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EXAMINING PATIENT ACCESS TO INVESTIGATIONAL DRUGS

TUESDAY, OCTOBER 3, 2017

House of Representatives

Subcommittee on Health

Committee on Energy and Commerce

Washington, D.C.

The subcommittee met, pursuant to call, at 10:30 a.m., in Room 2322 Rayburn House Office Building, Hon. Michael Burgess [chairman of the subcommittee] presiding.

Members present: Representatives Burgess, Guthrie, Barton, Shimkus, Murphy, Blackburn, Lance, Griffith, Bilirakis, Bucshon, Brooks, Mullin, Hudson, Collins, Carter, Walden (ex officio), Green, Engel, Sarbanes, Schrader, Eshoo, DeGette, and Pallone (ex officio).

Staff present: Ray Baum, Staff Director; Adam Buckalew, Professional Staff Member, Health; Kelly Collins, Staff Assistant; Zachary Dareshori, Staff Assistant; Paul Edattel,

Chief Counsel, Health; Adam Fromm, Director of Outreach and Coalitions; Jay Gulshen, Legislative Clerk, Health; Jennifer Sherman, Press Secretary; Hamlin Wade, Special Advisor, External Affairs; Jeff Carroll, Minority Staff Director; Tiffany Guarascio, Minority Deputy Staff Director and Chief Health Advisor; Samantha Satchell, Minority Policy Analyst; Andrew Souvall, Minority Director of Communications, Outreach and Member Services; Kimberlee Trzeciak, Minority Senior Health Policy Advisor; and C.J. Young, Minority Press Secretary.

Mr. Burgess. The Subcommittee on Health will come to order and I recognize myself for five minutes for the purpose of an opening statement.

I want to thank everyone for joining us this morning. We are here to explore an issue that is very personal to many patients, to their families across this country who are suffering from serious life-threatening conditions or terminal illnesses and that is the availability to access investigational drugs and devices.

Currently, the United States Food and Drug Administration conducts an expanded access program aimed at helping these patients who do not qualify for clinical trials to help them gain access to therapies that are unapproved by the FDA.

I understand the feelings and the passions of individuals who believe these therapies have the potential to save their life or offer them a chance to alter the course of their illness.

I also recognize that the Food and Drug Administration must strike the right balance between ensuring public safety and granting access to new treatments.

Today, we will convene four panels of witnesses. I first want to welcome Representatives Brian Fitzpatrick and Andy Biggs to our subcommittee. We look forward to hearing your statements this morning on the actions that you both have taken.

Of course, we are pleased to welcome Dr. Scott Gottlieb. Dr. Gottlieb, no stranger to this subcommittee, but I believe this

is your first opportunity to come before us as the commissioner of the Food and Drug Administration. So we, certainly, welcome your appointment to that post and welcome you to the committee. It's nice to have you here.

Afterwards, we welcome Mr. John Dicken, the director of healthcare at the United States Government Accountability Office, and then, finally, we will hear from other stakeholders who are deeply engaged on this issue.

Our nation has experienced an unprecedented amount of innovation and scientific breakthrough over the last decade from researchers in our finest academic institutions and from those working in the pharmaceutical and medical device companies.

However, I hear from patients with serious life-threatening conditions, constituents in north Texas, being frustrated with what they see as a regulatory barrier from trying and experimenting with new therapies when all others have failed them.

It seems we are at a crossroads when lifesaving treatments, while not yet approved, exist but patient cannot have access.

Since 2014, 37 states, including Texas, have passed a version of Right to Try laws through strong grassroots movements.

With that in mind, it is my hope that this hearing will start a constructive discussion on this important issue. The subcommittee will also examine several pieces of federal legislation -- S. 207, the Trickett Wendler Right to Try Act of 2017 authored by Senator Ron Johnson of Wisconsin;

Representatives Biggs' and Fitzpatrick's House companion bills; and H.R. 1020, the Compassionate Freedom of Choice Act of 2017, introduced by our fellow Health Subcommittee member, Morgan Griffith of Virginia.

Members of this subcommittee have many questions and are looking forward to hearing from all of the witnesses. We want to learn the Food and Drug Administration's steps to streamline and communicate the expanded access program.

We want to dive in to what the Government Accountability Office found recently regarding this expanded access program and we want to hear from our patient advocates and thought leaders on this topic.

There are strong view and I am confident that what comes out of this hearing will lead to a productive discussion and all of us getting closer to meeting the needs of our constituents and solving problems tomorrow that seem insoluble today.

I want to thank all our witnesses for being here today and I will not yield the balance of my time, Mrs. Blackburn. But will be happy to recognize any member on the Republican side who would like a minute and 12 seconds.

Seeing none, I will yield back my time and recognize the gentleman from Texas, Mr. Green, five minutes for an opening statement, please.

Mr. Green. Thank you, Mr. Chairman, and I would also like to thank our administrator, Dr. Gottlieb, for being here and our

two colleagues.

Mr. Chairman, expanding access, also known as compassionate use, allows patients to gain access to unapproved treatments that are on some stage of investigation outside a clinical trial.

The FDA has a long history of facilitating access to investigational therapies for terminally ill patients who are out of approved options and are ineligible for a clinical trial.

The 1997 FDA Modernization Act made amendments to allow patients to access investigational products under certain safety conditions.

In 2009, the agency revised its regulations to establish new categories of expanded access and streamlined the regulatory process for its program.

Last year, the FDA released guidance for industry about expanded access so that companies would better understand the rules of the road and avoid denying requests based on uncertainty.

It also streamlined the application, significantly reducing the time it takes to complete to, roughly, 45 minutes.

The FDA responds to individual patient access requests quickly and emergency requests are often granted immediately over the phone, something I know firsthand.

Today, we are examining two legislative proposals that are commonly referred to Right to Try bills. I am confident that we all strongly support helping patients with serious and life-threatening illnesses get the care they need and exercise

their right to make their own decisions about the risk they are willing to take.

Families with a loved one face terminal illness out of the FDA-approved options can and do have the right to seek out treatments that are in the early stages of investigation.

Unfortunately, the bills they are considering today are well meaning but based on inaccurate premise. These proposals would simply take the FDA out of the equation when the FDA authorizes more than 99 percent of all expanded access requests.

There are very legitimate frustrations with the current system and this committee, through the 21 Century Cures Act and the FDA Reauthorization Act, has worked to address some of them, but more can be done.

There is a widespread lack of knowledge about the FDA's expanded access program. We need to fill this education gap by partnership with doctors and nurses and patients organizations and local advocates so patients in need know what their options are.

The FDA has made its website more user friendly, streamlined the application process, and has a turnaround time of days, not weeks or months, and less than 24 hours in certain emergency situations -- again, something I have witnessed first-hand.

But the agency is correctly working to do more to clarify some myths and uncertainty that lead to a manufacturer -- to deny a request.

There is a rampant misunderstanding about compassionate use that also must be addressed. The FDA does not have the authority to force a company to make investigational products available.

From October 2015 to September 2016, the FDA received 1,554 requests for expanded access, investigational new drugs and protocols and ultimately allowed 1,545 of those requests to proceed.

This is an approval rating of 99.4 percent. Of course, there is some requests for investigational products that companies deny and we can do a better job of ensuring that doesn't happen in inappropriate reasons including a lack of clarity about how adverse events would be treated.

Ultimately, the best way to speed access to drugs and development is through a clinical trial process. We have worked to do a better job on making clinical trials available on an equitable basis for all patients.

But expanding clinical trial access we can reduce the number of patients seeking access to investigational drugs outside of the trials and ultimately help even more patients by getting drugs approved and widely available.

I believe we can and should do more to advance policies that genuinely increase access to promising investigational therapies for patients in need.

However, removing the FDA from the process of assessing a therapy outside a clinical trial is not likely to facilitate any

increased access to drugs in early trial stages.

Instead, we should be looking to examine principal reasons by patients interested in experimental therapies are unable to attain them through clinical trials or the existing expanded access and provide solutions to these real barriers.

We also must continue strong oversight of the implementation of requirements within the 21 Century Cures and have greater clarity from FDA on the use of adverse event data.

I appreciate our witnesses and, Mr. Chairman, I would like to ask unanimous consent to place a number of items into the record: the Patient Organization letter opposing right to try, American Cancer Society, Cancer Action Network, Friends of Cancer Research, the Leukemia Lymphoma Society, and 18 other organizations, a letter to Congress regarding S. 204 submitted by Public Citizen and 17 other organizations. Do you want me to read this whole list or can I just submit it?

Mr. Burgess. Without objection, so ordered.

[The information follows:]

**********COMMITTEE INSERT 1**********

Mr. Green. Thank you.

Mr. Burgess. Does the gentleman yield back his time?

Mr. Green. I yield back my time.

Mr. Burgess. Gentleman yields back his time.

Not seeing the chairman of the Full Committee having arrived yet, the chair is prepared to yield to the ranking member of the Full Committee, Mr. Pallone of New Jersey, five minutes for an opening statement, please.

Mr. Pallone. Thank you, Mr. Chairman.

Today's discussion is of great importance for 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 FDA and Congress have taken some actions that provide 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 1,500 requests and only nine were not approved.

And 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 nonemergency requests are processed in an average of four days.

Despite these quick turnarounds, FDA responded to these criticisms. Last year, the agency streamlined their 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 submissions process, and all this was done to alleviate any confusion that may have existed in the past and I want to commend the agency for its commitment to improving expanded access and for its responsiveness to the concerns it heard from doctors and patients.

Now, 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 treatment for patients who may not qualify for these trials.

These are all meaningful steps that I believe will help to address some of the criticisms we will hear today. Now, our discussion today is 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 one clinical trial, and that's an extremely small trial that does not determine the effectiveness of potential side effects of the 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 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 investigation product when reviewing a product for approval.

It also prevents any entity from being held liable for use of the treatment. Now, while I appreciate the intent of the Senate legislation, I have a hard time supporting it in its current form and I guess what I am hoping is that we will hear today about alternative solutions that may provide more meaningful access to investigational products without undermining FDA's ability to protect patients from this harm because 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.

So, hopefully, we will hear more about, you know, ways that we could make some changes that don't -- that don't sacrifice safety and I look forward to what I hope will be a thoughtful discussion about a path forward.

I yield back, Mr. Chairman.

Mr. Burgess. Chair thanks the gentleman. The gentleman yields back.

The chair now recognizes the gentlelady from Tennessee, Mrs. Blackburn, five minutes for an opening statement.

Mrs. Blackburn. Thank you, Mr. Chairman, and I am not going to take that full five minutes and will submit my full opening statement for the record.

I do want to welcome our colleagues here to the committee. I want to welcome Dr. Gottlieb. We are just so pleased that we are going to be able to take up what is, I think, a really important issue for us to address when we look at healthcare, and that is the right to try.

And we want to commend the FDA for going through the process and taking some efforts to simplify and expedite request. We do think that it is important for Congress to do something legislatively to ensure patient access to promising treatments but do it with the appropriate disclosure requirements and the liability protections.

So we welcome all of you that are here today. We appreciate the time and effort that has gone into this and the fact that we are going to have the multiple panels so we can kind of drill down and do a good solid look at this from the patient perspective, from the legislative perspective, from the regulatory perspective.

So to each of you, welcome, and thank you and I yield back.

Mr. Burgess. Chair thanks the gentlelady. The gentlelady yields back. This concludes member opening statements. The chair would remind members pursuant to committee rules all members' opening statements will be made part of the record.

The gentlelady from Tennessee is quite correct. We have a total of four panels of witnesses testifying before the subcommittee today.

To start us off, we are going to hear from two of our House colleagues -- Congressman Brian Fitzpatrick of Pennsylvania and Congressman Andy Biggs of Arizona.

We appreciate both of you being here with us this morning. Congressman Fitzpatrick, you're recognized for five minutes for your statement.

STATEMENTS OF HON. BRIAN K. FITZPATRICK, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF PENNSYLVANIA; HON. ANDY BIGGS, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF ARIZONA

STATEMENT OF MR. FITZPATRICK

Mr. Fitzpatrick. Thank you, Mr. Chairman.

Good morning. I want to start by thanking Chairman Burgess, Ranking Member Green, Vice Chairman Guthrie, and all members of this subcommittee for holding this hearing. It's a very important hearing on the right to try, and I also want to thank my colleague and friend, Andy Biggs, for you partnership on this issue.

Fellow colleagues, each year thousands of Americans receive the devastating news of a terminal diagnosis. Even with the amazing work done in American medical research and development, for far too many families access to these potentially lifesaving treatments will come too late or not at all.

Thousands of terminally ill patients suffer needlessly while waiting final approval for drugs, therapies, and other medical technologies, and while the Food and Drug Administration carries out its three-phase approval process, which can take years and cost billions of dollars, many patients simply want a chance to try treatments that are already demonstrated to be safe.

Mr. Chairman, it is my hope that we can come together with federal regulators and industry leaders to clear the path forward

to care for those who are fighting just for a shot at living.

A bill that was unanimously passed by the Senate -- unanimously passed by the Senate -- will offer them a chance to extend their lives.

The Right to Try Act would ensure that terminally ill patients, together with their physicians and pharmaceutical manufacturers, can administer investigational treatments where no alternative exists.

In fact, this bipartisan idea is already the law in 37 states in our nation. A federal Right to Try law would prevent the government from blocking access to potentially lifesaving medications.

It would require patients who are unable to participate in clinical trials to first try all other available treatments.

Mr. Chairman, I want to note that these provisions only apply to terminally ill patients. It does not undo the FDA approval process but, rather, provides a potential lifeline to those who simply cannot wait.

It requires a physician to certify that all other options were exhausted or unavailable. This maintains the incentives for patients to seek out and join clinical trials.

This bill requires that a product meet demonstrated levels of safety by obtaining FDA phase one approval. We have worked with the drug companies to ensure that adverse outcomes are not used against any ongoing application for approval.

Additionally, patients, doctors, and manufacturers do not assume any additional liability under this act. For those patients caught in between traditional drug approval delays, clinical trial process for which they do not qualify and limited time, this Right to Try legislation simply establishes the freedom for patients and their doctors to try therapies where the benefits far outweigh the risks.

It gives them the option of trying to save their life. Whether it is a father courageously battling ALS or a brave child living with Duchene muscular dystrophy, my colleagues, they deserve the right to try.

I want to sincerely thank the committee for your time and consideration and as your colleague I ask that you work with us to get this done on behalf of all terminally ill patients across America.

All that we ask -- all that we ask is that this bill be put -- be put on the floor of the House and allow each one of us to cast our vote and go home and answer for that vote.

Mr. Chairman, I yield back.

[The prepared statement of Mr. Fitzpatrick follows:]

**********INSERT 2**********

Mr. Burgess. Chair thanks the gentleman. The gentleman yields back.

Chair recognizes the gentleman from Arizona, Mr. Biggs, five minutes, please, for your statement.

STATEMENT OF MR. BIGGS

Mr. Biggs. Thank you. Thank you, Mr. Chairman.

I, first, also thank Chairman Burgess, Ranking Member Green, and all the members of the committee for allowing me to address you today.

I am here with my friend and colleague, Representative Brian Fitzpatrick, to fight for passage for the Right to Try Act.

