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Dear Senator Ayer, Chair, and members of the Senate Committee on Health & Welfare,

Thank you for the opportunity to testify in opposition to Senate Bill S.37, the "Right to Try Act," and I apologize that I am not appearing in person, as I had intended.

Let me begin by saying everyone engaged in discussion of this bill are seeking to help terminally ill patients and their loved ones. My opposition to this bill is not grounded in any concerns about the beneficent intention of this bill but rather on my sincere conviction that this bill will not help patients and may lead to unintended harm.

S.37 has numerous provisions that I fear would lead to harm for the people of Vermont. Before I enumerate these, I wish to point out that there is no need for this, or any, Right to Try bill in order for patients to seek access to experimental medical products (including drugs, devices, vaccines, and biologics) that are not approved for sale and use by the US Food and Drug Administration (FDA).

The FDA permits all seriously ill patients who have no other treatment options to seek what is called "expanded access" to unapproved medical products. The intention of Right to Try is to create an alternative pathway for patients to seek access to experimental products, one that circumvents the FDA.

If we are creating an alternative to the FDA's expanded access program, we ought to know what it is. It is a program that allows all seriously ill patients who have no other treatment options the chance to use experimental medical products without having to enroll in a clinical trial. Please note I said "all serious ill patients," not "terminally ill patients." Thus, the existing program is more generous than what right to try would offer.

The FDA's expanded access program allows over 99% of requests to try experimental medicines to proceed. While there is no published data concerning how quickly the FDA responds to requests, in my experience and that of my colleagues, it is quite rapid. There are stories of patients waiting weeks, months, or even longer to hear from the FDA. This is not my experience and, I believe, is not characteristic of today's FDA: indeed, the FDA has special procedures for handling emergency requests.

Not only does the FDA permit use of an experimental agent on patients with no other options over 99% of the time, a recent audit of 150 randomly-selected cases found that 11% of the time the FDA's review resulted in alteration of the proposed treatment plan. For example, after reviewing a proposal, the FDA might suggest to the treating physician that she change the dose to be used. This is a valuable service intended to protect these seriously ill patients, and it ought

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not be abandoned in favor of right to try policies which offer no such safeguard.

The FDA is not without flaws, but with regard to access to pre-approval treatments, the agency is hard at work and improving rapidly. For instance, after hearing some physicians complain that it could take up to 100 hours to complete the required paperwork, the FDA realized that their instructions were unclear, leading physicians to fill out a large document when they were only supposed to complete a handful of questions within the document. In response, the agency created a new form, released in 2016, containing only the required questions, leading physicians to now be able to complete the paperwork in less than an hour. And after learning that patients and their physicians had a hard time contacting the appropriate entity at a pharmaceutical company in order to ask for access to an experimental drug, the FDA tasked its Regan-Udall Foundation to work with a variety of stakeholders to create a "navigator" system to assist with this.

I hope I have convinced you that S.37 is unnecessary because the FDA is actively implementing patient-friendly best practices recommended by its critics - people such as me. But in case I have not, let me discuss some problems with S.37:

1. It is morally reprehensible to threaten terminally ill individuals with loss of their hospice care. In the US, terminal patients can legally seek access to investigational products outside of clinical trials (indeed, the entire point of right to try is to make this quest faster and easier), so there is no basis for subjecting these patients to harsh consequences for their choice to try to live longer. Given the scant likelihood of the experimental treatment working, there is no reason to discharge the patient from hospice at the start of treatment. Rather, if the patient benefits from the treatment and is deemed ineligible for hospice, then the person's hospice eligibility ought to be withdrawn at that time.
2. The prospect of imminent death may skew one's ability to weigh the risks and benefits associated with potential therapies. Thus, for those who are terminally ill and seeking access to an experimental product, truly informed consent is challenging. Informed consent will be all the more difficult because S.37 only requires the experimental medical product to have gone through Phase 1 of a clinical trial. With only limited data on safety (in many cases Phase 1 testing is done on healthy adult volunteers, not sick patients) and virtually none on efficacy, patients and their physicians are likely to be guided more by hope than data.
3. As patients already have access to investigational drugs through the FDA expanded access pathway, S.37 does very little for patients; rather, it is primarily a tool for limiting liability. Patient-centric provisions that are found in other right to try bills around the country include statements that the experimental medical product must be free from cost (Texas); that physicians must refer for treatment patients who they suspect are impaired by a psychological or psychiatric disorder (Oregon); that consent forms for treatments obtained via the right to try pathway must be as comprehensive as consent forms used in clinical trials of the experimental product (Missouri and Mississippi); that patients must be examined by a second, independent physician to confirm the terminal diagnosis (Virginia). These are just a few of the provisions that an ideal policy would have.
4. S.37, like all state right to try bills, may be preempted by federal regulations under the Supremacy Clause of the US Constitution. This will not be known for sure unless there is court challenge or unless the federal government intervenes to make right to try federal

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law. In the meantime, the uncertainty about which law to follow has led healthcare systems in states with right to try laws to be hesitant about allowing patients to try experimental agents under either the expanded access or right to try pathway.

Thank you for your efforts on behalf of your constituents, particularly those who are sick and in need of assistance. I hope I have convinced you that there is no need for a right to try bill and, indeed, it will cause more problems than benefits. If I have failed in this quest, I welcome the opportunity to work with you to try to make the bill the best that it can be and to make it truly patient-centric in all its provisions.

Sincerely,

Alison Bateman-House

Co-Chair, NYUSOM Working Group on Compassionate Use & Pre-Approval Access (http://www.med.nyu.edu/pophealth/divisions/medical-ethics/compassionate-use)

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