Statement of Diana Zuckerman, PhD, President

On behalf of the National Center for Health Research

Regarding Right to Try Legislation

House Energy and Commerce Subcommittee on Health

October, 3, 2017

Chairman Burgess, Ranking Member Pallone, and distinguished Subcommittee Members: Thank you for the opportunity to submit hearing testimony for the record. The National Center for Health Research is a non-profit organization which analyzes medical and scientific data and produces original health-related research to inform patients, the general public, and policymakers. We advocate for patients and consumers to have access to safe, effective, and affordable drugs and medical devices. We accept no funding from the pharmaceutical or medical device industries.

We all agree that terminally ill patients should receive the best medical treatment as quickly as possible. Some terminally ill patients are willing to take big risks to have a chance to live longer. Unfortunately, many of us know desperate patients whose efforts to "try anything" made their remaining days miserable and left their families even more devastated. What can and should Congress do to make sure that desperate patients won't be exploited, or suffer even more painful deaths as a result of legislation? That is the key question before you today.

It is essential that all Members of Congress understand what the various Right to Try bills would do. A key issue is to understand what it means to give access to any experimental treatment that has been in **at least one clinical trial**. The earliest clinical trials (known as **Phase I) often don't include even one patient**. Instead Phase I trials can include "healthy volunteers" that are much less likely to be harmed by an experimental drug or device than a terminally ill patient would.

In addition, these first (Phase I) clinical trials study **very small numbers of people**, and **do not study whether or not a product works**. They are designed to determine the immediate risks on just a few healthy volunteers or patients. Since so few people are studied, even **if a treatment is immediately and painfully fatal to 10% of patients, for example, these first clinical trials probably would not be able to provide that crucial information.**

The Right to Try bill introduced by Representatives Griffith and Brat do not even require that a first clinical trial be completed – it can have just started. In other words, there is no way that a patient could be warned about any terrible risks of those treatments.

The Johnson Right to Try bill (S 204) requires that a Phase I clinical trial be completed. That is an improvement over the Griffith and Brat bill, but it is important to know that 85% of drugs that successfully complete Phase I clinical trials are **later found to be unsafe or ineffective and are therefore never approved by the FDA. So neither version would help most patients.**

In contrast, the FDA's current expanded Access Program requires at least some evidence that an experimental treatment might possibly be helpful. That's not a very restrictive safeguard, but it is helps protect many patients. The FDA routinely utilizes what the agency terms "compassionate waivers" for very ill patients when doctors request them, and FDA grants such requests 99% of the time.

Another important issue for Congress to consider is whether these bills would exploit patients financially. The experimental drugs provided through the current FDA Expanded Access program are provided for free most of the time, or "at cost." The same is true for clinical trials. The Johnson bill also protects patients from financial exploitation by limiting what experimental treatments can cost. The Griffith and Brat bill allows **companies to charge whatever they want** to dying patients desperate for access to any experimental drug or device – even one that has absolutely no evidence that it is either safe or effective. **That means that desperate patients could be required to pay exorbitant fees for the "Right to Try" to be treated like guinea pigs. Many families would feel tremendous guilt if they could not afford to do so.**

FDA's compassionate use program could be improved, and improvements are already underway thanks to the Navigator program that the FDA has recently initiated with the Reagan Udall Foundation. Other access issues are inherent in the situation where patients want drugs that are not yet being manufactured in large numbers or when the companies are reluctant to provide drugs that they fear will be harmful to patients who are too ill to benefit. The GAO's July 2017 report was generally supportive, with a few recommendations for improvement. And, GAO pointed out that most experimental drugs distributed under Expanded Access eventually obtain FDA approval. In other words, the program is doing what was intended – giving patients earlier (usually free) access to experimental drugs that will eventually be proven safe and effective.

In addition to harming individual patients, making unsafe treatments available for sale harms our entire drug development enterprise, by eliminating the incentive for patients to participate in clinical trials that would help millions of patients in the future. If HR 1020 was to become law, then it is likely that the richest patients will buy access to experimental treatments and only the middle-class and low-income patients will participate in clinical trials. Reputable companies would continue to study new drugs and devices in clinical trials, but progress would be slowed because of difficulty attracting enough patients to participate in clinical trials. Meanwhile, scam artists and fly-by-night companies would be motivated to make as much money as possible on dangerous or worthless experimental drugs for as long as they are available, and HR 1020 and would make it impossible to gather information about how dangerous their products are.

Such problems have long been documented regarding unproven treatments sold at outrageously high prices in Mexico and elsewhere, where some patients have been irreparably harmed or killed because they sought unproven treatments that were marketed dishonestly. Indeed, tragedies arising from the "right to try" unregulated medical sales of the 19th Century and early 20th Century were the reason FDA was created, to protect patients and consumers.

To improve Right to Try legislation, Congress should:

1. Ensure that experimental treatments cannot be sold at a profit by companies or medical professionals;
2. Ensure that all experimental treatments have been proven safe in completed Phase I or Phase II trials conducted on a reasonable number of patients (not healthy volunteers);
3. All experimental drugs and devices available through RTT should be studied as part of FDA's regulatory process;
4. Information about harmful side effects and adverse events should be required to be reported to the FDA by the physicians.

We strongly urge this Committee to reject the Right To Try legislation that is currently under consideration, because it would undermine the successful FDA compassionate waiver program already in place to enable patients to have access to experimental drugs for free or at cost.

Thank you for the opportunity to present our views.