This bill that I introduced with Mr. Fitzpatrick in February now has dozens of bipartisan co-sponsors including members in this very room today.

I am particularly pleased that Senator Ron Johnson's bill will be considered today as well. As the committee may know, Senator Johnson's bill passed the Senate by unanimous consent.

Anyone who understands the arcane procedures of that chamber can attest that this is no mean feat. I am strongly supportive of Mr. Johnson's efforts. He has been a tireless advocate of right to try for years.

I won't take up a great deal of this committee's time elaborating on the virtues of the bill Representative Fitzpatrick and I introduced because, frankly, very little explanation is necessary and Mr. Fitzpatrick has done a great job explaining it.

Fundamentally, our legislation allows terminally ill patients who have no further options -- I repeat, no further options -- the opportunity to try experimental drugs that could

save their own lives.

Yes, there are also provisions in our bill to protect both the patients themselves and the pharmaceutical companies who want to participate.

But those provisions are secondary to its primary purpose. The primary purpose of our Right to Try Act is to give brave patients across this country some choice over their own destinies when all other avenues are closed.

We should all share the same goal of doing everything we can for patients fighting to save their lives and I have no doubt that the intentions of everyone in this room are good.

So what are we waiting for? Why isn't this committee doing everything possible to get Right to Try passed out of Congress and onto President Trump's desk? That's really the next step. We need to get this out of the House.

The status quo is not the answer. We will hear claims today from the FDA and other agency officials that their own expanded access program is working and continues to improve.

There may be some truth to that and I am sure that Commissioner Gottlieb works tirelessly to help as many terminal patients as he can.

But that program is simply not enough. Frankly, that program was not put into high gear without federal legislation looming.

I know that the program is simply not enough because I have

talked to dozens and dozens of patients, family members and advocates, who tell me it is not enough.

They come to my office. They call me on the phone. They write me impassioned letters. These same advocates have ensured that Right to Try has become law in 37 states.

Think about that for a moment. In half of those 37 states, Right to Try laws passed with unanimous support -- bipartisan support -- and in my home state of Arizona, voters approved this initiative with nearly 80 percent of the popular vote and I am convinced that the other 20 percent were just the folks that always vote no.

At a time when pundits are claiming that our politics are broken -- Republicans and Democrats can't come together on anything -- here is a cause -- here is the cause that Americans of all political stripes believe in.

I was first introduced to Right to Try while serving in the Arizona State Legislature with fellow legislator and friend, Laura Knaperek. By 2014, she was no longer a legislator but she was an advocate, suffering in the fight of her life against ovarian cancer.

Her mission became to see Right to Try passed into law. In the end, her efforts for this cause succeeded beyond everyone's wildest expectations. Unfortunately, Laura is no longer with us. She lost her brave battle with cancer but her legacy as a tireless patient advocate lives on.

I will continue to carry on Laura's fight, not just for her but for all those brave patients across this country who are battling against the odds every day.

I fight for Bertrand Might, for Jordan McLinn, for Matt Bellina, who is testifying today, and I fight for the countless other patients who deserve a right to try. I urge you to join in that fight. We must act further without delay.

Thank you again, Mr. Chairman, and Ranking Member Green and members of the committee. I yield back.

[The prepared statement of Mr. Biggs follows:]

**********INSERT 3**********

Mr. Burgess. And the gentleman yields back and the chair thanks the gentlemen.

The chair thanks both gentlemen for being here, taking time to share with us your stories and your passion and taking the time to testify before the subcommittee. It is helpful to us in our deliberations.

Again, I want to stress that there are four panels today so we are going to move smartly to the next panel. As is customary, there will not be questions for the -- for the members from the members but following each of the other panels there will be opportunities for questions from members.

Our second panel we are very, very pleased to have Dr. Scott Gottlieb, commissioner of the United States Food and Drug Administration.

Doctor, we certainly sincerely appreciate you being here today and you are now recognized for five minutes for your opening statement, please.

STATEMENT OF DR. SCOTT GOTTLIEB, COMMISSIONER, U.S. FOOD AND DRUG ADMINISTRATION

Dr. Gottlieb. Good morning, Mr. Chairman, Mr. Ranking Member, and members of the subcommittee.

I want to thank you for the opportunity to testify this morning. This is my first time testifying before the Energy and Commerce Committee and I'd like to take a moment to thank you for your strong support of FDA and its public health mission.

I know this committee and this subcommittee in particular worked hard to enact the 21st Century Cures legislation and FDARA, and I also want to acknowledge your continued efforts to modernize the review and approval of OTC products through user fee legislation and I look forward to working with you closely on all of our shared goals.

Throughout my career I've worked to advance policies to enable terminally ill patients to obtain earlier access to promising new drugs and this includes preapproval access to experimental medicines.

As a cancer survivor who used an approved drug in an off-label fashion in the treatment of my own cancer, I've grappled with some of these issues first-hand.

While my cancer was very curable, I know that many patients with serious illness face long odds and their best chance at gaining an advantage in those odds is with something unproven and

experimental and we need to make sure that we serve these patients.

Before I discuss these goals and the issues related to Right to Try legislation, I'd like to take a moment first to acknowledge the tragedy in Las Vegas and then to expand on another tragedy unfolding in the south, the crisis facing Puerto Rico.

I want to just brief the committee on some steps that are going on right there -- right now with respect to that crisis at FDA.

I was grateful for the opportunity to accompany the secretary of the Department of Homeland Security on her trip to Puerto Rico on Friday.

I visited with my FDA team stationed in San Juan where we have about a hundred full time staff. Our large staff is a reflection of the significant medical product manufacturing capacity on that island.

We are now engaged in a sweeping effort to cross the entire agency to provide direct assistance to our staff and fellow citizens on the island and this includes efforts to get food and medical products onto the island and get hospitals back into full operation.

But the devastation in Puerto Rico presents a broader challenge to the FDA because it is home to a very large medical product manufacturing base for both drugs and devices.

Some of these facilities make products that could be in shortage if production is sharply diminished or pushed offline.

This is particularly concerning because some of these products are critical to Americans.

A loss of access to these drugs and devices could have significant public health consequences. This includes products for the treatment of cancer and a lot of other unmet medical needs.

Getting these facilities back online is a public health priority. It's also a priority of Puerto Rico's recovery. I've discussed this matter directly with the governor of Puerto Rico and his staff.

These sites directly employ about 90,000 residents of Puerto Rico and represent 30 percent of the island's GDP. Puerto Rico is home to an excellent high-quality manufacturing for sophisticated medical products including many injectable drugs and complex devices.

A highly skilled, highly dedicated, highly productive Puerto Rican workforce enables the success of this industry.

If we don't get these facilities back online in a timely way and they decide to relocate after this disaster, it would jeopardize the island's economic future. For many reasons, not least our concern for the people of Puerto Rico, we need to work to help to restore this manufacturing base.

I can tell you the leadership of FDA is committed to all these efforts. We stand with the people of Puerto Rico. I have been personally engaged in troubleshooting these issues, working directly with my colleagues at HHS and DHS and the staff of the

governor of Puerto Rico and I am available to brief this committee directly on these efforts.

On the topic we are here to discuss today, I want to share some insight into some of the recent steps FDA took to improve our expanded access program and continue to facilitate access to promising drugs targeted to unmet needs prior to approval for patients with serious or immediately life-threatening illnesses who don't have other alternatives.

I have announced some new policy actions that we are taking today and we intend to take additional steps in the near future.

Critics of these efforts may look at our actions individually and say that none of these measures will materially change the current balance.

But this effort cannot be solved in one step. We need to look across the totality of what we are doing to measure the impact of our endeavors.

My goal is to establish a framework that preserves our current approval process while making sure that there are efficient achievable avenues for patients to access promising drugs targeted to unmet needs.

We need to serve all the interests of patients facing serious illness who lack good options. This includes their interest in trying unproven drugs.

I am committed to this goal. I believe in this right. I support this idea. I look forward to answering questions.

Thanks a lot.

[The prepared statement of Dr. Gottlieb follows:]

**********INSERT 4**********

Mr. Burgess. The chair thanks the gentleman for his statement and I am in agreement with the commissioner about the need for our intention upon helping the citizens -- United States citizens in Puerto Rico and their recovery and I think you'll see some of our efforts in the children's health insurance bill that we mark up tomorrow.

Already beginning some effort to -- with some help that is -- that is going to be available there. But it by no means completes that task and this committee -- this Congress will have a significant ahead of it in recovering from the -- from the storms of September.

I am going to recognize myself for five minutes for questions and we will alternate between Republicans and Democrats.

Commissioner, I guess my first question is, you know, when I arrived in the United States Congress, I don't think I had prior knowledge, as a practising physician for 25 years -- I don't think I knew about clinicaltrials.gov and so as a follow-on to that, how are we communicating, yes, clinicaltrials.gov and making sure people are aware that there are clinical trials that are available but then, moreover, the availability of these expanded use programs? So what do you see as a communication strategy coming from the agency in that regard?

Dr. Gottlieb. Well, Mr. Chairman, in answer to your question, I think the short answer is until recent years we probably didn't communicate very well and that's why patients face

more obstacles getting access to experimental drugs than perhaps they should have.

With the help of this committee we have taken new steps to try to make information about the availability of drugs through expanded access programs more available, easier to find.

There's provisions in 21 Century Cures Act that requires sponsors to post notification of the availability of drugs through expanded access programs on their website.

We are starting to work with sponsors to gain compliance with that. There's also provisions that they need to post information about clinical trials to clinicaltrials.gov and we are working with sponsors to broaden the compliance with that as well.

But we are not just relying on those measures, as potent and as important as they are. We are also working with the private sector and patient group interests to create some new tools and one of those tools is something I am talking about -- I talked about today in my written testimony for the record, something called the Navigator, which we developed with the Reagan-Udall Foundation, which is going to create a one-stop portal for access to information about expanded use programs.

Right now that tool is targeted to drugs for oncology, for cancer. We announced today that we are going to broaden it for drugs targeted to rare diseases and we look to broaden this even further.

I think that this could become a consolidated web portal,

if you will, for access to this kind of information so patients have one place to go and we've been working closely with sponsors to get them to report to this -- to this new tool.

Mr. Burgess. I thank you for that answer. In our next panel we are going to hear from the Government Accountability Office and their recommendation for action that they give at the conclusion of the GAO report.

So their recommendation -- let me just read it here -- the commissioner of the Food and Drug Administration should clearly communicate how the agency will use adverse event data from expanded access use when reviewing drugs and biologics for approval and marketing in the United States.

So we are going to hear testimony in the next panel that this recommendation has been given to the FDA and can you kind of brief us as to the status of that recommendation?

Is this something of which you were aware? Is this accurate?

Dr. Gottlieb. Well, I hate to short circuit the testimony of my colleague at the GAO but we've taken their advice and we've announced that today.

And so we've doubled down on a proposition that we have long held that this information typically isn't used in the consideration of a product and a product's approval and we've clarified in a new guidance document that we are posting today that the circumstances under which this information would and wouldn't be used and the bottom line is that information gleaned

from an expanded access program is exceedingly unlikely to be incorporated into a consideration of the approvability of a product.

We are saying today in the guidance document that we must consider the circumstances in which the product is being used as a component of whether we'll consider whether or not an adverse event recognized in the use of that product is attributable to the drug.

And in the setting of an expanded access program when you -- when you have a patient with a terminal illness who is oftentimes on a lot of other therapy, it is very hard to make a determination that any one drug was responsible for any one observation in that setting and so we are exceedingly unlikely to use that information.

And just to reinforce that, we looked across a decade of experience with expanded access, 322 products that were approved over that period of time, 28 percent of which had expanded access opportunities associated with the products, and we could find no instance where information gleaned from an expanded access program was used to deny approval of the drug.

We found one instance where information gleaned from the expanded access program was incorporated into drug labelling and we actually found one instance where the data gleaned from the expanded access program actually informed our consideration of the effectiveness of that product and helped lead to its approval.

Mr. Burgess. I thank you for the answer.

I will yield back and recognize the gentleman from Texas five minutes for questions, please.

Mr. Green. Thank you, Mr. Chairman, and again, Dr. Gottlieb, thank you for being here.

The legislation we are considering today offered by Senator Johnson proposes to offer terminally ill patients a new pathway to investigational products without FDA review or approval.

One of my concerns with this legislation how broadly it would apply. For example, under the Senate legislation, an eligible patient is defined as a patient diagnosed with a life-threatening disease or condition.

My first question, Commissioner, as I understood, S. 204 would provide eligibility to a much broader range of patients than those with terminal illness and even under state Right to Try laws.

Would you discuss further when a patient population is eligible for FDA's expanded access program currently and what patient population would be eligible under S. 204?

Dr. Gottlieb. I appreciate the question, Congressman.

I think -- I think the -- your statement embedded in the question is correct. Right now, our expanded access program is generally available for patients facing life-threatening conditions and terminal illness.

We provide for both emergency and nonemergency situations. As part of the technical assistance that we provided to Congress

in their consideration of this bill, one of the comments that we made is with respect to the definition of a terminally ill patient.

If you look across the state laws and states that have passed Right to Try laws, the language typically speaks about a patient being terminally ill to qualify for consideration under the Right to Try provisions.

Congress, in consideration of some of this legislation -- and there's various bills that have been considered by this body -- but in some of these legislative measures have broadened that to include life-threatening diseases or diseases that could be life threatening -- excuse me, diseases that are -- that are either terminal or life threatening, and this, in our estimation, could also potentially include chronic illnesses like diabetes or other diseases that while not -- don't set a patient on a terminal course in an immediate way, certainly are life-threatening diseases.

And so one of the suggestions that we've had in our technical assistance, and it is also a component in my written testimony, is to consider more carefully the definition and maybe map it more closely to what some of the states have done in their consideration of this measure.

Mr. Green. Okay. So the two issues would be terminal or life threatening?

Dr. Gottlieb. That's right, Congressman. Our -- as part of our technical assistance, we urge Congress to consider that language and consider whether or not it should be defined as a

patient who is terminally ill, similar to what the state laws have done.

The component of a life-threatening disease is a broader definition and, as Dr. Burgess would probably agree, there's a lot of chronic illnesses that are certainly life threatening but not immediately terminal.

Mr. Green. My understanding from supporters of the Senate legislation and from those supporting the state Right to Try laws is that the intent is to help support increased access to investigational products for terminally ill patients.

If we are to consider legislation moving forward regarding this goal, it is my hope that we would all agree that we should align any legislation with that targeting population we are discussing in terminally ill patients and that way to make most terminally ill patients access to the drug is to have drug approval by conducting clinical trials.

I am not convinced that the FDA is a barrier to investigational treatments and I continue to have concern about this legislation. But I appreciate your testimony on this today. Yes?

Dr. Gottlieb. I just want to -- if I may follow up, with your -- with your indulgence, Congressman.

The reason -- the reason why I think it might be important to consider how we define terminal illness here and not -- and make sure we are not too expansive if we do move forward with this

legislation is so as not to broaden it in a way that it might undermine its intended purpose.

I think the more we broaden this measure and the more it is opened up to a broader set of conditions, the more we risk undermining the central purpose of the legislation and that would be -- that would be the policy reason for considering how we define that.

