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Pallone Opening Remarks at Right-to-Try Hearing

Washington, D.C. – *Energy and Commerce Ranking Member Frank Pallone, Jr. (D-NJ) delivered the following opening remarks today at a Subcommittee on Health hearing titled, "Examining Patient Access to Investigational Drugs:"*

Thank you, Mr. Chairman. Today's discussion is of great importance to so many patients and families who are facing diseases with no other treatment options. And when someone has exhausted all of the available treatment options, they will sometimes explore the possibility of trying unproven experimental therapies. It is this desire that has led to calls for federal legislation that would grant patients the "right-to-try" investigational products.

It is understandable that someone suffering from a disease that has no more options would want to try anything that could help them fight their disease. Fortunately, both the Food and Drug Administration (FDA) and Congress have taken action that provides some hope.

Through the FDA's Expanded Access program, patients are able to get access to investigational products. This FDA program approves 99 percent of all requests for investigational drugs or biologics that it receives. Last year FDA received more than 15-hundred requests, and only nine were not approved.

Despite this high approval rate, supporters of "right-to-try" laws have argued that the process is too slow and burdensome. But I have not seen evidence that this is the case. In fact, FDA often grants emergency requests for Expanded Access immediately over the phone, and non-emergency requests are processed in an average of four days.

Despite these quick turnarounds, FDA responded to these criticisms. Last year the agency streamlined the current process even further so that filling out an application now takes less than an hour. FDA also released additional guidance to industry outlining the Expanded Access program's requirements, and addressing common questions related to the different programs and submission process. All of this was done to alleviate any confusion that may have existed in the past. I commend the agency for its commitment to improving Expanded Access and for its responsiveness to the concerns it heard from doctors and patients.

This Committee has also led efforts to facilitate greater access to investigational products for patients who are looking for additional options. Last year, we passed the 21st Century Cures Act which provides greater transparency to Expanded Access programs by requiring manufacturers or distributors of investigational drugs to make publicly available their expanded access policies for the first time.

And then this summer, we passed the FDA Reauthorization Act, which works to improve access to clinical trials for patients. The law does this by requiring FDA to conduct a public meeting on clinical trial criteria, report on barriers to patients participating in clinical trials, and offer potential solutions to include additional populations of patients. The FDA Reauthorization Act also requires FDA to issue additional guidance to manufacturers regarding how clinical trials can be expanded to include broader populations and improve access to treatments for patients who may not qualify for these trials. These are meaningful steps that I believe will help to address some of the criticisms we will hear today.

Our discussion today is also important because I am concerned that the legislation being considered could expose seriously ill patients to greater harm instead of the greater access that they are looking for. The Senate legislation would lower the bar for safety and effectiveness by allowing access to investigational drugs that have only completed a Phase 1 clinical trial. That is an extremely small trial that does not determine the effectiveness or potential side effects of a drug.

There is also no assurance in the Senate bill that a manufacturer will provide patients with an investigational treatment under this pathway. Today, pharmaceutical companies can choose to deny a patient access to an experimental treatment because there is not enough of the drug available or because they are concerned about dangerous side effects.

The Senate legislation also erodes important patient safeguards. It limits FDA's ability to use clinical outcomes associated with the use of an investigational product when reviewing a product for approval. And it also prevents any entity from being held liable for use of the treatment.

While I appreciate the intent of the legislation, I cannot support it in its current form. I hope that today's discussion will offer alternative solutions that may provide more meaningful access to investigational products without undermining FDA's ability to protect patients from harm. The last thing I want to do is give patients false hope, and to have Congress pass legislation that will not in fact help someone access investigational treatments.

I want to thank the witnesses for being here today, and look forward to what I hope will be a thoughtful discussion about a path forward.

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