Guidance for Research Teams on "Right to Try" Act		
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1. **PURPOSE:** To provide guidance to the University of Miami research community on the State of Florida's "Right to Try Act."

2. **REVISIONS FROM PREVIOUS VERSION:** NA

3. **UNIVERSITYOF MIAMI POSITION AND GUIDANCE FOR EMPLOYEES**
   a. UM receives federal funding for research and holds a Federal Wide Assurance (FWA) and therefore considers that Federal law supersedes State law when they are in conflict.
   b. UM requires that all human research subjects receive or use only those study medications, biologics, or devices that are approved or permitted by the FDA for use.
   c. UM strongly encourages the use of the currently updated expanded use pathway initiated by the FDA for patients who seek unapproved medications for terminally ill conditions.
   d. Therefore, patients who invoke the Right to Try Act should be gently informed that (i) the Act does not require institutions to accede to requests for unapproved drugs, (ii) UM follows Federal research regulations, and (iii) efforts to obtain unapproved drugs should be made to the FDA under its emergency use expanded access program.

4. **INTENT OF THE LAW**
   a. *What is the Right to Try Act?* The "Right to Try Act" (FS 499.0295) allows investigational drugs, biological products or devices that (i) have been through "phase 1" of a clinical trial, and (ii) have not been approved for general use by the U.S. Food and Drug Administration (FDA), but remain under an FDA approved clinical trial, to be used in patients with terminal conditions. The law provides liability protections to physicians and drug manufacturers.
   b. *Which patients are eligible?* Approved in 2015, the law stipulates four eligibility criteria. An eligible patient:
      i. Has a terminal condition that is attested to by the patient's physician and confirmed by a second, independent evaluation by a board-certified physician in an appropriate specialty for that condition;
      ii. Has considered all other treatment options for the terminal condition approved by the FDA;
      iii. Has given written informed consent for the use of an investigational drug, biological product, or device;
      iv. Has documentation from his or her treating physician that the patient meets the requirements of the Act.

c. *Why was this law approved?* The Act is based upon a Model Law developed by the Goldwater Institute, a libertarian think tank. The driving principle behind the law is that terminally ill patients have nothing to lose by trying unproven treatments, and that they ought to gain access to those treatments without undue encumbrances. The Florida legislative staff analysis prepared in support of the Act, points to putative delays in the FDA's approval process for its allegedly burdensome, time-consuming, and confusing emergency use expanded access (sometimes called "compassionate use") program. , In fact, in the preceding year only three of more than 1000 emergency-use requests to the FDA had been denied. The analysis also cites a lawsuit brought against the FDA by an advocacy organization formed by a father whose 19-year-old daughter died from head and neck cancer after she failed to meet the inclusion criteria for clinical trials related to her condition. A U.S. appeals court found that terminally ill patients did not have a constitutional right to receive unapproved experimental drugs.

d. *Problems with the Law:*

i. The U.S. Constitution's Supremacy Clause states that federal law is the "the supreme Law of the Land … any Thing in the Constitution or Laws of any state to the Contrary notwithstanding." Therefore, state laws cannot permit manufacturers to provide patients with access to unapproved drugs when the FDCA mandates that "no person shall introduce or deliver for introduction into interstate commerce any new drug," unless FDA has approved an application for such product or otherwise authorized use of the investigational drug through a clinical trial or an expanded access program. To the extent that a Right to Try (RTT) law provides a right to direct access to an investigational product without FDA approval or oversight, that law would be "preempted" by federal law, meaning that the federal laws supersede and effectively nullify their state RTT counterparts. Although several federal courts have concluded that FDA's comprehensive regulatory regime governing the manufacture, approval, labeling, and distribution of drug products preempts state laws designed to legislate in this area, no court challenge to a state RTT law has been decided yet. If challenged, however, it is likely that RTT laws will be treated similarly.

ii. RTT laws creates a false hope and therapeutic misconception. The phase 1 studies are quite small, and the results not generalizable to a larger population with no data on efficacy. Of all drugs that pass phase 1 trial, only about 20% continue on to phase 3. Of those that fail, half fail due to lack of efficacy, about 30% fail for strategic reasons (e.g., they are not substantially different from existing treatments) and close to 20% fail due to safety

problems. Although protagonists of RTT laws say that terminally ill patients have nothing to lose by trying an unapproved drug, there is a significant chance that the drug will cause mortality and morbidity without any added benefit.

ii. The potential financial burden on the patient is yet another concern. While manufacturers may provide the experimental drugs for free, they are not required to do so. Similarly, insurers could agree to do the same, but are also not required to do so. This leaves the patient or their estate (but not heirs) to foot the bill if the patient dies while being treated pursuant to the Act. It therefore allows disparate treatment of patients who cannot afford the costs of participation.

iii. There is no requirement that an IRB or other human subjects protection group should evaluate the risks and potential benefits to this vulnerable population.

5. **RESPONSIBILITIES:** UM faculty and research staff.

6. BIBLIOGRAPHY

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