

Before The Public Health Committee

February 24, 2016

**Written Testimony of CURE (Connecticut United for Research Excellence)
RE: H.B. No. 5270 (RAISED)
AN ACT CONCERNING THE RIGHT TO TRY EXPERIMENTAL DRUGS**

Chairman Gerratana, Chairman Ritter, Members of the Public Health Committee:

My name is Anthony Sabatelli. I am a partner with the intellectual property law firm of Dilworth IP in Trumbull. I am submitting this written testimony on behalf of CURE, Connecticut United for Research Excellence, where I chair CURE's Government Affairs Committee. CURE serves the bioscience cluster of Connecticut.

CURE recommends that the Public Health Committee not move forward with H.B. No. 5270 (RAISED) AN ACT CONCERNING THE RIGHT TO TRY EXPERIMENTAL DRUGS. The Bill is not in the best interest of critically ill patients, the State's thriving biotechnology and pharmaceutical industries, nor the public at large. While this legislation is well-intended, it neglects to consider serious challenges associated with providing access to experimental medicines.

This legislation has the potential to undermine the clinical trial process and may jeopardize current on-going clinical trials.

Right to try legislation can hurt the integrity of the clinical trial process by hindering enrollment in clinical trials. The best way for patients to access new medicines is for companies to complete their clinical trials and get their drugs approved. Drug and biologic manufacturing is a costly and complex undertaking. Companies often have very limited manufacturing capacity early in the drug development process, and thus may be able only to produce enough drug supply for their clinical trials and not enough for additional expanded access. Furthermore, there is the very real risk that an adverse event in a patient using an experimental drug outside the clinical trial can jeopardize or disrupt the drug development process, potentially resulting in the loss of the opportunity for other patients to have access to an approved safe and effective medicine.

The FDA drug approval process is rigorous and scientifically based for a reason.

The FDA drug approval process is rigorous to ensure biopharmaceutical products are safe and effective for patient use. The normal FDA approval process, including clinical trials that show the benefits of a drug outweighing its risks, is the best way to establish the safety and efficacy of a drug or biologic, and should remain the primary pathway for these products to be made available to patients.

Many companies have limited resources and would be negatively impacted by such legislation.

Many biopharmaceutical companies, particularly small biopharmaceutical companies, would be limited in their ability to provide expanded access because of resource and product supply constraints, and would not have the experience or personnel to manage an expanded access program. Furthermore, a company's priority is the completion of their clinical trials so that they can receive FDA approval and make their products available for all patients who need them.

“Right to Try” legislation would create false illusions.

“Right to Try” legislation may give patients a false sense of hope. For example, a company may be unable to provide the experimental medicine, regardless of the existence of a “Right to Try” law. Irrespective of the provisions of such a state law, federal law still requires FDA permission before providing an unapproved drug to a patient. Also, medicines in early stages of development have yet to be proven safe and effective for patient use.

Many companies already have “expanded access” programs in place, which address many of the concerns raised by advocates of “Right to Try” legislation.

Formal legislation is not necessary because some companies already have “expanded access” programs in place so that patients in need, who do not qualify for the company's clinical trials, but who may benefit from a particular medicine, can access that medicine prior to FDA approval.

A State Resolution would be an acceptable alternative.

If it is absolutely necessary for the Committee to act on this bill, it is proposed that the Committee instead consider a State Resolution as a more worthwhile, acceptable alternative. Such a Resolution would have the State of Connecticut call on the FDA to work with Congress, patient organizations, physicians, and the biopharmaceutical industry under all applicable federal laws to improve efficiency and timeliness of the approval process for potentially lifesaving drugs in critically ill patients, without compromising patient safety. Such a Resolution can realistically deal with the challenge of “expanded access” in a well-balanced manner, and would represent a fair compromise for all parties involved. An example of such a Resolution is attached as Exhibit A.

Summary

For the foregoing reasons, H.B. No. 5270 is not in the best interest of critically ill patients, the State's thriving biotechnology and pharmaceutical industries, and the public at large. While this legislation is well-intended, it neglects to consider serious challenges associated with providing access to experimental medicines.

Thank you.

Anthony D. Sabatelli, PhD, JD
Chair, Government Affairs Committee
CURE (Connecticut United for Research Excellence)

Exhibit A Attached

Exhibit A

Example of a State Resolution

Resolution on Patient Access to Investigational Drugs

WHEREAS, innovations in the development of lifesaving drugs, biological products, and medical devices have brought new hope to patients with cancer and other life-threatening diseases.

WHEREAS it is in patients' best interest that innovative medicines be approved by FDA as safe and effective and available for all patients who need them, as efficiently as possible.

WHEREAS, there is a critical need to review, improve, and accelerate current FDA processes so investigational drugs can be approved more efficiently and be available to more patients in need without compromising patient safety.

WHEREAS, FDA's "Expanded Access to Investigational Drugs for Treatment Use" rule, promulgated in 1987 and finalized in 2009, provides criteria for authorizing qualified patients to be eligible for treatment programs using investigational and unapproved drugs.

WHEREAS, the life science industry works closely with the FDA to develop "expanded access" programs so patients in need, who do not qualify for the company's clinical trials but who may benefit from a particular medicine, may in some cases access investigational medicines prior to FDA approval.

WHEREAS, safety must be carefully considered not only by the patient, caregiver, and physician, but also the manufacturer prior to giving any patient access to a drug not yet approved by the FDA.

WHEREAS, equity in distribution must be considered so that patients in need have an equitable opportunity to access an investigational drug that may be in limited supply.

WHEREAS, there is a very real risk that an adverse event in a patient using an investigational drug outside a clinical trial may put at risk or disrupt the development of the drug, potentially jeopardizing eventual access by other patients to an approved safe and effective lifesaving medicine.

WHEREAS, irrespective of state laws, federal law regulates the distribution of unapproved drugs.

WHEREAS, the Prescription Drug User Fee Act (PDUFA) negotiations, under which manufacturers agree to pay fees to FDA to help fund FDA operations, including independent scientific reviews for product approvals, provide industry, patients, physicians, and other stakeholders an opportunity to evaluate and recommend refinements to current FDA review processes.

WHEREAS, the U.S. Congress 21st Century Cures initiative is reviewing steps federal agencies can take to accelerate the pace of cures in America, and FDA activities, including the drug review process and accelerating access to lifesaving drugs by patients who need them, are part of this process.

WHEREAS, navigating the expanded access process can be complex for patients and physicians.

NOW, THEREFORE, BE IT RESOLVED, that the State of XXXXX call on FDA to work with Congress, patient organizations, physicians, and the biopharmaceutical industry under all applicable federal laws to improve the efficiency and timeliness of the approval process for potentially lifesaving drugs in terminal patients, without compromising patient safety.

BE IT FURTHER RESOLVED, that the State of XXXXX call on FDA to simplify its processes for obtaining access to investigational medicines; improve its communication among patients, their physicians, and manufacturers; and educate the public more effectively about access to investigational medicines.