Mr. Green. Thank you, Mr. Chairman. I am almost out of time and I don't think you have time to answer the question.

One of my concerns it requires doctors and distributors to report adverse events. We need to make sure that's -- if the legislation moves forward we need to make sure that's defined correctly, Mr. Chairman, and I yield back my time.

Mr. Burgess. Gentleman yields back. The chair thanks the gentleman.

Chair recognizes the gentleman from Oregon, Mr. Walden, chairman of the Full Committee, five minutes for questions, please.

Mr. Walden. I thank the gentleman and I appreciate the indulgence of the committee. We have been down with another hearing on Equifax and the little data breach issue that only affected 145.5 million Americans. So I have been done at that hearing.

Dr. Gottlieb, first of all, delighted to have you before the committee. We are delighted you're at the FDA. We appreciate

the reforms that you're bringing to that agency and we look forward to a long continued interaction with you and this committee in the work that you're doing.

The FDA recently took action to simplify the expanded access process, specifically the new form for physicians as 11 elements compared to the previous 26 elements, and there is now a partnership with the Reagan-Udall Foundation to help patients and physicians navigate the process.

I know it may be a bit premature, but are you able to share any statistics on the impact of these two modernizations to the expanded access program?

Dr. Gottlieb. Congressman, it is too early for us to really draw any conclusions about, you know, the direct impact that it has had. We hope it will be very impactful.

Mr. Walden. And I appreciate your testimony and that of our colleagues, certainly, Mr. Fitzpatrick and Mr. Biggs, and I know your own personal experience.

And, you know, having lost loved ones to really tough diseases, especially cancer, and I think we all sort of grasp isn't there something else out there I can try, and it is that balance in public policy of patient safety versus trying to help people with terminal illnesses get access to something that could help them.

And so when you look at this legislation -- I know you talk about better definition on the terminal illness piece -- could

you speak more to that and what -- why that clarification might be necessary?

Because I have been told by at least one of the Senate sponsors of this bill that they are not looking for the House to make any changes out of fear it may fail if it goes back with changes. And so I am concerned about that.

Dr. Gottlieb. Well, I mean, the bottom line is that the definition, if it were to incorporate life-threatening diseases is broad and as a clinician I can certainly contemplate a lot of diseases that are life threatening but not immediately life threatening, and the way it is written I think the agency would have to, as a matter of legal policy, have to interpret that potentially expansively.

So it could -- it could sweep in a whole range of conditions for which we didn't intend. And I would just be mindful that if the goal is to make sure that we are serving the interests of patients who are facing terminal illnesses, the more we broaden this provision and the more we potentially sweep in conditions for which we might be exposing people to unwanted side effects from experimental therapies, the more we risk undermining the whole venture that we are trying to engage in here, which is to narrowly tailor something to people who really don't have good options from available therapy.

Mr. Walden. And do you think the way this is currently written could hurt people then?

Dr. Gottlieb. Well, I think the way this --

Mr. Walden. The process?

Dr. Gottlieb. I think the way this is currently written it could undermine some of the goals of the policy and we've been consistent in providing that technical assistance all the way through.

And so I am representing the agency's point of view. In terms of hurting people, to the -- to the extent that we are trying to strike a balance between taking potentially some significant risk in a setting of a terminal illness and allowing patients to take that risk and make that informed judgment and then opening up that same risk to patients who don't necessarily face the same circumstances, we are certainly going to be exposing patients with potentially less severe conditions to a risk that we might think as a matter of public policy is only appropriate if we are being good stewards of the public health is only appropriate in a setting of a terminal illness.

And so I think we need to be just cognizant of that. We are -- we are willing to allow patients to take certain risks in one setting. We think it is their right.

The question is do we think it is appropriate for patients who are in a much different setting to contemplate those same risks outside of a regulatory process that we've carefully constructed. That makes careful balances.

Mr. Walden. So help me understand this, if you can, the term

life-threatening disease or condition. What -- in real people speak, what does that mean? Who would be -- what sort of conditions?

Dr. Gottlieb. Well, as a physician who used to treat until recently hospitalized patients, I would consider advanced diabetes a life-threatening disease. I would consider class two heart failure a life-threatening disease.

There's a lot of Americans with those conditions. They're not immediately life threatening. A lot of those patients will go on to live many years but they face a chronic illness that is life threatening, certainly. They might eventually succumb to their illness. That is a broad category of patients.

So this will -- this -- with that language we potentially open it up to a very broad category of patients and I can tell you through discussions that I've had with attorneys at FDA I think we'd have to interpret that broadly.

I don't think that we'd be able to, as a matter of our own interpretation of the law, further narrow that. I think, if anything, we would have to interpret that fairly expansively.

Mr. Walden. All right. My time has expired. Thank you very much.

Mr. Burgess. Chair thanks the gentleman. The gentleman yields back. The chair recognizes the gentleman from Oregon, Dr. Schrader, five minutes for questions, please.

Mr. Schrader. Thank you, Mr. Chairman. Appreciate that.

Somewhat along, I guess, the same lines of the question that's been going on here, I guess, Mr. Gottlieb, seems like there are a lot of provider groups that are not enthusiastic about the need for this legislative change. Would you comment on that?

Dr. Gottlieb. Well, we've heard from a lot of provider groups, certainly, and some groups that represent patients about concerns related to this legislation and I think the general concern is about the risk of undermining a regulatory process that has been carefully crafted over many years to strike a very careful balance.

I think people do worry about upsetting that balance, given all the thought that has gone into how we've created that framework.

Mr. Schrader. Was the -- you have indicated that you already made some changes based on the GAO report. Was the report overall favorable or unfavorable to the current program?

Dr. Gottlieb. I felt that -- I can only speak for my own interpretation of the report -- I felt the report was overall favorable -- a favorable view of what FDA was doing with some targeted recommendations about improvements that we can make.

Mr. Schrader. And, you know, again, it has been mentioned that 99 percent of the expanded use or compassionate use applications are approved.

How does it get much better than that with this new legislation? How would this new legislation affect that approval

rate?

Dr. Gottlieb. Well, the legislation is certainly not going to affect -- you know, affect an approval rate that, to your point, is 99 percent and actually getting better than 99 percent is a sweep of over a decade and when you look in the more recent years I think the agency has gotten even more vigilant at trying to move these things through the agency in an efficient fashion and approve these.

I think there is a perception, and I can't speak to the perception, that there are certain companies and products that aren't necessarily being offered under the current construct and the Right to Try legislation might provide more of an incentive and an opportunity.

Probably an opportunity incentive would be the wrong word -- an opportunity for companies to offer products in a different -- in a different setting.

We don't -- I don't necessarily see that same opportunity because I think that the biggest obstacle to offering drugs through expanded access is the supply constraints.

I think we ought to think about how we address that separately. It think there might be ways to address that through incentives.

But from my perception where I sit -- and I've been on the other side of this -- I've worked in -- with small biotech companies before I came to the agency, as the committee knows --

the biggest obstacle I see is the availability of supply for patients who want to get access to unproven therapies.

Mr. Schrader. Given the fact that there are all these states that are passing or have passed Right to Try legislation, why would they be doing that if the program seems to be working so well by, you know, your testimony -- would indicate working so well at this time?

Why are states doing that? Are we seeing a big upsurge or uptick in new drugs, new medical devices being approved in these Right to Try states we wouldn't through your process?

Dr. Gottlieb. It is hard to tell. We don't have data yet, Congressman.

I mentioned that I used a drug experimentally in my -- in the treatment of my own cancer at the outset. I had a very curable cancer. I was told that I had an over 90 percent chance of curing my cancer.

What I was looking for was how do I get 90 percent to 91 and 92 percent, and the way I was going to do that was to look for pristine clinical data that could help inform me how to use available therapy in a better fashion to slightly improve my odds.

That's very different from a patient who's told that they have a 10 or 20 percent chance of surviving their illness and they are looking for something very different.

They're not looking necessarily for a study that's going to tell them how to get 20 percent to 25 percent. They are looking

for something unproven -- a silver bullet -- something that could dramatically change their odds, and invariably that's going to be something experimental, and if it wasn't then they wouldn't be told that they only have a 20 percent chance of surviving.

I think we need to make sure we serve both patients. I am not sure that we always do. I am committed to doing that. That's why we are working on the reforms that we are doing.

I think that there's a broad perception out there that we don't always serve both patient communities well and that's been the impetus for these Right to Try laws.

I think that there are things we can do. We'll certainly work with Congress on this legislation. If Congress passes it, we will certainly implement it in a robust fashion.

I still think that there is a lot that I can do as the FDA commissioner to try to improve programs for patients who are told that your chances of surviving your illness are 20 percent.

Mr. Schrader. Very good. Thank you. I yield back.

Mr. Burgess. Chair thanks the gentleman. The gentleman yields back.

The chair recognizes the gentleman from Texas, Mr. Barton, the vice chairman of the Full Committee, five minutes for questions, please.

Mr. Barton. Well, thank you, Mr. Chairman. Thank you for holding this hearing.

Doctor, we appreciate you being here. I really just have

one basic question and that is if we -- if we believe in the doctor-patient relationship, which I do, if your doctor comes to the decision that all reasonable conventional therapeutic efforts have been exhausted in trying to protect your life and is willing to state that, and if the patient is willing to forego any legal lawsuit claims against some of these new therapies, why wouldn't the FDA approve that?

And I am told at the staff level that the FDA has been extremely responsive the last three or four years in approving requests for new treatments when the patient has exhausted all of their options.

But, you know, I listened to your answer to Chairman Walden and it sure does seem to me that even with the best of intentions the FDA still thinks they know better than the -- than the doctor who's treating the patient.

Dr. Gottlieb. Well, Congressman, I appreciate the questions. I am not sure that I -- that I agree with the conclusion.

We do approve it. The bottom line is we do approve it and, you know, data has been quoted here that in more than 99 percent of cases that we have a request even on an emergency basis or a nonemergency basis we do approve it, and in 10,000 encounters, requests for expanded access in a nonemergency setting where we -- where we denied about 25 of them, in about half of those denials it was because the drug just wasn't available and in other cases

it is because we know that the drug is on a clinical hold for a significant safety reason but the public doesn't know that because the existence of the clinical hold is confidential information.

You know, we are committed to continuing to push on this and to make it easier for patients to access it. I think the issue isn't do we approve it do we not approve it.

The bottom line is in the vast, vast majority of cases we do approve it. The issue is, is it always available and do patients always know about it. And I think on the question of do patients always know that they can pursue these options, we can make that easier.

We can make that information more readily available with the help of Congress and the provisions in the 21 Century Cures.

On the question of whether or not it is always available, the answer is, unfortunately, it is not. Unfortunately, these products are supply constrained because of manufacturing constraints.

I think there, too, there are things that we can do through how we design clinical trials that potentially could make more product available in the setting -- in the preapproval setting.

Mr. Barton. Then why not just empower the FDA to say that we approve it but you may not be able to get the drug -- you may not be able to get the therapy because it is not available.

Or, if you tell them no, say because this stuff is most of the time not working -- we put a hold on it because it is not helping

anybody, I mean, I am with you on that.

But my brother died of liver cancer and they tried all the conventional therapies in the world on him and it just wasn't working, and we got him into a clinical trial that was helping 90 percent of the liver cancer patients but the 10 percent it didn't help it expedited the disease and he was, unfortunately, one of the -- in the 10 percent group that it accelerated his cancer as opposed to terminated it.

But we knew what we were doing. We took that chance. He and his wife and his -- myself and my mother, we all -- and his pastor, we -- we said we are going to give this a shot because if it works it will really help, and it didn't.

But we didn't -- we didn't then go back and say, oh, jump on the FDA for doing it. We knew up front what the risk was and I don't -- I just don't see -- I mean, Mr. Griffith has a bill before this committee right now, and there are others, let's err on the side of the doctor/patient knows more.

And I am not being negative on the FDA but you're trying to protect the broad public health, which is commendable. But I would -- I would say in this case let's pass some law that makes it easier to get this stuff -- agreeing that in most cases you folks have been very positive about giving them the chance.

With that, Mr. Chairman, I yield back.

Mr. Guthrie. [Presiding.] Thank you. The gentleman yields back.

I recognize Ms. Eshoo for five minutes for questions.

Ms. Eshoo. Thank you, Mr. Chairman, and welcome, Dr. Gottlieb. It's wonderful to have you here, and congratulations on heading up the FDA.

In listening to everyone, I am reminded that we are all a diagnosis away from something and I admire how you not only handled your own challenge.

But it is a source of comfort to me that -- not to you probably but that you had this challenge and that you can view so many of these issues through that lens and I think that that is very important and it has really added a lot to, I think, to your testimony today.

What I am struggling to figure out what is broken here. The FDA has very good figures. I have read the GAO report and, overall, I agree with your description of it and they do add some things that the FDA can do.

But what do you think is broken here? It is my understanding that if a patient -- it starts with the patient. Patient goes to the doctor and says, I have either read out or I have heard about or whatever such and such a experimental drug and I want it.

The doctor then has to request that of the manufacturer? Is there something broken down that breaks down in that process? Because we have bills before us that suggest that it is larger than what the numbers -- what the data suggests.

So can you identify what you think is broken?

Dr. Gottlieb. Well, I would like to just start, Congresswoman -- I appreciate the opportunity to answer your question.

I am -- in response to the last question, I am in favor of giving patients -- sick patients options and in the setting of a patient who's suffering -- in the setting of most patients that's the safe and approved option that's been reviewed by the FDA.

Ms. Eshoo. Right.

Dr. Gottlieb. But sometimes that's an unproven option and sometimes the risk of nothing is worse than the risk of something experimental and we need to consider that and we do consider that through our expanded access program.

But this is a complicated issue, and to your point, there are things that aren't working that are frustrating the ability of patients even who have a physician who's willing to work with them, even who I have identified a drug that they think can help their illness, even with an FDA that is devoting a lot of new resources to trying to facilitate access to these products.

Even with all of that, patients still have trouble getting access to products that they think can help save their life.

Ms. Eshoo. But why are they having trouble getting access to it?

Dr. Gottlieb. The supply -- the biggest reason is supply.

Ms. Eshoo. It is the supply?

Dr. Gottlieb. The biggest reason is that when we do clinical trials -- when companies do clinical trials, they don't have continuous manufacturing.

They don't have large facilities online pumping out endless supplies of a drug. They will do what they -- what they call discontinuous batches.

They'll do -- they'll do runs just to create batches of drug supply and API -- active pharmaceutical ingredients -- sufficient for the clinical trial and that supply doesn't go through the good manufacturing standards that a supply of drug goes through that's commercially available.

Ms. Eshoo. On this supply issue, do either one of the bills address any of this?

Dr. Gottlieb. No. We would have to think of different ways to provide incentives or perhaps a different clinical trial framework to try to get at that issue.

Ms. Eshoo. Uh-huh. Now, one of the bills before us today would allow patients to access the investigational drugs while the other would allow patients to access investigational drugs and devices. That's a -- that's a whole another very important area.

Now, if patients are granted access to unapproved medical devices that a physician isn't trained to use, there could be, I think, some bad outcomes.

