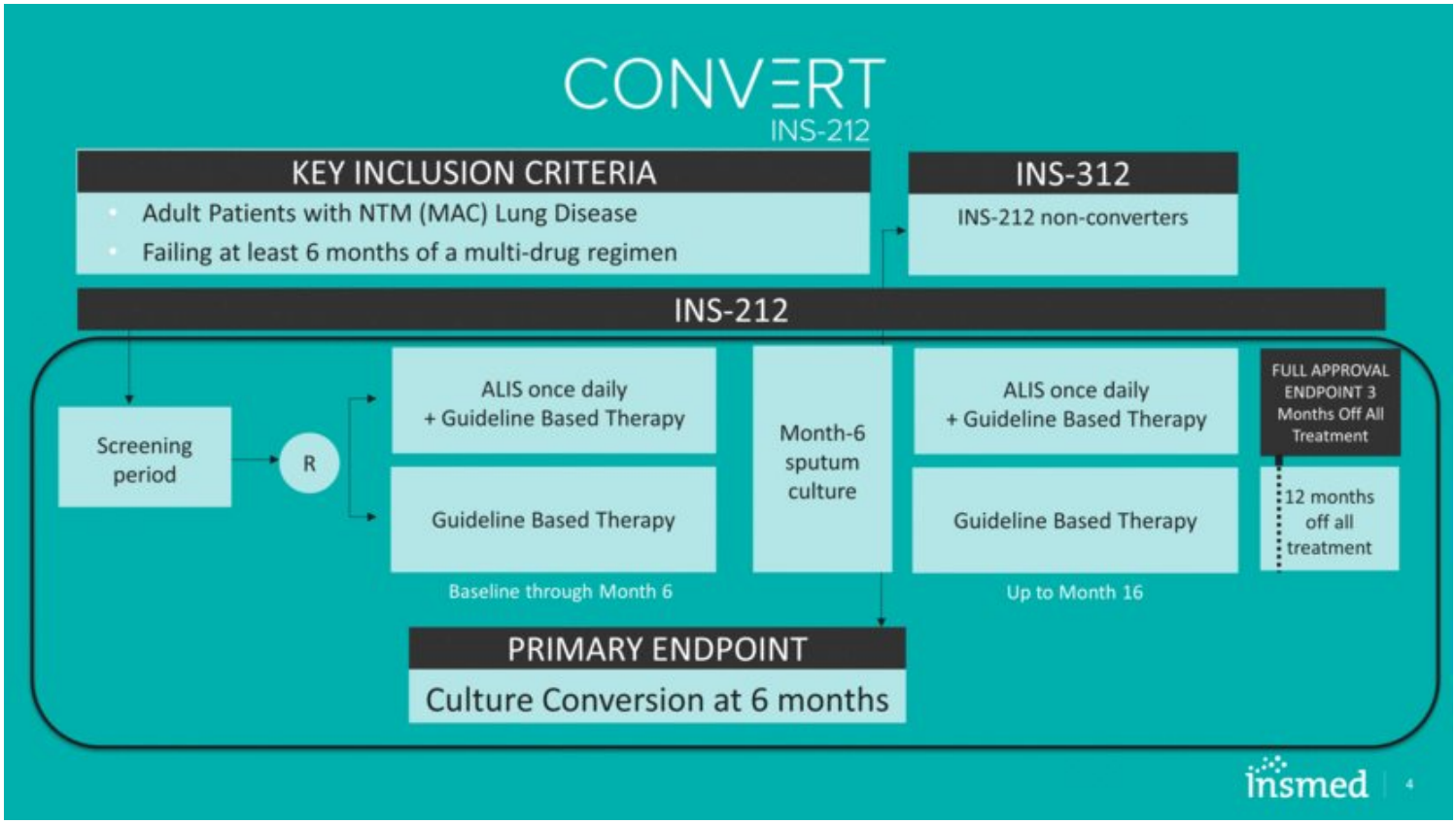
Safe Harbor Statement

This presentation 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) 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 factors include, among others: risks that the full six-month data from the CONVERT study or subsequent data from the remainder of the study's treatment and off-treatment phases will not be consistent with the top-line six-month results of the study; uncertainties in the research and development of our existing product candidates, including due to delays in data readouts, such as the full data from the CONVERT study, patient enrollment and retention or failure of our preclinical studies or clinical trials to satisfy pre-established endpoints, including secondary endpoints in the CONVERT study and endpoints in the INS-312 study; lack of safety and efficacy of our product candidates; failure to develop, or to license for development, additional product candidates, including a failure to attract experienced third-party collaborators; failure to obtain, or delays in obtaining, regulatory approval from the United States Food and Drug Administration, Japan's Ministry of Health, Labour and Welfare, the European Medicines Agency, and other regulatory authorities for our product candidates or their delivery devices, including due to insufficient clinical data or selection of endpoints that are not satisfactory to regulators, complexity in the review process for combination products or inadequate or delayed data from a human factors study required for US regulatory approval; lack of experience in conducting and managing preclinical development activities and clinical trials necessary for regulatory approval, including the regulatory filing and review process; failure of third parties on which we are dependent to conduct our clinical trials, to manufacture sufficient quantities of our product candidates for clinical or commercial needs, or to comply with our agreements or laws and regulations that impact our business; failure to comply with license agreements that are critical for our product development, including our license agreements with PARI Pharma GmbH and AstraZeneca