KEY QUESTION 1: Will a federal "Right to Try" law help patients get access to experimental drugs?

KEY ANSWER 1: No.

"Right to Try" (RTT) legislation is based on 2 myths.

The FDA's "expanded access" program is slow and ineffective.	▪ FDA approves 99+% of "compassionate use" requests. ▪ FDA turns around emergency requests in 1 day or less; non-emergency requests in 4 days, on average. ▪ FDA form can be completed in less than 1 hour.
RTT legislation would let patients get experimental drugs faster by cutting out the FDA.	▪ Only drug companies can decide whether or not to provide their drugs to patients. Companies have many reasons to say no. ▪ ***No patients have gotten drugs via RTT that they did not have access to via FDA's expanded access program.*** ▪ RTT would create an alternative to the FDA's proven expanded access program. Having two paths would cause confusion.

FDA involvement helps, rather than harms, patients

- FDA's objectivity, expertise, and knowledge of all the various drugs in development can help patients determine their best options.
- FDA's review and its ability to require changes to treatment plans help physicians design treatment plans most likely to help, and not hurt, patients.
- RTT could place drugs for which there is very little safety or efficacy data in the hands of doctors who are not familiar with drug development or experimental drugs. Thus, despite their best intentions, doctors may have insufficient know-how to help—and not hurt—their patients.
- The FDA's requirement that any severe or unexpected outcomes be reported is designed to protect future patients.

"ASCO is concerned that existing and proposed RTT laws do not adequately protect patients, do little to facilitate patient access to such therapies, and potentially interfere with recent reforms that are already streamlining patients' access to investigational agents." *—American Society of Clinical Oncology position paper, April 2017*

"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)*

KEY QUESTION 2: How can legislators help?

KEY ANSWER 2: To help patients get access to experimental drugs, you need to ensure that they, and their doctors, understand how to request access. Then, you need to make such access both appealing and practical for drug companies.

1. **Strengthen FDA's expanded access program. It works, but it can be improved.**

- **Preserve** FDA's role in reviewing expanded access proposals. This protects patients from charlatans and improves the proposals by adding FDA's expert input.

- **Empowe**r FDA's Office of Health and Constituent Affairs and the Division of Drug Information, which help physicians and patients with the expanded access process. They need more resources, autonomy, and/or personnel to function optimally.

- **Require** FDA to expand its current Guidance Document on Expanded Access to include clear, specific information about what, if any, the consequences will be of an unexpected or severe adverse event occurring in the context of expanded access.

- **Strengthen** the agency's ability to require physicians to report to it all serious and unexpected adverse events that happen in the context of expanded access.

- **Allow** FDA to require physicians to report to it additional data (such as outcomes) from expanded access attempts.

- **Require** FDA to prioritize the Reagan-Udall Foundation's in-development expanded access navigator, which will help physicians and patients who seek access to experimental drugs. It must be easy-to-use, up-to-date, accurate, and designed for both physician and patient users. It must be appropriately designed and built from the outset, and it must be properly maintained over time, if it is to help patients.

- **Use** PDUFA to require FDA to develop/implement an outreach plan to address fears and misperceptions about expanded access for all stakeholders: industry, advocacy organizations, patients, and the public.

- **Use** PDUFA to require FDA to better educate healthcare professionals and researchers on the expanded access process and the relevant reporting requirements. FDA should partner with industry, academia, and healthcare and advocacy organizations on educational endeavors and participate in relevant conferences.

- **Encourage** FDA to work with industry to investigate how data from expanded access can serve to advance drug development and supplement clinical trial data.

2. Clarify authority over experimental drugs.

- Unintentionally—but not unforeseeably—the 34 state RTT laws have created confusion for healthcare professionals and organizations about whether to follow state or federal rules governing access to experimental drugs outside of clinical trials.

- Congress must clarify that, according to precedent, FDA has authority over non-approved drugs.

3. Explore, with the pharmaceutical and biotech industries, ways to make expanded access a more appealing prospect to companies large and small.

- Companies are concerned that providing experimental drugs for expanded access may threaten their drug development timeline due to either FDA or Wall Street concerns. Legislators and industry must partner to mitigate risks to drug development and companies.

- Some companies, particularly small ones, may be unable to provide experimental drugs to patients even if they wish to. They may lack the human and financial resources to 1) create a sufficient supply of drug and/or 2) handle the requirements of an expanded access program, such as monitoring the proper use of the drugs and overseeing the required reporting. Legislators and industry must work together to make these activities financially feasible for companies.

- Companies are concerned about possible liability for adverse outcomes resulting from an experimental drug. Legislators and industry must create solutions for this concern. We suggest considering the possibility of immunity ONLY when 3 conditions are met: the FDA expanded access pathway is used; sellers and prescribers act with a reasonable belief that the treatment is in the patient's best interests; the patient or his legal representative provides informed consent.

4. Commission an expert group to explore whether institutional review board (IRB) oversight is needed for single-patient expanded access protocols in which the patient has no other possible therapeutic options.

- It may be that single-patient, therapeutically intended uses of experimental drugs do not need to be reviewed by IRBs. An expert group should examine this and make recommendations.

5. Utilize legislative and other means to promote awareness of, access to, and equity in clinical trials - the safest, most valuable, and most common way for patients to get access to an experimental drug.

For more information about these proposals, please contact the NYU School of Medicine Working Group on Compassionate Use and Pre-Approval Access