Now, I understand that medical device companies already face

many challenges to enrolling patients in clinical trials. The Right to Try proposals that include devices could divert patients from otherwise -- I think from participating in a clinical trial.

So give us your thoughts on Right to Try legislation including medical devices in addition to drugs.

Dr. Gottlieb. Well, I think your statement is correct. I agree with it. Medical devices are tools in the hands of physicians. Physicians often have to undergo very rigorous training on devices, even after they are newly approved.

And so there's a different set of considerations and potential risks associated with making devices available in a setting where you don't have the normal structure -- regulatory structure in place.

But setting that aside, we believe that the compassionate use framework on the medical device side of our house is working quite well, has a very quick turnaround time.

We don't necessarily see the same considerations in that setting that we see in the setting of new drugs nor are we likely to see the availability of the devices to be used in a preapproval way like we might have.

At least in certain circumstances, you have drug supply preapproval that could be lotteried out in many cases to patients who want to get access to it on an expanded access basis. In the medical device setting, you typically would not have excess medical device supply.

Ms. Eshoo. I don't understand your answer. [Laughter.]

Do you -- are you saying that you don't think it is necessary to include devices to drugs?

Dr. Gottlieb. We don't -- we don't see the same -- I don't see the same concerns in part because the compassionate use program on the medical device side house -- of the house is working well and I also would say I don't see the same opportunity for patients because to the extent that I've said that the supply of the drugs is constrained preapproval the supply of devices preapproval is even more constrained.

Ms. Eshoo. Thank you very much.

Thank you, Mr. Chairman.

Mr. Burgess. [Presiding.] Gentlelady yields back.

The chair recognizes the gentleman from Kentucky, Mr. Guthrie, five minutes for questions, please.

Mr. Guthrie. Thank you very much, and I will ask a question, Dr. Gottlieb. Thanks for coming. I appreciate you being here.

Kind of the scenario that my friend from Texas, Mr. Barton, gave when he said that his brother or people get to the point where the doctor said everything conventionally has been done for you -- there's nothing else we can do for you and then you have the right to try, and that's a traumatic time. I know it is a traumatic thing and people are looking at opportunities and that's what's available for them.

So they choose to go to the experimental side -- the unproven

side -- and agree to pay for that treatment. So but what's unclear in the legislation is what if -- and I quote, you said that you could get unwanted side effects.

So what if the unwanted side effects creates a whole series of health -- puts them back in the hospital? And so instead of agreeing to pay what -- an X amount for some kind of treatment all of a sudden there's new hospital bills that could be astronomical that's not looked at in the legislation.

So my question, do you have any insight or opinion on how to best examine or solve this type of issue?

Dr. Gottlieb. Well, I think it is one of the unknowns associated with using, you know, any product that hasn't gone through a full evaluation where we don't know the scope of the effectiveness of the product, you know, and we don't know -- we certainly don't know the full scope of the side effects.

A phase one -- a product that has gone through a phase one clinical trial -- we call a phase one trial a safety trial but it is a trial for determining safety -- to answer the question on whether or not the study -- the drug can proceed into the next phases of clinical trials doesn't fully establish the safety profile of the product.

We are continuously learning about the safety of a product all through the three phases of a clinical study and, in fact, a lot of what we learn about the safety of products is in the post-market setting.

So there are a lot of unknowns in this setting and we need to be cognizant of that and, you know, patients who use these products through an expanded access program we make sure that they are cognizant of it.

Mr. Guthrie. But my question gets into if they agree to pay for this and then it leads into further medical costs outside of just the experiment that puts them back in the hospital and so forth, which I guess would be part of it, do you have any opinion how that should be addressed?

Dr. Gottlieb. The system -- I would -- I would put that question to my colleague, Seema Verma, at CMS. I mean, this is going to be an issue for the broader health system and for the payers to have to contemplate because the cost would be -- would be born back on the -- on the payer system.

Mr. Guthrie. Okay. Thanks for that.

And I do want to mention just a comment to you while you're here. I do want to mention one more issue regarding the potential threat of glass fragment contamination.

As you may know, the FDA issued an advisory regarding glass fragment contamination for injectable drugs in 2011. I ask that you look into and fully consider updating the advisory to reflect recent discoveries. So no reason for that.

And for my final couple of minutes, I understand -- and I was in a -- there's an Equifax hearing going on downstairs so I was there earlier and I understand you talked about your trip to

Puerto Rico.

I just ran into my colleague on the way up here from the Virgin Islands and, you know, it is very dramatic or very -- very drastic situation and dramatic as well and that's going on there.

And would you just kind of update us on what you're doing to combat potential drug shortages and access to issues that may come as a result of damages. I know your trip to Puerto Rico and also the Virgin Islands.

Dr. Gottlieb. As I mentioned, we have a list of about 40 drugs that we are very concerned about. It reflects maybe about 10 different first. These are drugs -- 13 of them are sole-sourced drugs.

They're only manufactured in Puerto Rico to supply the entire U.S. market and these are -- these are important medicines. These are drugs -- these are HIV medications and chemotherapeutics and injectable drugs that are hard to manufacture.

There's biologics. There's very sophisticated medical devices manufactured down there. The biggest issue right now -- well, there's a lot of issues.

One is getting gasoline and basic sustenance to employees so they can return to work. People are living in very difficult circumstances there and I met a lot of -- a lot of local resident who work for FDA. But the longer-term issue that we are grappling with and worried about is power supply.

The grid is probably going to be stood back up. They'll

create some micro grids. They won't stand up the whole electrical grid. They'll create micro grids.

But the challenge for the manufacturers is that they need stable power and typically they need dual feeds coming in because of the equipment that they use. And we know that the grid is going to be unstable for a long period of time.

In fact the power company would like to reconnect the manufacturing facilities because as they bring up the power system they need load balance and the manufacturers are regular users of power. But manufacturers want to stay off the grid right now.

And so they are going to be operating for long periods of time, potentially, on their generators -- generators that were never meant to operate for months and months on end.

So they don't have necessarily the fuel Thanks to do it and they might not have generators that are up to that challenge.

And so we are trying to trouble shoot that with them on an individual basis now and trying to put in place contingencies if things do go wrong and backups if we need them.

We've been doing that manufacturer by manufacturer, working very closely with DHS and the staff of the governor of Puerto Rico, who we are now in personal contact with who understands the implications and the importance of this manufacturing base not just for all of the United States but for the island of Puerto Rico as well.

Mr. Guthrie. Thank you very much. I appreciate your

efforts.

Mr. Burgess. Chair thanks the gentleman. The gentleman yields back.

The chair recognizes the gentleman from Maryland, Mr. Sarbanes, five minutes for questions, please.

Mr. Sarbanes. Thank you, Mr. Chairman.

Thank you, Dr. Gottlieb, for your testimony here today. I am sort of picking up on the line of questioning from Congresswoman Eshoo in terms of trying to understand what the piece of this process that you would view -- that one would view as broken when you've got over a 99 percent rate of responding to these requests for approval.

And, obviously, there is a constituency out there that feels that notwithstanding what the FDA is doing in its efforts to respond to these inquiries and requests that there's still something more than can be done in terms of accessing expanded treatment options.

So maybe -- could you give me the 30-second caution that FDA -- sort of a digest of a lot of what you've been saying -- the caution you would give us as we are examining and reviewing and debating the pieces of legislation that kind of stimulated the hearing today and that we got some testimony about at the outset?

From your perspective, what would you just say to us -- here's what I would look out for, be cautious about as you're examining the kind of Right to Try legislation that's being proposed?

Dr. Gottlieb. Well, I would just say, you know, you asked about the -- and I know I am limited to 30 seconds. You asked about the obstacles.

You know, one of the obstacles is are patients informed and we are trying to do all we can to make sure they are informed of these opportunities.

The other obstacle I talked about was -- which is just the supply. That's harder to fix. I think there's a perception that this legislation will create more pressure on companies to offer the drug so that might create more pressure on the companies to have a supply available.

I think that's an open question. I think that's something Congress should contemplate.

But in terms of the question of the caution, you know, in addition to the technical assistance we've provided that is more detailed about legislative language, I just would be mindful that we don't create a process and a policy framework where the only people who take advantage of the avenue are people who have the least promising products.

I think what we want to -- what we want to do is create a framework where the most promising products are being made available to patients and this doesn't become sort of an opportunity for those sponsors or maybe even individual clinicians who want to do some advanced marketing of a product to use this vehicle.

And I am not saying that this legislation will do that. I am just saying if I was providing feedback to Congress of what to be mindful of, that's something that I would caution Congress around.

Mr. Sarbanes. Well, actually I appreciate that because you've led -- in your answer you've gone right to the place that I have some anxiety about, which is the potential to create something that may start small but would grow as a kind of unregulated space and that once established as a kind of alternative route, not just for patients that are genuinely seeking whatever option is available to them but for manufacturers as well, it becomes a kind of alternative space in which to operate and then it could be vulnerable to some unscrupulous activity, in a sense creating a place where the opportunity to experiment with experimental drugs is expanded and that's what makes me a little bit nervous.

So in the one minute that's left maybe you could speak to that.

Dr. Gottlieb. Well, I would -- I would build on it by saying you said -- used the word manufacturer. I would build on it by saying it is not just the manufacturer.

We recently took regulatory action against two clinics that were marketing unapproved products as regenerative medicine. In one case, we had U.S. marshals seize a product that we felt was creating certain public health concerns, and I won't get into the

details of it today since it is an ongoing activity.

But there's also going to be individual providers who potentially could promulgate products under this -- under this framework and one of the -- one of the elements of feedback that we've given to Congress through our technical assistance is to make sure that the patient protections that Congress intended to be available under this legislation are also available to patients who are getting products directly from physicians or physician-operated clinics and not just manufacturer because the way Congress crafted the draft legislation it could be interpreted in certain settings as those patient protections only applying to products promulgated by manufacturers, by sponsors, and in fact under this legislation it will also be providers who are promulgating products.

Mr. Sarbanes. Okay. That's very helpful. Thank you.

Mr. Burgess. Gentleman yields back. The chair thanks the gentleman.

The chair recognizes the gentleman from New Jersey, Mr. Lance, five minutes for questions, please.

Mr. Lance. Thank you, Mr. Chairman.

Good morning to you, Dr. Gottlieb. The legislation passed unanimously in the Senate. Were you involved in that or was it a situation with your -- with a predecessor?

Dr. Gottlieb. That happened on my predecessor's watch.

Mr. Lance. And it is unusual -- not unique but it is unusual

when legislation passes unanimously in the Senate I think that would be fair to say and from your perspective, reviewing it, knowing that you were not then in charge, why do you think that this legislation passed unanimously in the Senate?

Dr. Gottlieb. Actually, I'll reopen the record to say I am not sure the date that it passed. It might have been on my watch but --

Mr. Lance. Fair enough.

Dr. Gottlieb. -- so we'll just -- we'll leave it open.

Mr. Lance. But you were new to your responsibilities. I understand that.

Dr. Gottlieb. But, look, I think as I've stated in my comments here today, this touches on a very important issue and it touches on an issue that I think is very visceral for most Americans.

We have all -- most of us have seen loved ones, unfortunately, or friends succumb to serious illness and in certain situations we've seen them do that in a setting of feeling like they didn't have good options to try to beat back -- beat back a serious illness.

And so, you know, the idea of being able to get access, we are seeing all this new technology, all these extremely promising drugs and development.

We are seeing the potential to fundamentally cure pediatric inherited disorders through things like gene therapy and

regenerative medicine.

With all this technology coming online, I understand the desire of people who are -- who are stricken with the disease now to want access to that. I think that this -- I think this phenomenon is being driven in part by the opportunities we have available to us now.

Mr. Lance. Thank you. As a matter of full disclosure, since you have kindly indicated you might technically have been in charge but certainly the bulk of the work in the Senate was before you were in charge, I have worked with Congressman Fitzpatrick.

The district that I am honored to represent borders his district although we are in different states, and I've worked with Mr. Worthington, who is in this room, on this very important issue.

The FDA may place a clinical hold on a drug. If, for example, human volunteers are being subject to unreasonable and significant risks of illness or injury, has the FDA placed a clinical hold on a drug as a result of an adverse event during an expanded access protocol?

Dr. Gottlieb. I don't know the answer to the question. I would tend to think not, just given the numbers of situations where we've recognized adverse events in the setting of an expanded access program that have led to any kind of regulatory decision.

You know, we've done some systematic looks back and found very few instances where something we observed in the setting of

an expanded access program has prompted us to take certain regulatory actions.

Certainly, the inverse case where we have -- you know, I think I mentioned previously a large percentage of the very small number of cases where we might deny a patient a request for -- to use a drug in an expanded access setting is predicated on the existence of a clinical hold that might not be known to the public because it is commercially confidential information.

Mr. Lance. Thank you. If you would, at your convenience could you get back to us, to the subcommittee, on whether or not that has occurred? I would appreciate that.

Dr. Gottlieb. Sure.

Mr. Lance. Thank you.

This is a very difficult issue and I certainly understand your point of view. I think there are many of us in Congress who are sympathetic to what occurred in the Senate and, certainly, sympathetic to the legislation of our colleagues who testified, to my -- to my immediate right and we want to continue to work with you.

But, certainly, I believe there is merit to the legislation that's being considered.

Thank you for your testimony, Dr. Gottlieb.

Dr. Gottlieb. Thanks a lot, Congressman.

Mr. Burgess. Chair thanks the gentleman. Gentleman yields back.

The chair recognizes the gentleman from Virginia, Mr. Griffith, five minutes for questions, please.

Mr. Griffith. Thank you very much, Mr. Chairman. Thank you, Dr. Gottlieb for being here with us today.

In testimony earlier today you were talking about the definition of terminal. I would note that neither House bill, neither Mr. Fitzpatrick and Mr. Biggs' or mine goes the step beyond terminal -- that the Senate went and I can appreciate that. Then we got into, you know, what the definition of terminal ought to be.

I am happy to work with you all on that. I think, if I remember correctly, and you correct me if I am wrong, that you indicated somewhere around 20 percent survival odds was where you would probably put it. I'd probably push it a little higher.

Dr. Gottlieb. I wouldn't. I didn't --

Mr. Griffith. I misunderstood --

Dr. Gottlieb. -- mean to suggest that there is a -- there's an objective -- objective figure. I was just using the example of a patient who's given a very grim prognosis. I would certainly consider 20 percent odds of survival grim.

Mr. Griffith. And I would, too, and I think that's where this is coming from. I might push that a little higher. Anything less than 50/50 -- you know, if it were me I'd want to be able to find out what was out there.

Dr. Gottlieb. It was -- it was grim when I was told it was

90 percent odds of living five years. That felt pretty grim at the time, too, Congressman.

Mr. Griffith. Yes, sir. I can appreciate that.

So I want to work with you on that but I do think that we need to pass something and we'll try to figure it out.

But I can see where you're concerned about chronic -- you know, FDA was created in 1906 to protect Americans, not to get in the way of them taking treatment and I will -- if it were to be me and I -- right now I am fine.

But as Ms. Eshoo said, we are all one diagnosis away from facing something. I would take the chance with the silver bullet or the Hail Mary, and we are going to hear testimony later today that the -- some wealthy Americans are going to other countries to get treatments or to get drugs.