AB; inaccuracies in our estimate of the size of the potential markets for our product candidates; failure to maintain regulatory approval for our product candidates, if received, due to a failure to satisfy post-approval regulatory requirements, such as the submission of sufficient data from confirmatory clinical trials; uncertainties in the rate and degree of market acceptance of product candidates, if approved; uncertainties in the timing, scope and rate of reimbursement for our product candidates; competitive developments affecting our product candidates; inaccurate estimates regarding our future capital requirements, including those necessary to fund our ongoing clinical development, regulatory and commercialization efforts as well as milestone payments or royalties owed to third parties; inability to repay our existing indebtedness or to obtain additional financing when needed; failure to obtain, protect and enforce our patents and other intellectual property; inability to create an effective direct sales and marketing infrastructure or to partner with third parties that offer such an infrastructure for distribution of our product candidates, if approved; the cost and potential reputational damage resulting from litigation to which we are a party, including, without limitation, the class action lawsuit pending against us; failure to comply with the laws and regulations that impact our business; loss of key personnel; and changes in laws and regulations applicable to our business, including those related to pricing and reimbursement of our product candidates. For additional information about the risks and uncertainties that may affect our 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, 2016. The Company cautions readers not to place undue reliance on any such forward-looking statements, which speak only as of the date of this presentation. The Company 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.

OUR MISSION

To transform the lives of patients battling serious rare diseases

- *Top-line data indicates Phase 3 CONVERT trial met primary endpoint of culture conversion*
- *Company plans to pursue accelerated approval and request priority review*

