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Committee on Energy and Commerce

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Chairman Walden, Ranking Member Pallone and members of this distinguished committee- I am honored to be testifying before you today on this vital topic. I am Ellen Sigal, Chair and Founder of Friends of Cancer Research, a nonprofit advocacy organization ensuring patients receive the best treatments in the fastest and safest way possible.

I founded *Friends* over 20 years ago, driven by the profound loss of my dear sister Gale who was only 40 with a 4 year-old-daughter.

After many years battling cancer Gale had exhausted every option. As metastatic breast cancer raged through her body, defeating all conventional treatments, she faced a final decision: succumb to the disease or wage one last battle with an experimental bone marrow transplant known to kill 20 percent of patients.

Gale chose to fight, opting to use the unproven therapy at a time when institutional review boards and scientific peer review regulated this experimental therapy rather than the Food and Drug Administration (FDA).

Now, three decades later, the FDA has an expanded access policy, also known as “compassionate use,” that seeks to ensure the quality of unproven therapies used by dying patients. In Gale’s case, the side effects of the treatment were swift and violent. Within two days, she was dead. Clinical trials have since demonstrated that the therapy had limited efficacy and a greater risk of lethality than reported at the time.

All of us here today agree on the basic premise that more must be done to save patients lives. We must take steps to allow patients to gain access to clinical trials and to continue to ensure our regulatory system is expediting therapies as safely and quickly as possible. My organization, took huge steps toward this 5 years ago when we worked with many members of this committee to create the breakthrough therapy designation. Breakthrough that has truly changed the way the FDA approves and industry develops life-saving therapies for patients that so desperately need them. Because of this designation there have already been over 60 new drugs approved for serious and life-threatening diseases. This is progress, but I will acknowledge, much more needs to be done.

It is imperative to examine a predominant reason why patients are interested in expanded access to experimental therapies; they are unable to obtain them by enrolling in clinical trials. We at Friends of Cancer Research, working with the American Society of Clinical Oncology (ASCO), have taken steps to address this problem through the work we have done on issues surrounding expanding eligibility criteria – taking down barriers that often times disqualify a patient from participating in a trial to begin with.

While these do not, by any means, solve all of the issues we are discussing here today, it is important to acknowledge the work being done by so many. Still I acknowledge that challenges remain.

The legislation before Congress seeks to grant all terminally ill patients the “Right to Try” experimental therapies once approved alternatives have failed. Although the FDA authorizes 99 percent of compassionate use requests, advocates of Right to Try claim

the process is too slow. The FDA has streamlined the current process so that requests are reviewed within 24 hours; filling out an application now takes less than an hour. Unfortunately, the proposed federal legislation provides almost no protections for patients. Everyone with a late-stage terminal illness like my sister deserves the chance to try an experimental therapy. However, serious changes to today's legislative proposal are needed before this law is safe for patients.

First, provisions for informed consent are essential. Upwards of 90 percent of new drugs never make it to market because they are found to be dangerous or ultimately proven ineffective Right-to-Try laws allow patients to request from companies therapies that have passed a Phase I trial with the FDA. But this is only a preliminary step in which a small group of patients receive the experimental therapy under carefully controlled conditions. The trial is designed to detail obvious toxicities and identify a tolerable range of potentially effective doses before the drug advances to a larger, Phase II trial.

Before the results of that second phase of study, there is no reliable data on whether the therapy works and, even after clearing a Phase I trial, toxicities can be discovered in later phases.

Any legislation that goes forward cannot circumvent the FDA and must be carefully crafted to assure that we don't create a loophole for charlatans and snake oil salesman to take advantage of desperate patients. Profiting off of the sick by offering false hope is reprehensible, but there is a long history of such occurrences to this very day. Without proper protections we risk a market outside of the FDA approval system.