And so my question would be if it has already been approved somewhere else and you have a terminal diagnosis, why shouldn't you be able to get that in the United States?

Dr. Gottlieb. Well, I think you're touching on the issue of reciprocity, which is -- which is some legislation that has been introduced in other -- other settings, whether or not FDA should predicate approvals here in the U.S. on the basis of foreign approvals.

And we are certainly happy to work with Congress on those legislative ideas. The framework that we operate in right now is a requirement that we determine safety and efficacy based on

our statute and clinical trials that we work with sponsors to conduct and evaluate.

You know, another element of this consideration is also relying more on foreign data, which we are doing as a matter of regulatory policy and that is something we can do without new legislative authority.

We can do that within the constructs of our current regulatory considerations and we are looking for ways to do that.

Mr. Griffith. And I appreciate that and I appreciated your comments earlier about some of the new things that you're doing and that you're announcing today and I appreciate that as well.

You know, the GAO report found that when the FDA did not allow a request for expanded access to proceed one of the reasons listed was due to the requested drugs demonstrated a lack of efficacy for its intended use.

But what if data showed that the intended use -- it may not have been the intended use but that it actually had benefits that were unexpected in another area and you're facing that terminal illness that it does have the benefit for. I am just curious how the FDA would deal with that in its current process.

Dr. Gottlieb. Well, most of the cases where we are authorizing or, you know, allowing drugs to be used, in 99 percent of the cases where we -- where we allow patients to use drugs in the setting of expanded access it's in a setting that is not the intended use of the product -- that they'd be used for which the

drug itself is being studied.

So it's in an unstudied indication or an indication that might be being evaluated in very small clinical trials.

When we -- when you -- when the GAO says that it was something when we didn't allow it to be used and something unproven because of something we knew, typically it was something we knew because we get a lot of clinical data from a lot of different sponsors and we might know that a drug in a certain class doesn't work in that class because of other data that we are seeing.

And so then we might make a judgment that we shouldn't allow that drug to be made available in a setting of expanded access when we have objective proof that it's not going to provide any benefit.

I mean, bear in mind, we know that 70 percent of all drugs that are offered in an expanded access are never approved by FDA. So the vast majority of people who will use a drug through expanded access are using a drug that doesn't work.

Mr. Griffith. And I appreciate that. I am running out of time so I am just going to make one last comment. I do think that the two House versions both have device in there.

I think we should keep that because, as you said, science is moving fairly quickly. That's one of the reasons that people want to try these things before you all have had a chance.

There's some wonderful things out there with science. Again, if I had a diagnosis with inoperable cancer and they had

a new nanobot technology, I'd be finding out where I could get that, and that is considered a medical device and it may be very, very helpful.

Not ready yet, but if it were when I was ready or needed it, I'd want to be able to use it.

Thank you so much for your time, and I yield back.

Mr. Burgess. Gentleman yields back. The chair thanks the gentleman.

The chair recognizes the gentleman from North Carolina, Mr. Hudson, five minutes for questions, please.

Mr. Hudson. Thank you. Dr. Gottlieb, thank you for being here today with your testimony.

FDA categorizes expanded access and the four different types of requests, as you're aware, are single patient, single patient emergency, intermediate size, and treatment for widespread populations.

While the standard process seems to get a lot of attention, I'd like to ask more about the intermediate size and treatment for widespread populations.

How are these two types of requests separate and unique from the larger clinical trial?

Dr. Gottlieb. Congressman, we could get you more detailed information because there's a spectrum of opportunities.

It is the case that, for example, and I think you mentioned this -- when a drug is -- in the period of time when it's completed

its clinical trials but is awaiting approval decisions, companies will open up large expanded access programs typically like simple large protocols and offer drugs on a protocol basis.

I think that these are -- these are important opportunities because what we are talking about today, a one-off request for a drug -- an individual patient and their doctor working with the agency to ask for a drug in a single situation.

I think what we'd like to see is more opportunities to offer products in things like simple large safety trials and certain simple protocols where patients aren't being randomized but some basic information is being collected that can help inform -- inform what we know about that product but also provide for more wide scale access.

And this gets into a broader question around how do we embrace different clinical trial designs and if we can go down these routes we can come up with constructs I think can enable much broader access preapproval.

Mr. Hudson. Makes sense. Are these patients incorporated into the broader clinical trial population for the purposes of data collection and efficacy?

Dr. Gottlieb. Sometimes. Sometimes we are collecting data from these kinds of protocols. Sometimes we are not.

I think to the extent that we can get into collecting more data and being able to make efficient use of that data it can help accelerate the development process.

So this is something, you know, that we are looking at when we talk about seamless clinical trials. You know, we talk about allowing the study of different indications within the confines of a single clinical trial.

These are all some of the new scientific frameworks that we are looking at to try to -- try to evolve how we do clinical trials and I think can both allow us to get better information and make the development process itself more efficient but also enable larger, more access to drugs preapproval and in some kind of clinical trial where there is -- where there is good protections being afforded to patients as well.

Mr. Hudson. Have there been any cases where patients have been denied access to a clinical trial but received access through an intermediate size or treatments for widespread populations as a result of the expanded access program?

Dr. Gottlieb. Oh, I am sure there has, Congressman.

Mr. Hudson. Is the expanded access program alone adequate to address the needs of patients and physicians who are seeking to obtain investigational drugs?

Dr. Gottlieb. Well, I don't think we'd be here today if there was a perception by Congress and the broader community that the existing system was adequate.

And I am not going to tell you that the existing system is perfect. That's why we announced a set of changes today and that is why, as part of that announcement, I committed to do additional

things down the road that -- some of which we are working on right now to help continue to improve that process.

Mr. Hudson. Great. We look forward to working with you on that.

Dr. Gottlieb. Thanks a lot.

Mr. Hudson. With that, Mr. Chairman, I will yield back.

Mr. Burgess. Chair thanks the gentleman.

The chair recognizes the gentleman from Georgia, Mr. Carter, five minutes for questions, please.

Mr. Carter. Thank you, Dr. Gottlieb, for being here.

Help me understand, basically. We are talking about two different scenarios here. We are talking about drugs that have been approved already by the FDA for something but what they are wanting to be used for is not an indication so physicians are trying to use it off label, if you will.

And we are also talking about investigational drugs that have not been approved yet but are in the pipeline and is -- am I right in that?

Dr. Gottlieb. Well, I think that you're right that those are two constructs that exist for patients to get access to unproven therapy.

I was a patient who used an approved product in an off-label fashion and that is actually typically what you see in these settings.

You'll see products used -- especially oncology you'll see

products used off-label. I think what we are focused on with respect to the legislation here, respectfully, is the second scenario that you offered, which is a product that hasn't yet been approved by the FDA but patients want to use it in an experimental or investigational way.

Mr. Carter. Okay. It's my understand the FDA -- your responsibility is to protect the public from any side effects, any bad effects that a medication may have but also to make sure that it's available if it could benefit the public as well. Is that correct?

Dr. Gottlieb. Well, I think the scope of the FDA's mission is broader. I think the scope of our mission and our responsibility to patients is much broader in this context.

I would -- I would tweak it by saying I think our responsibility is to make sure that patients and providers are fully informed of both the risks and the benefits in these settings.

Mr. Carter. Okay. Having said that, can you explain to me why the FDA keeps putting their head in the sand when it comes to medical marijuana?

I am not -- and I don't want to hear marijuana is a Schedule One drug for investigational use only. But here we have -- I don't know how many states we are up to now -- that have approved it.

Here we have all these states and most of them with a different strength of what they've approved, and yet the FDA just

continues to ignore that.

Isn't it your responsibility to address that?

Dr. Gottlieb. Well, I see people who are developing products based on marijuana, making all kinds of clinical claims on the market.

I see people who are developing products making claims that marijuana has anti-tumor effects in the setting of cancer, and I think reasonable people can ask reasonable questions about whether marijuana is a chemotherapeutic agent.

So, you know, it's a much broader question, Congressman, about where our responsibility is to step into this and start to ask questions about the claims that are being made.

Mr. Carter. And that is my question. Where does your responsibility come in? It would appear to me, when you've got all these states that are approving it, it would appear that the FDA should be stepping in to give some kind of consistency here.

Dr. Gottlieb. Well, I think that we'll have some answers to this question very soon because I think we do bear a responsibility to start to address these questions.

Mr. Carter. Let me ask you, the bills that we are considering today how will that change your approach? Will it change your approach at all? Will it change your role in the process at all?

Dr. Gottlieb. If these bills are passed, we look forward to working with Congress to make sure that they are faithfully

implemented.

It will -- it will open up a new vehicle for patients to potentially get access to certain therapies. I think the question that I outlined throughout my testimony today still remains about whether or not sponsors will offer these opportunities on any -- on any greater basis and whether or not this legislation alone is enough to compel sponsors to have supply available to offer products more generously on an expanded access basis.

I think that those questions remain unanswered. I don't have an answer to those questions.

Mr. Carter. Okay. Two more things, real quick.

First of all, you've read over the legislation, I assume, that is being proposed?

Dr. Gottlieb. Certainly.

Mr. Carter. Is there any part of it that you think that the FDA potentially could have trouble because what -- understanding or implementing because what I don't want to happen is to have legislative intent interpreted by the agency when that is not what we were intending to do?

Dr. Gottlieb. Well, I mean, I've outlined some of the -- some of the places --

Mr. Carter. We've had that experience before in other areas.

Dr. Gottlieb. Right. Well, look, and legislation can be

interpreted differently by different FDA commissioners as well, as you're well aware.

I have outlined some of the areas where we think that there might be ambiguity in the current language right now where Congress might take closer consideration of how certain things are crafted and how certain things are worded to potentially tighten this up.

And we have tried to be constructive. We will continue to try to be constructive and work with Congress if this legislation does advance.

Mr. Carter. Okay. Once last question -- I just -- you know, and I go back to my question at the beginning -- I am to understand your answer about medical marijuana is that FDA is going to be addressing that situation very soon?

Dr. Gottlieb. Congressman, you know, the question is should we be taking enforcement action against people who are making certain claims in the market? Because I don't necessarily understand your question. We don't have -- we have 20 --

Mr. Carter. My question is simple. Why does the FDA continue to ignore medical marijuana when we have states who are approving it?

Dr. Gottlieb. Well, this --

Mr. Carter. We have states who are going -- who are actually taking on the responsibility of approving medications.

Dr. Gottlieb. We have two frameworks that we operate in.

One is sponsors who bring us applications requesting that we approve a product for a certain intended use. We have 20 INDs and active INDs in house right now that are for marijuana products.

They are typically for marijuana extracts because delivering an active pharmaceutical ingredient through inhalation isn't always the most efficient route.

The other question that gets to your question is whether or not there are certain claims being made in the market by people who are marketing marijuana in interstate commerce that are unapproved new drug claims and could potentially put people at risk. That's a separate question.

I think that we are addressing -- we will address the sweep of these questions in time, including the questions put before us from sponsors that have 20 INDs.

Mr. Carter. If you can -- can I get some kind of idea of when you're going to address this?

Dr. Gottlieb. Well, we have 20 INDs in house and so we are addressing those as part of our review process.

Mr. Carter. Do those 20 INDs have all these states approving them already?

Dr. Gottlieb. These are -- these typically are sponsors who are putting products through -- trying to put products through a scientific process and not just marketing it on a website.

Mr. Burgess. And the chair would advise there's likely to be a multi-agency approach to this. It is not going to be

exclusively through the Food and Drug Administration.

Mr. Carter. And that is well understood. But, certainly, they have a role in it that I feel like they are ignoring.

Mr. Burgess. And the gentleman's time is expired and the chair will recognize the gentlelady from Colorado, Ms. DeGette, five minutes for questions, please.

Ms. DeGette. Thank you very much, Mr. Chairman. Tempting though it may be to follow up on this medical marijuana, being from Colorado, I want to talk to you --

[Laughter.]

Ms. DeGette. -- broadly about the current safeguards that are in place under the FDA's expanded access program to protect patients.

Can you please describe those for me?

Dr. Gottlieb. The safeguards that we have in place with respect to patients who get products through our current --

Ms. DeGette. Right.

Dr. Gottlieb. Well, the requests come into to FDA and we are asked to evaluate them, and we do go through the protocols and make certain assessments and in certain cases we provide feedback to the providers.

As has been stated here, we grant over 99 percent of the requests. But there are about 10 percent where we make certain modifications to protect patient safety and the most common modification that we'll make is to give feedback to adjust the

dose and that will be on information that we might have about what the -- what the most potentially beneficial dose of the product might be.

Another modification that we'll oftentimes make is on the informed consent. Sometimes the consent that is being provided to the patient might not be comprehensive. And so we'll ask for modifications to be made to the informed consent.

So that gives you a flavor of the kinds of protections that we think we are providing by being part of this process and part of the evaluation.

Ms. DeGette. You know, as you say in your testimony, the Senate Right to Try legislation tries to apply some of the protections to investigational product use under Right to Try but it doesn't make clear that the requirements apply to all individuals who might provide a drug under Right to Try.

Can you explain how this loophole might be exploited?

Dr. Gottlieb. Well, I think you're referring to the -- how the legislation currently tries to map to existing regulations in terms of importing some of the existing patient protections that exist in regulation to apply to patients in the setting in one version of the bill.

The way we interpret it there is the potential that as a matter of law you could interpret the regulations that exist as applying to sponsors, companies, and I think what we are likely to experience in the setting of Right to Try, if we look at some

of the anecdotal experience in the states -- and right now we only have anecdotal experience because we don't have any data about the availability of drugs that have been provided through these Right to Try law -- but it is -- it is possible that it will be the case that some of the products that will be offered under the framework contemplated by this legislation will be offered by individual sponsors or small clinics that might not qualify as a sponsor for purposes of the way the regulation is currently crafted.

Ms. DeGette. Yes. Thanks.

Now, as I understand it, of the 99 percent of requests for expanded access that FDA has approved, the agency proposed changes in 10 percent of the applications to ensure patient safety either through dosing changes, informed consent, or safety monitoring.

Under the Senate-passed legislation, the FDA review of INDs would no longer be required. Can you talk to us a little bit further about how -- about what you see the FDA's role in reviewing these INDs and whether it protects patients -- whether under this new legislative paradigm some things could potentially be missed because the FDA is not reviewing it?

Dr. Gottlieb. Well, we certainly believe that we are helping to provide additional safeguards and protections to patients. I think we would state very strongly that we also think we are providing additional opportunities to patients because, you know, in terms of -- you mentioned the issue of the dose

adjustments.

Sometimes we will request dose adjustments because we might have information to suggest, based on other trials ongoing that we are looking at, that if there is a benefit to be derived it would have to be a higher dose or it might have to be a lower dose.

And so we are making adjustments to help maximize the opportunity for the patient to derive a benefit and not experience a side effect.

Ms. DeGette. So what I am hearing you say is, is the agency is really concerned about making sure these -- that the dosages are correct and all of that.

You're not really trying to use this as a barrier to people getting much-needed medication for some of these diseases.

Dr. Gottlieb. Well, I think statistics speak louder than anecdote and if we are granting well over 99 percent of these requests, both the emergency and nonemergency requests, the agency -- the agency's process once a patient walks up to the door and is able to walk through that process, that process where we are applying a level of review is not in and of itself a barrier.

