VERMONT MEDICAL SOCIETY

Date: February 8, 2017

To: Senate Health & Welfare Committee

From: Jessa Barnard, VMS Vice President for Policy

Re: S. 37, Access to Treatment for Patients with a Terminal Illness

The Vermont Medical Society appreciates the invitation to testify before you today regarding S. 37, Access to Treatment for Patients with a Terminal Illness. The VMS is the state's largest physician membership organization, representing over 2000 physicians, medical residents and medical students across specialties and geographic and practice location.

VMS does not have an overarching position on the passage of S.37. Our physicians have deep empathy for patients who have terminal illnesses and have exhausted other approved therapies. We understand the desire behind the bill to provide those patients with the ability to try other options.

On the other hand, the bill does raise some regulatory considerations the Committee may want to consider:

Equity and access issues: The bill does not require manufacturers to provide treatments to patients nor does it require insurance companies to reimburse for treatments. Therefore, there may be limited access, largely based on economic means.

Safety and efficacy considerations: While patients who take advantage of the bill must be diagnosed with a terminal illness, taking unproven products may still cause serious side effects during that individual's lifetime. In addition, there will be no study of a product's effectiveness before the patient accesses the medication.

The FDA provides a helpful overview of their approval process here: http://www.fda.gov/Drugs/ResourcesForYou/Consumers/ucm143534.htm Phase I trials typically involve 20-80 patients and evaluate the drug's most frequent side effects. However, safety and side effects of a medication continue to be studied during Phase II trials, and efficacy of a medication begins to be established. Safety, efficacy and side effects continue to be studied in Phase III. Phase II has the lowest success rate of the FDA development phases.

The FDA has established and Expanded Access or Compassionate Use pathway whereby an individual may access investigatory drugs if (1) the individual has a serious or immediately life-threatening condition and there is no satisfactory alternative therapy; (2) the probable risk to the person from the product is not greater than the risk from the disease or condition and the potential patient benefit justifies the potential risk; and (3) providing the investigational drug will not interfere with the clinical trial's process or otherwise compromise the product's development. The physician must also request approval from an Institutional Review Board (IRB). You can find a description and application information here:

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http://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/default.htm

In FY15, 1416 applications for Expanded Access were approved and 14 were denied. VMS's concern with circumventing the FDA process include not having scientific or ethical review by an IRB and potentially weakening the clinical trial process by diverting resources, medication and/or available patients.

Informed consent: The bill requires physicians to provide to patients a "realistic description of the most likely outcome" of taking the product based on the "physician's knowledge of the proposed treatment." While the bill does limit physician liability as long as complying in good faith with the requirements, it is still unrealistic to expect that a physician can provide a "realistic…most likely outcome" when there is no efficacy data, and limited safety data, on a particular product. In contrast, with the FDA Expanded Access program, the FDA is the entity to weigh potentially risks versus benefits and the physician also receives the guidance of the IRB process.

Thank you for considering the views of the VMS and please let us know if we can be of further assistance as you move forward.