Summary of US Expanded Access Policy

Expanded access generally refers to access to, and the use of, an investigational product (i.e., one that has not been approved by the FDA) outside of a clinical trial. Whereas the primary purpose of use of an investigational product in a clinical study is to gain information on the safety and efficacy of the product with the goal that such information could ultimately be used to support a marketing approval decision, the primary purpose of access to investigational product through expanded access programs is to diagnose, monitor, or treat patients.

The following is a summary of Insmed's expanded access policy for the United States. Please note that this policy does not serve as a guarantee of access to any Insmed investigational product by any individual patient. This policy is subject to change from time to time, and Insmed reserves the right to terminate or modify it at any time.

Procedures for Submitting Requests to Insmed

Insmed will consider expanded access requests from treating physicians subject to US laws and regulations. All requests should be submitted via e-mail to compassionateuse@insmed.com. Receipt of a request will be acknowledged within 5 business days.

Process for Review of Requests

Insmed is committed to a fair and impartial evaluation of each request for access to investigational products. Therefore, all decisions are based solely on clinical circumstances and are guided by the criteria outlined below. Patients will be referred to ongoing clinical trials as the primary way to access investigational products.

Review Criteria

Insmed may provide individual patients access to an investigational product outside of a clinical trial only when, in the sole judgment of Insmed, all of the following criteria are met:

1. The request must come from a U.S.-licensed physician, who will take primary responsibility for supervising use of the investigational Insmed product and will comply with all applicable FDA regulatory requirements associated with treatment use of an investigational product.
2. The investigational Insmed product must be part of an active clinical development program that has completed at least Phase 2b.
3. Granting access to an investigational medicine must not interfere with the completion of important clinical trials that could support FDA approval, or otherwise compromise the potential development, of the investigational Insmed product. Additionally, patients must not be eligible (i.e., do not qualify) for ongoing or soon opening clinical trials of the investigational product.
4. The potential benefit to the patient seeking access to the investigational Insmed product must always be considered to outweigh the collective potential risks to the patient of offering the investigational product, including the outcome of the disease itself.
5. As applicable, there is sufficient clinical data to identify an appropriate dose (amount and frequency of the medicine given).
6. The investigational product will only be provided if adequate supply exists to support both the ongoing clinical trials and approved expanded access, until and if product becomes commercially available.
7. The arrangement complies with applicable regulatory and legal requirements.

Information regarding Investigational Products

Insmed currently has one investigational product, liposomal amikacin for inhalation (LAI) that has completed Phase 2b and may be available for expanded access, subject to the policy. More information about the

clinical trials for LAI can be found here. Investigational products have not yet received regulatory approval from the FDA; therefore, their potential risks and benefits are not yet established. Physicians and patients should consider all possible benefits and risks when seeking expanded access to an unapproved product.