I mean, the numbers demonstrate that. I mean, the question is are patients able to walk up to that door and that's where we are making reforms and trying to put in place new tools like the Navigator to get more patients into that door.

Ms. DeGette. And you're open to more requests like that?

Dr. Gottlieb. We absolutely are.

Ms. DeGette. Thank you. I yield back.

Mr. Burgess. Chair thanks the gentlelady. The gentlelady yields back.

And recognizes the gentlelady from Indiana, Mrs. Brooks, five minutes for questions, please.

Mrs. Brooks. Thank you, Mr. Chairman, and good to see you, Dr. Gottlieb.

I want to continue to discuss briefly about the expanded access program but then also I want to make sure we spend a little bit of time just talking about your recent trip to Puerto Rico.

But with respect to the expanded access and the FDA's desire to increase the requests and so forth, of the 99 percent of the requests made for expanded access and which are approved, I understand that only about 30 percent of those therapies actually make it through the full clinical trial process.

And so what are the steps a manufacturer has to then go through to proceed on with the clinical trial when they are including the expanded access?

Dr. Gottlieb. Well --

Mrs. Brooks. How does it impact their clinical trials?

Dr. Gottlieb. We would say it doesn't impact the clinical trials and, you know, one of the questions has been does -- could something observed in the setting of an expanded access program where you have drugs being provided in a more unstructured way, typically by physicians who might not be as familiar with the

product itself -- could something -- could an observation made in that setting go on to help delay the development process and that's always been argued to be something that causes manufacturers' reluctance to offer these.

We would say no, and what I would say simply in response to your question is these two things can exist in parallel and they do exist in parallel.

Companies will offer drugs on an expanded access basis and they'll have an ongoing clinical development program. The question for the sponsor -- and I mentioned earlier I have been on the other side of this working with small biotech companies before coming into this position -- the question for the sponsor is just the ability to both service the expanded access program -- these oftentimes are small companies -- but also have the product available -- also have the supply.

Mrs. Brooks. And then does the process -- how do the results from the expanded access -- are they included in the data and the findings in the clinical trials or are they in a little separate set of findings?

Dr. Gottlieb. Well, we don't sequester the information but what we've said today in the guidance that we promulgated and what we've observed when we've gone back and looked at this systematically is typically the information, if there is any information to be gleaned from the expanded access program, doesn't have an impact on the development program one way or the

other.

We found very few situations -- we looked at 321 regulatory approvals over a 10-year period. There were 28 -- 28 percent of the drugs had expanded access.

We could find only two instances where something observed in the expanded access setting informed the drug approval.

In one case, it led to labelling around a certain safety issue and in one case it actually helped us approve the drug by helping to augment the information we had about the effectiveness of the product.

So it is atypical, very atypical, that information gleaned in this setting would impact the drug approval and the guidance we put out today is sort of doubling down on our assertion that it is atypical.

We are saying it is very, very atypical that we would consider something in that setting in part because these settings are very unstructured and the patients are very sick.

Mrs. Brooks. Thank you. I would like to turn to Puerto Rico and thank you for making the trip to Puerto Rico and, obviously, manufacturing over 50 pharmaceutical facilities -- as you said, thousands of employees producing treatments for cancer, HIV, immunosuppressants, and so forth.

Has the agency ever faced this kind of challenge before after a natural disaster and has FDA ever dealt with something with this much impact, with this many companies ever being impacted?

Dr. Gottlieb. I, certainly, have no recent memory. I've been around the agency for 15 years either as an observer on the outside doing policy work or working for three separate commissions.

I've never seen something on this scale where we've had -- where we've had a region that had so much concentrated -- important concentrated manufacturing impacted in such a profound way.

I mean, our priority first and foremost is to the people of Puerto Rico and we are doing a lot to provide them direct assistance. But this is an existential risk that we face as a -- as a nation if these facilities are permanently impacted.

And I will just state that the facilities themselves are intact.

Mrs. Brooks. Right.

Dr. Gottlieb. The challenge is going to be the logistics of maintaining their operations and moving -- getting their workers to work, maintaining their operations on what are right now generators and in moving product off the island.

The issue of moving the product off the island is improving. The getting the workers to work is starting to get better.

The companies themselves have done a lot to provide direct assistance to their employees. They are opening the cafeterias. They are offering three meals a day, providing gasoline to their employees. I've been on the phone with many of these CEOs.

My biggest long-term concern right now is the power and also

the secondary supply chain -- are they going to be able to get supplies from their local suppliers who we are not necessarily monitoring as closely and they might not be FDA-regulated facilities.

Mrs. Brooks. My time has expired.

However, I did wonder, since we haven't dealt with this, might there be protocols to be put in place in the future in case anything like this were to happen, unless there are already protocols in place to work with these manufacturers to mitigate the shortfalls?

Dr. Gottlieb. There -- there are, and these are hardened facilities that have substantial generators -- I mean, 800,000 kilowatt generators on some of these facilities, bigger than that.

I don't think anyone anticipated something on this scale where the -- where a category four hurricane went through the longitudinal access of the island and decimated the entire island.

Mrs. Brooks. Thank you. I yield back.

Mr. Burgess. Chair thanks the gentlelady. Gentlelady yields back.

Dr. Gottlieb, if I could, let me just ask you, on the guidance that you're going to be providing does it address the issue for someone who has been on -- someone who's been on a critical trial, the drug is not approved, and yet the perception of the patient is this is the only thing that helped me, and so now that product is not going to be available? Would that be available under an

expanded use?

Dr. Gottlieb. Well, I don't -- it depends on is it a circumstance where the company made a decision not to go forward with the further development of product or the company is continuing to develop the product and then they are going to provide it subsequent to the clinical trial in sort of an open label fashion.

In the latter circumstance, we see a lot of companies doing that. In the former circumstance, when companies do curtail development of products in clinical trials it's because they've deemed them not to work but certain patients felt they are deriving a benefit, this doesn't address it.

I think it would take something that Congress would have to do to address that kind of a circumstance.

Mr. Burgess. Very well. Good to know.

I do want to thank you for being here and your indulgence through the testimony and the questions.

We are going to transition without a break to our third panel, and we are ready to hear from Mr. John Dicken, director of health care in the United States Government Accountability Office.

Dr. Dicken, we'll give you a moment to get situated and then you'll be recognized for five minutes whenever you're ready.

STATEMENT OF JOHN DICKEN, DIRECTOR FOR HEALTH CARE, U.S. GOVERNMENT ACCOUNTABILITY OFFICE

Mr. Dicken. Great. Thank you, Chairman Burgess, and members of the subcommittee.

I am pleased to be here today to discuss GAO's recent report on FDA's expanded access program. As you have been hearing this morning, this program allows patients with serious or life-threatening ailments and no other comparable medical options to obtain access to investigational drugs and biologics -- that is, those that are not yet approved for FDA marketing.

FDA receives and reviews these expanded access requests and determines whether to allow them to proceed. It's also important to note that other entities also have roles.

For example, manufacturers decide whether to give patients access to their investigational drugs, Institutional Review Boards must approve their investigational access treatment plans, and physicians treat the patients with the investigational drugs and monitor their progress.

My testimony today briefly highlights three key findings from our July report. First, I will speak about what is known about the number, type, and time frames of expanded access requests received by FDA; second, what actions FDA and other stakeholders have taken to improve expanded access; and third, how FDA uses data from expanded access in the drug approval

process. In addition, I will highlight a recommendation we have made to FDA to improve the program.

First, we found that FDA allowed to proceed nearly all, 99 percent, of the nearly 5,800 expanded access requests that were submitted from fiscal years 2012 through 2015.

Almost 96 percent of these requests were for single patients with more than 2,400 requested on an emergency basis. FDA typically responded to these emergency requests within hours and responded to all other requests within 30 or fewer days.

In the rare cases when FDA did not allow a request to proceed, the most common reasons were incomplete applications, unsafe dosing, the treatments demonstrated lack of efficacy, or the availability of adequate alternative therapies.

We also found that FDA and others have taken steps to improve patient access through this program. For example, in response to concerns that the process to request expanded access was cumbersome, FDA simplified its website, guidance, and forms.

Efforts by other stakeholders include a project to educate and streamline the process by which Institutional Review Boards approve treatment plans for expanded access use and the creation of an advisory group to help drug manufacturers manage expanded access requests.

Finally, we examined FDA's use of safety reports based on the use of drugs allowed through expanded access. Manufacturers sponsoring clinical trials included -- including any expanded

access use must submit safety reports to FDA that include adverse events data.

FDA reported using adverse events data from expand use in a few cases during the drug approval process but not more widely because expand access use does not have the same controls as clinical trials.

For example, FDA data show that there were only two instances from 2005 through 2014 in which adverse events from expanded access use contributed to FDA delaying a drug's development by imposing a clinical hold on the drug's use.

However, several manufacturers and other stakeholders we interviewed raised concerns that FDA is not consistently clear about how it uses expanded access adverse events data during the drug approval process.

Our review of documents that FDA uses to communicate with drug manufacturers about expand access found that only one included a reference to FDA's use of these data.

Manufacturers know that this lack of clear information can influence their decision whether or not to give patients access to their drugs.

Based on this finding, we recommended that FDA should clearly communicate how the agency will use adverse events data from expand access use when reviewing drugs and biologics for approval.

FDA agreed with our recommendation and I was pleased to hear FDA Commissioner Gottlieb announce this morning new guidance in

response to GAO's recommendation.

We believe that this additional clarity could help allay manufacturers' concerns and help meet the goal of FDA facilitating expanded access to drugs for patients with serious or life-threatening conditions when appropriate.

Chairman Burgess and members of the subcommittee, this concludes my statement. I will be pleased to respond to any questions you may have.

[The prepared statement of Mr. Dicken follows:]

**********INSERT 5**********

Mr. Burgess. I thank you for your testimony.

We will move into the question and answer portion of the hearing. I would like to recognize Mr. Guthrie from Kentucky five minutes for his questions, please.

Mr. Guthrie. Thank you very much. Thank you, Mr. Dicken, for being here. Appreciate you being here today.

And what are some of the ways, like the 14 other stakeholders you spoke with -- what are the ways they are working to improve the expanded access process?

Mr. Dicken. Right. We heard of several efforts that were ongoing. Certainly, some deal with the transparency and education about the program.

There's an effort by the Reagan-Udall Foundation that's working with FDA to create a Navigator that will allow more information.

Certainly, the effort by this committee and Congress and the 21 Century Act make legislation that requires manufacturers to include information about their policy for expanded access also adds two kinds of transparency in the information that's available.

There are other efforts that we heard about that deal with streamlining the Institutional Review Board process or having assistance from manufacturers' pilot program.

I think a witness in the last panel will speak more about efforts to help manufacturers consider and manage these types of

requests.

Mr. Guthrie. Okay. Thank you. And you got sort of to the answer of this next question but I am going to ask it again and give you a chance to elaborate at the very end of your -- of your comments there.

It says your report found the FDA does not consistently and clearly communicate how it uses adverse effects data from expanded access used in the drug approval process.

Can you please summarize how FDA communicates currently, which you sort of did, and how GAO recommends they change? If you can elaborate again.

Mr. Dicken. Sure. We -- during the course of our work, we reviewed a range of materials that FDA provides to communicate with manufacturers and others about the expanded access program. That includes various guidance documents as well as acknowledgments when they are expand access requests.

Across those multiple documents we found that FDA had updated one that provides some general information. They had a question and answer that provides some information at a general level about how they would use adverse events data.

But we continued to hear from drug manufacturers' and others' concerns that was not as consistent or as clear as possible.

So we were pleased that FDA did agree with the recommendation and, certainly, Commissioner Gottlieb's testimony in the case that they intend to clarify more guidance, going forward.

Mr. Guthrie. Okay. Thank you.

And your report indicates that FDA allowed 99 percent of expanded access requests they received to proceed. Did you look at the reasons for why FDA would not allow a request to proceed and if so, what did you find?

Mr. Dicken. Yes. We looked at FDA's data on why. They indicated that they did not allow the exceptions -- the 1 percent that did not proceed.

The types of issues that FDA indicated were either the FDA had identified that there was evidence that was ineffective for the incident treatments -- either that there might have been availability of other treatments including clinical trials that individuals may have been able to participate in or also incomplete information or safety concerns.

Mr. Guthrie. Well, I think Dr. Gottlieb mentioned earlier when he was here that part of the reason was that there was a confidential hold because there were some adverse effects that was identified but wasn't public knowledge.

Did you see that and I guess what I am getting at it appeared that unless there was a specific reason that people weren't getting approved to go into the right -- were getting the right to try unless there was some specific adverse effect.

I think you said -- I think you said two things -- one, there was non-availability. Two was that there was some confidential -- I forget the term that he used but a hold -- that they knew

there was an adverse effect but they couldn't put that out publicly.

And so is that what you found and, you know, it seems like everybody, unless there's a specific reason not to, are getting the chance to try.

Mr. Dicken. And so I think that's a fair characterization -- that it's only in very isolated incidences that they had additional information that may have raised concerns about the safety.

I will note that this is not the only player, that there are other decisions including that they need to have approval from the manufacturer before proceeding with an expanded access request.

Mr. Guthrie. Okay. Thank you.

That concludes my questions and I yield back.

Mr. Burgess. Gentleman yields back. Chair thanks the gentleman.

Chair recognizes the gentleman from Texas, Mr. Green, five minutes for questions, please.

Mr. Green. Thank you, Mr. Chairman, and thank you for being here, and looking at the GAO helps us in looking at what we may do with this legislation.

The report found that some manufacturers expressed concerns regarding how adverse events associated with expanded access would impact the drugs' development or ultimate approval.

GAO recommended the FDA should clearly communicate how it uses adverse events data from expanded use in the drug approval process, a recommendation that FDA just a few minutes ago agreed with.

Can you elaborate on this recommendation and how will greater clarity from the FDA on the use of adverse event data from expanded use improve upon patient access to these investigational therapies?

Mr. Dicken. Yes. This was a concern that as we interviewed manufacturers and other participants in the process that several raised that this was affecting their decision making about agreeing to expanded access requests.

We also heard, and you have heard testimony today, that there have been very rare instances -- only two instances -- when there has been a delay or a clinical hold.

And so that led to the if there were more clarity as to what the circumstances where FDA would consider that information that in many cases, because this information does not have the same controls that a clinical trial has, I think Commissioner said it's often not useful in the drug development and approval process.

But when it is, there was concern and so more clarity that it's only in isolated circumstances and that that would be a concern the appropriate context seemed important to help allay those manufacturers' concerns and hopefully help improve access for patients that could get investigational therapies when

appropriate.

Mr. Green. Okay. Thank you.

A central component of the 2017 report focused on what's known as number type and the time frames of expanded access requests received by the FDA.

The bills we are considering today would take the FDA out of the process altogether. Would it be possible to even know the universe of expanded access or Right to Try requests made absent any FDA involvement and do you think this lack of accountability by a company potentially illegitimate claiming to have an IND expose patients to bad actors.

If the FDA is out of the picture, how do we know the adverse actors?

