Fact Sheet and Recommendations from the NYU Langone Health Working Group on Compassionate Use and Pre-Approval Access (CUPA)  
[Updated March 6, 2018]

**Key Question 1:**  
Do patients need “Right to Try” laws to get access to experimental drugs?  

**Key Answer 1:**  
No. Patients already get experimental drugs through the FDA expanded access program.

It's been successful for decades: 99+% of requests for experimental drugs are approved.

- FDA reviewers have access to confidential drug data that doctors/patients don't.  
- FDA can amend treatment plans to help ensure that they give patients the best possible chance.  
- FDA monitors adverse events to help protect the safety of future patients.

**Right to Try (RTT) is based on 2 myths:**

1. The FDA’s “expanded access” program is slow and ineffective.
   - FDA approves 99% of requests in 4 days on average — in hours for emergency requests.
   - The FDA form takes less than an hour for physicians to fill out.
   - FDA speeded up the process by requiring a single IRB reviewer, not a full board, to approve requests.
   - Concerns that adverse events might derail drug development are unfounded: in recent years, FDA has halted clinical trials due to adverse outcomes twice — and trials resumed quickly after adjustments.

2. RTT would let patients get experimental drugs faster by cutting out the FDA.
   - Drug companies, not the FDA, decide whether to provide drugs. They have many reasons to say no.
   - No patients have gotten access to investigational drugs via RTT that they did not have access to already via FDA’s expanded access program.
   - RTT would create an alternative to the FDA’s proven expanded access program. A second path would cause confusion — while the new RTT path would offer no additional benefits to patients.

**Key Question 2:**  
How can legislators help patients who want to try experimental drugs?

**Key Answer 2:**  
Work to ensure that patients and doctors understand how the FDA program works and incentivize drug companies to provide access to their products.

The FDA’s expanded access program works — but it can be improved.

- Preserve FDA’s role in expanded access to protect patients from charlatans and ensure treatment safety.
- Require FDA to devote more resources and autonomy to its Office of Health and Constituent Affairs and the Division of Drug Information, both of which help physicians and patients with expanded access.
- Require FDA to issue clear, specific information about what — if any — consequences companies will face if there’s an unexpected or severe adverse event related to their product. There are many fears to be allayed.
- Strengthen FDA’s power to require physicians to report all unexpected/serious adverse events in expanded access. This is essential to ensure the safety of future patients.
- Require FDA to develop/implement a plan to address fears and misperceptions about expanded access for all stakeholders: industry, advocacy organizations, patients, doctors, and the public.
- Facilitate FDA partnering with industry, academia, and healthcare and advocacy organizations to better inform all stakeholders on the expanded access process, including relevant reporting requirements.
- Encourage FDA to work with industry to see if real world evidence from expanded access can serve to advance drug development and supplement clinical trial data.
Clarify authority over experimental drugs.

- 38 state RTT laws have created confusion for healthcare professionals and organizations. Congress must clarify that FDA has authority over as of yet unapproved drugs.
- Many state RTT laws have patient hostile provisions: denying coverage to terminally ill patients for hospice or home health care, or denying insurance altogether. Congress must ensure these cannot be used against patients.

Make expanded access more appealing to big and small pharma and biotech companies.

- Companies worry that providing experimental drugs could threaten their drug development timelines due to FDA or Wall Street concerns. Legislators and industry must partner to mitigate such risks.
- Some companies, particularly small ones, may be unable to provide experimental drugs, even if they wish to, due to expense. Legislators and industry must work together to make this financially feasible.

Clinical trials are the safest, most common, and most valuable way for patients to get access to experimental drugs. Legislators must promote awareness of, access to, and equity in trials.

- FDA is holding a meeting in April to investigate allowing more patients to be considered eligible for clinical trials. This is an important first step, but more needs to be done.
- Geographic variability in access to clinical trials must be addressed to make participation viable for all Americans.

Members of CUPA are available to answer questions or discuss details about these proposals or other aspects of expanded access. Visit our website for contact info.

What Patient Groups, Medical Organizations, Industry, and Others Say About Right to Try:

“No ethical company that I know of would release an experimental medicine outside of the FDA’s regulatory process.” —Kenneth Moch, president/CEO of Cognition Therapeutics (House Energy & Commerce Committee hearing on patient access to investigational drugs, 10/3/17)

“There’s a perception that RTT legislation “might provide more of an incentive and an opportunity…. I think the biggest obstacle to offering drugs through expanded access is the supply constraints.” —FDA commissioner Scott Gottlieb (House Energy & Commerce testimony)

“We are committed to helping patients with serious illnesses and their families request access to our investigational medicines. We support these requests through our established review and evaluation processes, which includes independent review by the FDA to assure full consideration of available safety data of which the FDA may be uniquely aware.” —Janssen Pharmaceuticals

“[T]he Right to Try bills currently under consideration in the House do not effectuate policy changes that would afford our patients greater access to promising investigational therapies. Instead, these bills would likely do more harm than good.” —Letter to House leadership from 38 patient and provider groups (2/6/18)

“This legislation sells vulnerable patients and families false hope at the expense of weakening the FDA’s critical role in making sure that all Americans can have confidence in the safety and effectiveness of our medical products.” —Open letter to House Energy & Commerce committee signed by 336 academics, physicians, patient advocates, and others (2/5/18)

“Any legislation should protect the integrity of clinical trials and the FDA oversight of expanded access to maintain the best interests of patients.” —Pharmaceutical Research and Manufacturers of America (PhRMA) spokesperson (RAPS.org, 2/17/17)

“ACRO’s [the Association of Clinical Research Organizations] view is that proposed Right-to-Try legislation is deeply flawed because it: contains insufficient patient protections; compromises the clinical trial process; and undermines the FDA’s authority to assess safety and effectiveness.” —ACRO policy statement (5/22/17)