Meanwhile, patients like Gale who receive the therapy may risk a sudden and painful death from unanticipated side effects, as early-phase trials rarely evaluate the risks of extended or repeated administration. Key information about the safety or efficacy of experimental therapies is typically not made public until after drug approval. Provisions insuring informed consent would guarantee that patients requesting expanded access can judge the magnitude of their decision.

Second, the limits of Right to Try must be clear.

Today patients have the right to request an experimental therapy from a sponsor, but the sponsor is under no obligation to provide it. Under the proposed legislation there is no new “right”. A patient still has the right to ask, but a sponsor still has the right to say no. While the term “Right to Try” sounds appealing, this legislation grants no such right.

There are legitimate reasons for a sponsor to say no, including supply shortages, a lack of financial incentives, and concerns that negative “compassionate use” outcomes could be used by the FDA to delay or deny approval (which is protected against by the Right-to-Try legislation). Development roadblocks would stop therapies from reaching patients.

However, the FDA cannot simply ignore expanded access outcomes. Patients petitioning for expanded access deserve accurate information about whether the potential benefits of an experimental treatment outweigh the risks. What are the side effects? What are the chances of success? This highly personal calculus is impossible if drug companies do not monitor and report side effects.

A key component of effective Right to Try legislation is transparency. The Reagan-Udall Foundation for the FDA (RUF), which I am honored to chair, is a nonprofit created by Congress to modernize regulatory science, promote public health and help the US Food and Drug Administration achieve its mission. This year, RUF launched an Expanded Access Navigator to raise awareness among doctors, patients and families when it comes to the compassionate use of experimental therapies. The Navigator is currently being piloted in oncology with the goal of increasing the accessibility of information to patients and providers.

Patients have long been frustrated that they could not find information about expanded access on sponsor websites and didn't know how to make a request of the sponsor. The Navigator is the most comprehensive tool available for patients and physicians to research single-patient expanded access in part because companies list their EA policies, contact information and available therapies in one, consolidated Directory. The 21st Century Cures Act required greater transparency on the part of sponsors, and we have already had 3 dozen companies contribute their information and had 10,000 visitors to this site. In the very near future RUF will expand this program to include rare diseases.

Everyone agrees that dying patients should have access to promising experimental therapies when all available options have been exhausted. Right to Try is a path to achieving that goal; in pursuing it, however, we must not subject patients to false hope or unacceptable side effects. Informed consent and transparency—currently lacking from the proposed legislation—are essential.

With significant adjustments, federal Right-to-Try legislation could help very sick patients easily obtain necessary information to decide what is best for them and improve

processes to access otherwise unavailable drugs. One of these adjustments is that patients must have more immediate access to information about significant adverse events or death of patients that have previously been given the therapy they are seeking access to. This must be done in a much more efficient way. Another adjustment would be the establishment of a designated central institutional review board (IRB) with the predominant focus of coordinating and dealing with expanded access requests.

In its current form, however, Right-to-Try does nothing for patients other than allow them to request a drug they may never receive. This drug may be more likely to hurt them than to help them.

I want to reiterate how important it is to support patient access to unapproved therapies, however, S.204 and HR 878 do not accomplish policy changes that would afford patients greater access to promising investigational therapies. Instead these bills would likely do more harm than good.

I encourage the committee to consider other policy options that would truly improve the ability for patients to safely access unapproved therapies.

Thank You for the committee's efforts on this vital issue to patients.

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ABOUT FRIENDS OF CANCER RESEARCH:

Friends of Cancer Research drives collaboration among partners from every healthcare sector to power advances in science, policy, and regulation that speed life-saving treatments to patients.

During the past 20-plus years, Friends of Cancer Research (*Friends*) has been instrumental in the creation and implementation of policies ensuring patients receive the best treatments in the fastest and safest way possible. We've been successful due to our ability to convene the right people at the right time and put forth revolutionary, yet realistic ideas. We are energized now more than ever to continue this critical work with our trusted partners, creating innovative solutions to overcome barriers standing in the way of conquering cancer.