Mr. Dicken. So on the first part of that about the total universe, you know, we know the data on how many are reaching FDA. We reported on that.

We did also talk to manufacturers -- a subset of nine manufacturers -- with experience in the process and their experiences really varied. There was no consistent data on how much requests they are getting.

But, certainly, they had requests, from dozens to hundreds of requests in some cases, for expand access. But there is not consistent information across all manufacturers of how often they would be getting these requests.

Certainly, under current authority FDA's key part of

developing -- the drug development approval requires a clinical trial and approvals in that process and looking at information from other sources including, where appropriate, expanded access use.

Mr. Green. Okay. And you mentioned in your testimony the use of this data by FDA, while limited, is still a source of concern for manufacturers looking to get their products approved.

Could you elaborate on the concerns expressed in your interviews with manufacturers regarding FDA's guidance on this issue?

Mr. Dicken. Yes. I think the concerns were that if there is uncertainty as to whether or not and how FDA would consider a situation where the therapy doesn't work.

These are terminally ill individuals. There will be outcomes that no one wants but that are negative. And so in the uncertainty of how FDA would consider that information, that led them to have concerns about making some of the approvals.

That's where we think more clarity on the limited circumstances in which FDA does consider this is very important and recognition of the context that these are individuals that are not in clinical trial settings, that are terminally ill, and how -- whether or not that is relevant information that FDA could -- would find useful.

Mr. Green. Thank you. Thank you, Mr. Chairman.

Mr. Burgess. Chair thanks the gentleman. Gentleman yields

back.

The chair recognizes the gentleman from Virginia, Mr. Griffith, five minutes for questions, please.

Mr. Griffith. Thank you, Mr. Chairman.

Let me just say I appreciate your report. I appreciate the fact that the FDA -- Dr. Gottlieb, earlier today, said he's going to take a number of those recommendations and they are announcing some steps that may improve the process.

We have already heard from other witnesses that -- or from other members of Congress who have asked you about the concerns of manufacturers and I think you covered that, which is where some of my questions were going to go.

Let me ask a little bit of a follow-up in a slightly different direction. Does the FDA also require that safety data include the reporting and use of data on patients that benefited from the expanded access treatment?

So previously we've talked about all the concerns about manufacturers about the adverse. Does the FDA use the things that turned out well and how did manufacturers -- if so, and how did manufacturers respond to that?

Mr. Dicken. Right. There are requirements and then this, as you've acknowledged, on safety reporting. I think we heard from FDA that there were circumstances when they saw other information such as dosage or other information that might be useful and they can prove it and that there were limited instances

that's also limited here where the information could be used by the manufacturer in supporting its application for approval.

And so in those cases, if the manufacturer is providing information to FDA in some limited cases this also helps support FDA's decision for approval or labelling or dosing.

Mr. Griffith. Well, and I do think that it's important that FDA consider both because, you know, it may not be the best evidence. We might want to have the full clinical trial to get the best evidence.

But when you have somebody who is using this process it is at least some evidence of whether it's good or bad or helpful or not helpful, and I do appreciate it.

With that, Mr. Chairman, I yield back.

Mr. Burgess. Chair thanks the gentleman. The gentleman yields back.

And Mr. Dicken, let me just ask you. You heard Commissioner Gottlieb -- Dr. Gottlieb and I discussed a little bit and he didn't want to steal the testimony that you were -- you were going to provide.

It didn't bother me at all. I was perfectly willing to pre-empt any impact that you might make. But do you feel that the answer that I got was that satisfactory? Was that fulsome in that response as far as the adverse reporting issue?

Mr. Dicken. Yes. Certainly, it did not -- I was pleased to have that discussion happen earlier as well and agree that,

you know, certainly, the adverse event reporting I think was a fair characterization. So yes, thank you.

Mr. Burgess. And I am going to -- again, your preface or your premise, as you started out with your report, was that there was the perception that the program has been criticized by physician and patient advocacy groups for being too burdensome and confusing.

But now as we've worked through this process with the guidance that the FDA is going to be providing with perhaps some of the legislative products that are out there, do you feel like we are generally moving in the correct direction to get -- to get therapies to patients in a timely fashion that will actually impact their clinical course?

Mr. Dicken. Yes. I think we heard from patients and groups and providers and manufacturers that they thought progress was being made in improving the expanded access program and, certainly, continued to streamline and educate providers, individuals, and manufacturers about that.

We still are hearing, still, during the course of our work, that even though FDA has streamlined their application that some others, such as Institutional Review Boards occasionally may still ask for the more complex information, and there have been efforts to kind of educate so that more streamlined information can be used not only by FDA but other entities that need to approve this expanded access use.

Mr. Burgess. So, I mean, the Institutional Review Board, that's a -- that's a good thing. We want that independent look at a request for expanded use.

At the same time, I mean, if someone is not -- if someone is just out practising in the community and they have a patient who has this request, it can be difficult for them, that -- the IRB itself becomes a barrier, does it not?

Mr. Dicken. And that is where I think there were some efforts to help educate IRBs who may only in some cases experience these requests occasionally and so some efforts to both educate IRBs to perhaps have some specialized IRBs that would have more experience with this process and help minimize and streamline that as an obstacle.

Mr. Burgess. And to even provide some flexibility within the IRB structure itself where something needs to happen in a more -- where time becomes a critical factor. Do I understand that correctly?

Mr. Dicken. That is correct.

Mr. Burgess. Now, you did not -- at least -- well, let me just see if I can ask this in the right way. It really wasn't your function to assess the liability concerns that some manufacturers might have. Is that correct?

Mr. Dicken. That's correct.

Mr. Burgess. Is that a fair statement? That's why it's not really addressed in your report?

Mr. Dicken. Yes. We did not independently assess that. We did ask manufacturers and others about what their concerns were and I think you've heard about some of those concerns.

Others are outlined in our report and those dealt more with supply, with concern about any public backlash if they should deny it, about risks and potential benefits.

Mr. Burgess. Yes. I think we are going to hear a little bit more about that.

Well, seeing no other members wishing to ask questions, I do want to thank you for your testimony today. Thank you for your participation in the -- in the hearing.

We are going to transition to our final panel, again doing so without a break in the action. It will take a few minutes more because we do have a little bit larger panel now for our final panel.

But I ask our witnesses to take their seats and each witness -- after you get a chance to get situated each witness will have an opportunity to give a statement followed by questions from members.

And there is no pressure on the technical challenge to get the name -- and, again, each witness is going to be recognized for five minutes to give a general statement and then we'll follow that with questions from the members.

On our fourth and final panel, we are going to hear from Ms. Naomi Lopez-Bauman, director of Healthcare Policy at the

Goldwater Institute; Lieutenant Commander Matthew Bellina, United States Navy, patient and advocate; Mr. Kenneth Moch, president and CEO of Cognition Therapeutics; Dr. Alison Bateman-House, assistant professor, Department of Population Health, New York University, Langone Health; and Dr. Ellen Sigal, chairperson and founder, Friends of Cancer Research.

We appreciate each of you being here with us today and you will each be recognized five minutes for an opening statement.

Ms. Lopez-Bauman, we will recognize you for five minutes.

STATEMENTS OF NAOMI LOPEZ-BAUMAN, DIRECTOR OF HEALTHCARE POLICY, GOLDWATER INSTITUTE; LIEUTENANT COMMANDER MATTHEW BELLINA; KENNETH I. MOCH, PRESIDENT & CEO, COGNITION THERAPEUTICS, INC.; DR. ALISON BATEMAN-HOUSE, ASSISTANT PROFESSOR, NEW YORK UNIVERSITY (NYU) LANGONE HEALTH, DEPARTMENT OF POPULATION HEALTH; DR. ELLEN V. SIGAL, CHAIRPERSON AND FOUNDER, FRIENDS OF CANCER RESEARCH

STATEMENT OF MS. LOPEZ-BAUMAN

Ms. Lopez-Bauman. Chairman Burgess, Ranking Member Green, and other members of the committee, thank you for the opportunity to address you today.

My name is Naomi Lopez-Bauman and I am the director of healthcare policy at the Goldwater Institute. We began our work on Right to Try about five years ago.

Doctors and patients approached the institute because dying patients were not getting access to the innovative treatments. Meanwhile, the wealthy and well-connected could seek innovative treatment overseas, leaving most others behind with few options.

Diego Morris, who was diagnosed with osteosarcoma at age 10, is one of those lucky few. His family relocated to England for an entire year so that he could obtain a leading treatment that seven years later has yet to receive U.S. approval.

It's also considered the standard of care in many countries around the world. Diego is now a healthy 17-year-old who is now

helping to ensure that other patients like him are not left behind.

Something is desperately wrong when terminal patients who are out of options are required to stand in line for permission to seek an investigational treatment that their doctor is recommending and that a manufacturer is willing to make available.

Right to Try is about the terminal patients who don't fit into a control group, who can't afford to travel overseas or move to another country, and who simply want permission to seek the same treatments that other patients, sometimes in the same medical facility, are already receiving.

This inequity occurs despite the fact that one of the bedrock principles of medical ethics is patient autonomy. When a life hangs in the balance, decisions about healthcare are ultimately for the patient to make.

That is the basis of the state Right to Try laws, and I am very happy to report that yesterday the Senate in Pennsylvania unanimously passed Right to Try so now in Pennsylvania it has passed both chambers unanimously and we hope will be the thirty-eighth state that will be a Right to Try state, and we are still proceeding in the additional states as well.

But under these state laws, if you have a terminal diagnosis and you have exhausted all other options, you may seek, under your doctor's care and direction, investigational treatments that have passed phase one of the FDA clinical trials and are continuing to undergo FDA evaluation.

Simply put, this law extends to all terminal patients who are dying and out of options the same right to try to save one's own life that is already enjoyed by the wealthy and well-connected and the lucky few that are in the clinical trials.

At the worst time of his life, Mark Hayutin of California was facing terminal cancer and insurmountable odds when he became a patient of Dr. Ebrahim Delpassand, a nuclear medicine physician who was testing a promising treatment.

Then the FDA terminated the study that Mark was participating in because there was no longer a need for more patient data. Mark was left without the ability to complete his treatment.

It is because of the Texas Right to Try law that Mark was eventually able to complete the treatments. Today, Mark credits Dr. Delpassand and the Texas Right to Try law for saving his life.

The federal Right to Try legislation under consideration today is not a call to ignore research or undermine science or for doctors to abandon their obligations to their patients or for drug companies to disregard the complex ethical questions such as how to distribute limited supplies of drugs.

And, obviously, Right to Try is not a guarantee that an investigational medicine will work or that patients and doctors who will have -- will have perfect information to make these informed decisions. And as the FDA admits, no system can ensure against all risks.

But that isn't the question for us today. The question is

who should ultimately decide what level of risk is acceptable to a dying patient -- federal officials or the patients themselves, in consultation with their doctors?

Thank you for your consideration of Senate Bill 204, the Right to Try Act. I yield back to the chair.

[The prepared statement of Ms. Lopez-Bauman follows:]

**********INSERT 6**********

Mr. Burgess. Chair thanks the gentlelady for yielding back.

Lieutenant Commander Bellina, you are recognized for five minutes for a statement, please.

STATEMENT OF LT. BELLINA

Lt. Bellina. Ladies and gentlemen, thank you for inviting me here to speak today in my -- I don't think I am on.

Is that better? Yes, way better. All right.

And my diaphragm is failing a bit here so if I get hard to hear, Mr. Moch, if you'd give me a poke I will speak up.

Ladies and gentlemen, thank you for inviting me to speak here today. I also want to extend a heartfelt thank you to my representative, Congressman Brian Fitzpatrick. He has been a tireless advocate not only for the ALS community but for all terminally ill Americans.

In my advocacy work, I have met literally thousands now terminally ill people and their families and the vast majority ask me how can anybody oppose the Right to Try bill.

I appreciate that sentiment, but I also do respect the fact that they are well-meaning people with ideological differences. I would like to illustrate the arguments I've heard and why I believe they are based on faulty logic.

The one that I hear all the time and it's been thrown around a lot today is, you know, we had this expanded access program -- we already approved 99 percent, you know, why do we need this bill.

On average, there are less than 2,000 applications per year, by conservative estimates. There are nearly 30 million Americans living with incurable conditions. I would like to draw an

analogy.

Imagine there were 30 million Americans eligible or food stamps. Two thousand applied and were approved. The other 29,998,000 never completed an application and they starved to death.

Would we be congratulating ourself on that kind of stat? That never offends me. The major difference I see is that food stamp reform would involve a fiscal note and this bill doesn't. So, in my mind, it's better.

The FDA's involvement -- and, really, Dr. Gottlieb, I think, did a great job. Their involvement -- they've tried so hard but their involvement has a chilling effect on the manufacturers and that is the supply issue that he was talking about.

The other argument I hear pretty often is that the state Right to Try bills have had little impact so why should we pursue a federal bill. The hundred or so case patients in Texas that you mentioned would have a very different opinion.

But let's assume for argument's sake that that hundred people is not enough for us to make an effort here today. I think the big issue is the courts and their broad interpretation of the -- of the interstate commerce clause.

Most pharmaceutical companies are trying to sell a drug in more than one state. So, you know, we need a federal law to protect them in that case.

I am sympathetic. You're going to hear from Mr. Moch here

in a minute and other pharmaceutical executives. I know this makes -- you know, it makes their job harder when, you know, you have patient communities and social media calling them out -- you know, why aren't you giving the drug to this person or that.

I would say the issue is that, you know, they have to have the courage and tell the community what they think is right and wrong and sometimes the answer is no. And I do appreciate that, but we can't let the FDA be the bad guy.

And I will sum up by saying I know it's probably too late for me. I made my peace with that.

I need to know before I die that if my children find themselves in this unenviable position, this nation that I proudly serve will respect their liberties and the right to make their own decision about their medical treatments.

Thank you for having me. God bless.

[The prepared statement of Lt. Bellina follows:]

**********INSERT 7**********

Mr. Burgess. We thank the gentleman for his service and thank him for his testimony.

Mr. Moch, you are recognized for five minutes, please.

STATEMENT OF MR. MOCH

Mr. Moch. That's a tough start for me.

Good afternoon, Chairman Burgess, Ranking Member Green, and members of the subcommittee.

My name is Kenneth Moch and I am the president and CEO of Cognition Therapeutics, a company developing what we hope is a new medicine for Alzheimer's disease.

Over the course of my career, I have been the CEO or co-founder of five biotechnology companies focussed on developing new medicines for terminal or life-threatening diseases including serving as CEO of an anti-viral therapeutics company called Chimerix.

Starting in late 2009, Chimerix provided its experimental anti-viral called brincidofovir under expanded access to 430 critically ill individuals.

This was one of the largest expanded access programs undertaken by a biotech company, at its peak accounting for an estimated 6 percent of the expanded access requests to the entire FDA and an estimated 30 percent of the requests to the anti-viral drug division.

The FDA was never a hindrance to granting these requests and the FDA staff we dealt with including the division director were extraordinary in their help and their compassion and their clear understanding of the critical needs of the patients.

Right to Try legislation would not have changed anything that we did during this multi-year program. At the end of 2012, we made the difficult decision to cease the expanded access program and focus on the pathway to FDA approval.