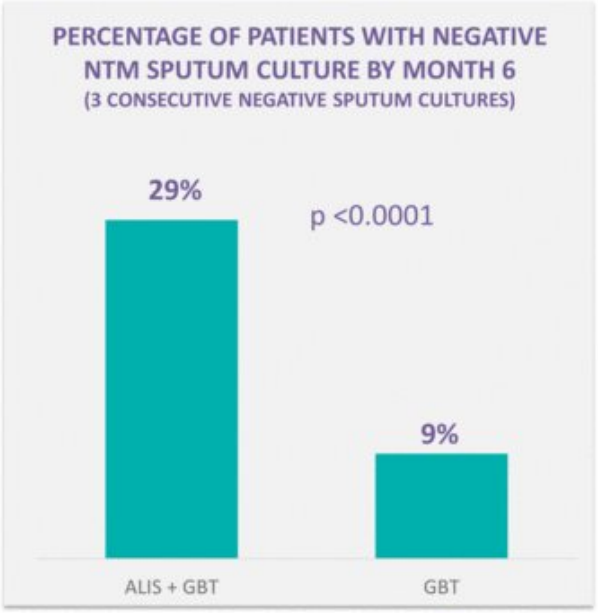

CONVERT
INS-212
KEY INCLUSION CRITERIA
Adult Patients with NTM (MAC) Lung Disease
Failing at least 6 months of a multi-drug regimen
INS-312
INS-212 non-converters
INS-212
Screening period
R
ALIS once daily + Guideline Based Therapy
Guideline Based Therapy
Baseline through Month 6
Month-6 sputum culture
ALIS once daily + Guideline Based Therapy
Guideline Based Therapy
Up to Month 16
FULL APPROVAL ENDPOINT 3 Months Off All Treatment
12 months off all treatment
PRIMARY ENDPOINT
Culture Conversion at 6 months
insmed
4

Top-line Data Indicates CONVERT Study Met Primary Endpoint

- Addition of ALIS to GBT* eliminated evidence in sputum of NTM lung disease caused by MAC by Month 6 in 29% of patients, compared to 9% of patients on GBT alone (p <0.0001)

- *20% absolute difference in treatment groups in favor of ALIS arm*

*Guideline Based Therapy



Top-line CONVERT Study Results – Secondary Endpoints at Month 6

6-MINUTE WALK DISTANCE (6MWD)

- No statistically significant difference between patients in the two arms
- Analysis per a pre-specified endpoint shows that patients who achieved culture conversion across both arms demonstrated an improvement in 6-minute walk distance vs. patients who did not culture convert ($p=0.0108$)

TIME TO CONVERSION

- Patients in the GBT-only arm took approximately 30% longer to convert when compared to patients on ALIS plus GBT ($p<0.0001$)

CONVERT Safety Summary

- Adverse events, consistent with those seen with use of inhaled antibiotics, more frequent in ALIS + GBT arm
- Serious treatment emergent adverse events were similar between treatment arms
- No distinctions between treatment arms of hearing loss or renal impairment

SERIOUS TEAES >3%		2:1 Randomization ALIS (n=223)	GBT (n=112)
Patients Reporting at Least One Serious Treatment Emergent Adverse Event		20.2% (45)	17.9% (20)
System Organ Class	**Preferred Term**		
Respiratory, Thoracic, Mediastinal Disorders		11.7% (26)	9.8% (11)
	Hemoptysis	2.7% (6)	4.5% (5)
	COPD (exacerbation)	3.1% (7)	0.9% (1)
Infections and Infestations		9.0% (20)	5.4% (6)
	Pneumonia	3.6% (8)	1.8% (2)
Cardiac Disorders		0.4% (1)	4.5% (5)
Patient Deaths		2.7% (6)	4.5% (5)
Drop Outs (%)		19.6% (44)	8.9% (10)
Total Drop Outs (%)		16.1% (54)	

CONVERT Study Demographics Summary

	ALIS (n=224)	GBT (n=112)
SMOKERS	11.6% (26)	8.9% (10)
GUIDELINE BASED THERAPY PRIOR TO ENROLLMENT		
ON TREATMENT	89.7% (201)	90.2% (101)
OFF TREATMENT FOR > 3 MONTHS	10.3% (23)	9.8% (11)
GEOGRAPHY		
US	41.5% (93)	42.9% (48)
ASIA	21.4% (48)	17.9% (20)
EU + ROW	37.1% (83)	39.3% (44)
MALE	26.3% (59)	39.3% (44)
FEMALE	73.7% (165)	60.7% (68)
MEAN AGE (YEARS)	64.6	64.9

Pathway to US Submission – Division of Anti-Infective Products

Accelerated Approval	• NDA Submission planned based on primary endpoint under Subpart H
Orphan and QIDP	• 12 years of exclusivity (7 orphan and 5 QIDP) and likely priority review
Breakthrough Designation	• Guidance on efficient drug development; allows for rolling submission
Full Approval Requirement	• Confirmatory endpoint contained within study design

Insmed: A Global Biopharmaceutical Focused on Rare Disease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