Fifteen months later in March of 2014, the family of a critically ill seven-year-old boy named Josh Hardy started a social media campaign access to brincidofovir.

The high profile #SaveJosh campaign catalysed international debate on issues of ethics and equity in expanded access and raised questions regarding the role of patient advocacy and social media and, in many ways, led to the ongoing discussion today about right to try.

Let me state clearly that I am an advocate of expanded access -- what I prefer to call preapproval access -- when it is appropriate for the medicine under development.

The testimony today has been heartfelt, truly heartfelt, and I believe that everybody in this room, if we had a family member who was critically ill -- a child, a parent, a sibling -- or if we were critically ill ourselves would do everything in our power to gain access to an experimental medicine that might increase the chance of survival.

That being said, expanded access programs raise social, ethical, and moral conflicts and dilemmas regarding access to experimental medicines.

How does society or a company balance the immediate needs

of a critically ill individual, in many cases a child, versus the potential needs of many future patients?

Who is advocating for those future patients who might not receive a needed medicine because FDA approval is delayed by even a week or a month?

And I am not talking about the FDA delaying the approval process -- the review process -- but rather, what might happen if because of an unexpected finding our outcome some percentage of potential participants choose not to enroll in a clinical trial, slowing down the development time line?

Being very granular, what would have happened to the brincidofovir clinical development program and even to Chimerix if, after a global social media campaign, Josh Hardy had received brincidofovir and shortly thereafter died?

We live in a world of social media, and while the FDA might not react to patient -- to the -- might not react, the patient community likely would have.

In other words, you can't look at Right to Try legislation without looking at all of the implications and applications of this law.

At the time of the #SaveJosh campaign, I characterized this ethical dilemma as not being about Josh but about the many future Joshes. This question is the challenge that faces each of you as you discuss and think about Right to Try legislation.

Let me also say that I am not a supporter of Right to Try

legislation. In my opinion, this legislation does nothing to help patients in need.

I believe there are things that need to be done but Right to Try is not in any way addressing the complexities of drug development.

And given that the FDA only considers expanded access requests when it is received by the drug sponsor and approves over 99 percent of these requests, the decision to grant expanded access requests fall to the leadership of the company developing the new medicine, not the FDA.

It is crucial to understand the extraordinary complexity of developing new medicines as well as the fragility of the biotechnology companies that are the predominant sources of these innovations.

No ethical company that I know of would ever release an experimental medicine outside the FDA's regulatory process. A basic mantra is that all drugs have side effects and cutting scientific corners creates unbounded risks.

There is simply no monolithic answer to the question of when circumstances and timing are right to undertake an expanded access program because each experimental medicine is different, the safety and efficacy parameters are different, the clinical development processes and regulatory pathways are different, and the patient populations in need are different.

Expanded access is not drug development and Right to Try is

not drug development, and given this fact, it is not unreasonable for a company to decide not to initiate an expanded access program until there is sufficient data demonstrating the efficacy and safety of an experimental medicine.

In closing, I believe that Right to Try legislation as currently crafted is not the answer to any of the questions that have been raised about providing experimental medicines to critical or terminally ill patients.

Bypassing the FDA is not in anyone's interest and no ethical company I know would do so. At the best, Right to Try will not help people and at the worst, I believe, it could do harm.

I thank you for your time.

[The prepared statement of Mr. Moch follows:]

**********INSERT 8**********

Mr. Burgess. And we thank you for your testimony.

Dr. Bateman-House, you are recognized for five minutes, please.

STATEMENT OF DR. BATEMAN-HOUSE

Dr. Bateman-House. Mr. Chairman, Ranking Member Green, and the members of the Health Subcommittee, I am Dr. Alison Bateman-House, an assistant professor of medical ethics at NYU Langone Health.

Thank you, first, for having this hearing and also for the opportunity to be here with you today. It's wonderful to see so many people engaged in trying to help patients who find themselves in exceedingly dire straits.

I co-chair a working group on compassionate use and preapproval access. This group is composed of patient advocates, members of the pharmaceutical industry, individuals with clinical trial and compassionate use experience, bioethicists, lawyers, venture capitalists, and individuals with both experience at the FDA or the Reagan-Udall Foundation for the FDA.

This working group was formed before the Right to Try movement began and there was no litmus test of any sort on Right to Try or any other topic for members to pass to be invited to the group. And yet, every member of our group opposes Right to Try on ethical, legal, and pragmatic grounds.

The working group was founded in the aftermath of Josh Hardy's quest to gain access to brincidofovir that Mr. Moch just spoke of. That case and others made public headlines and indicated that there was dissatisfaction with the existing system

for accessing investigational medicines outside of clinical trials.

So our task was a specific mission -- to study access to investigational drugs outside of clinical trials from the vantage point of all stakeholders to identify what problems existed and to propose solutions.

We have identified many concerns with the current system and we have proposed several ways to address these concerns. I will review some of these briefly. But before I go any further, I want to make two points very clear.

First, after more than three years of studying all facets of compassionate use or preapproval access, including the right to try, the working group has found that the FDA's expanded access program has been doing an excellent job in helping patients obtain access to experimental drugs.

Earlier today, we heard Representative Fitzpatrick say that Right to Try would "prevent the government from blocking access to potentially lifesaving treatments."

This is a solution for a problem that does not exist. We have heard repeatedly today that the government is not the barrier to people getting access.

The second point I want to drive home is that no piece of Right to Try legislation either on the state or federal level addresses the myriad issues the working group has identified in this space.

So what issues have we found? First, as we've heard today, there's a widespread lack of knowledge about the expanded access program.

My working group has tried to address this dearth of knowledge by hosting webinars, publishing and speaking extensively and partnering with patient organizations for events like Ask an Expert sessions.

But, obviously, our small volunteer group is unable to fill a national educational gap.

So we have told the FDA that it needs to step up and to make sure that there's more understanding in this process. But this responsibility for increased education cannot rest solely on the FDA.

Doctors and nurses organizations, pharmaceutical trade associations, and all sorts need to step up and be involved.

Another especially troubling issue is that of rampant, inaccurate, even mythological beliefs. Some patients believe the FDA can force companies to give access to drugs. This is not true.

Another widespread myth is that the FDA is slow in handling requests. This is not true. Another myth is that the United States somehow has an incredibly small number of patients being served.

We don't know if this is true or not. When people say that less than 2,000 requests have been approved, those are protocols.

We don't know how many patients are in those protocols.

We know about half the protocols are single patients, so just one, but the others could be anywhere from hundreds to thousands of patients. We don't know.

And the last myth that I have heard is that, you know, focussing on legal liability prosecution is necessary -- that somehow we need to protect companies from legal risk. This is also a myth.

And because these myths are persistent, widespread, and may well be leading companies, doctors, or hospitals to turn down patient requests, they have to be dealt with.

So these among others are some of the problems that the working group has identified and you will note that I have not identified Right to Try much in what I've said because none of these issues are dealt with in any of the Right to Try laws.

I will quote a recent letter from 22 patient organizations that say, quote, "Our organizations support patient access to unapproved therapies."

But S. 204 and H.R. 878 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.

In closing, I want to point out one way that Right to Try laws have already caused harm and that is by taking what was already a confusing situation and making it even more confusing.

We now have 37 state laws -- it's not one coherent law, they are each individually different -- plus a potential for a federal law.

You know, especially when patients cross state lines to seek health care or when you have hospital or insurance organizations that span state lines, such complexity is the enemy of patients.

I thank you for your time and I look forward to your questions and I yield back to the chair.

[The prepared statement of Dr. Bateman-House follows:]

**********INSERT 9**********

Mr. Burgess. Thank you for testimony.

Dr. Sigal, you're recognized for five minutes for a statement, please.

STATEMENT OF MS. SIGAL

Ms. Sigal. Chairman Burgess, Ranking Member Green, I am honored to be here today, and members of the committee.

I am Ellen Sigal, chair of Friends of Cancer Research, a group -- a nonprofit that is committed to innovation, accelerating better treatments for patients that are safe and effective.

I founded Friends over 20 years ago, driven by the profound loss of my dear sister, Gale. After many years battling cancer, Gale had exhausted every option.

As metastatic breast cancer raged through her body, defeating all conventional treatments she found, 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. In Gale's case, the side effects of the treatment were swift and violent. Within two days, at the age of 40 she was dead, leaving her four-year-old daughter and husband behind.

All of us here today agree on the basic premise -- more must be done to save patients' lives. We must continue to ensure our regulatory system is expediting therapies as safely and quickly as possible.

Friends of Cancer Research took huge steps towards the beginning -- towards this beginning five years ago when we worked

with many on this committee to create the breakthrough therapies designation. It has been incredibly successful.

This is progress, but I will acknowledge much more needs to be done. In addition, a predominant reason why patients seek expanded access to experimental therapies in the first place is that they are unable to attain them by enrolling in clinical trials.

By expanding eligibility criteria and taking down barriers that oftentimes disqualify a patient from participating in a trial to begin with, we can make additional progress.

Legislation before Congress seeks to grant all terminally ill patients the right to try experimental therapies once approved alternatives have failed, even though the FDA authorizes 99 percent of compassionate use requests.

Serious changes to today's legislative proposal are needed before this law is safe for patients. First, provisions for informed consent are essential.

A significant majority of early-phase drugs are dangerous and ultimately prove ineffective with upwards of 90 percent never being brought to the market.

Any legislation that goes forward cannot circumvent the FDA and must be carefully crafted to assure that we do not create a loophole for those seeking to profit off the sick by offering false hope. This is reprehensible.

Second, the limits of right to try must be clear. Even if

patients receive the right to request an experimental therapy, the drug company developing the therapy is under no obligation to provide it.

Patients petitioning for expanded access deserve accurate information about whether the potential benefits outweigh the risks. This is highly personal calculus. It's impossible if drug companies do not monitor and report side effects.

A key component is transparency. Patients have long been frustrated that they could not find information about expanded access on sponsor website and didn't know how to make a request for the sponsor.

The Reagan-Udall Foundation, which I am honored to chair, recently launched an expanded access Navigator for compassionate use of experimental therapies.

The Navigator is currently being piloted in oncology with the goal of increasing accessibility to information for patients and providers.

We have already -- we already have three dozen companies that contribute their information and had 10,000 visitors to the site. In the very near future, this program will expand to include rare diseases.

While I fully believe that dying patients should have access to promising treatments, we must not subject patients to false hope or unacceptable side effects. With significant adjustments, federal Right to Try legislation could help very sick

patients.

One of these adjustments is that patients must have more and immediate access to information about significant adverse events or death of patients that have previously been given the therapy.

Another adjustment would be the establishment of the designated central Institutional Review Board with the predominant focus of coordinating and dealing with expanded access requests.

The current legislative proposals 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 very much.

[The prepared statement of Dr. Sigal follows:]

**********INSERT 10**********

Mr. Burgess. I want to thank each of you for your testimony. It's been a very powerful panel. We will begin the member question portion by recognizing the gentleman from Kentucky for five minutes for his questions.

Mr. Guthrie. Thank you, Mr. Chairman.

Lieutenant Commander, thank you for your service.

Lt. Bellina. Thank you.

Mr. Guthrie. We appreciate what you do and appreciate your service. I am one that's -- we need to figure out how to handle expanded access.

I think, Dr. Sigal, you summed it up. We need to do it right, do it correct, and give people opportunities to make informed decisions within what we have to sort out and try to figure out so we don't do more harm than good.

I think, as you mentioned, so we need to do it right and that's why I think this hearing has been important and your willingness to testify has really been helpful and we appreciate that.

One thing that -- that when you start getting into this that some of the unknown, not just in the FDA or all the other issues is just other things and, one, I understand that Hospice services are provided once you've exhausted all options, and then after you've exhausted all options you get Hospice services for care and comfort until the end of life -- kind of end of life care.

And my understanding is it could jeopardize Hospice services if you go into an experimental treatment. So it -- and so the

question I get to, I think, in Dr. Bateman-House's testimony you said that 19 state patients receiving expanded access drugs lose their Hospice coverage and six states say these patients may be denied coverage for home health assistance.

So my question -- I think you talked about it too, Ms. Lopez-Bauman -- if both of you would talk about this and two questions.

How would a federal Right to Try Act impact access to Hospice services, and the second, how do states who have passed this legislation balance access to Hospice services with a right to try? Do you want to start first?

Ms. Lopez-Bauman. Thank you. The Right -- thank you for your question -- and the Right to Try Act, Senate Bill 204, was amended prior to the Senate vote in order to address and accommodate a lot of these concerns and such as specifying how adverse event data can be used, requiring reporting to the FDA, capping allowable charges to direct costs only, and limiting manufacturer reliability.

I can tell you that that is -- that in the insurance area, end of terminal and end of life and Hospice benefits and those things, those are terms that under the insurance -- under the state insurance laws and regulations.

And so even if -- you know, even if you might technically not be eligible for Hospice because you are continuing to seek treatment, it doesn't mean that that patient will not get that

treatment.

I would also like to point out that these laws have undergone four years of addressing these kinds of stakeholder concerns and in the 37 states and counting where it is now law, and the message from across the country is really loud and clear that terminal patients shouldn't have to beg the federal government for permission to pursue these options.

And I would also like to point out something. We've heard a lot about opposition to Right to Try because of -- based on false hopes.

But I would like to point out that we've heard a lot today about how the FDA is addressing the adverse event issue.

I very quickly took a look at the guidance that they issued today and it's really important to point out -- and I direct you to questions 25 and 26 in the guidance -- where they are actually -- they are changing the -- basically the standard for reporting adverse events.

But they are -- they are not addressing how they are going to actually deal with those adverse events.

So after a GAO report, after years of patients talking to Congress and the FDA about the need for actually clarifying how the FDA will address adverse events, it's still not done, not even today, even though we've heard about it all morning.

Mr. Guthrie. Okay. I only have just a little bit amount of time. So I want to give Dr. Bateman-House a chance to answer

the question about access to Hospice services.

Dr. Bateman-House. Right. So in addition to potentially losing access to Hospice services under some of these state bills, you could lose, as you mentioned, access to home health care.

You also, under a few of the bills, could actually lose access to health insurance for six months post, you know, this treatment that you get through Right to Try.

Mr. Guthrie. Are states trying to address -- then fix, as Dr. Lopez-Bauman kind of --

Dr. Bateman-House. Well, so as --

Mr. Guthrie. Once that's been passed they look at it and they say, oh, unintended consequence and we try to fix --

Dr. Bateman-House. I always say, unfortunately, we are working in a data-free environment. Despite our best efforts as a group, we have only found two doctors who admit to giving treatments to patients under Right to Try. They're both in the state of Texas. Texas does not have a reporting requirement.

We have no idea what happened with those individual patients. One of the states that does have a reporting requirement, Oregon, we contacted them to try to find out, you know, the experience of the patients and did they lose access to anything, et cetera.

They had no record of anyone being treated under Right to Try. California also has a reporting requirement but it's only -- the law has only been in effect for about a year or so.

Mr. Guthrie. And we've got about 30 seconds. I know your

opposition to the bills before us. But is there a protocol or, as Dr. Sigal kind of suggested -- is it Segal or Sigal? Sigal?

Ms. Sigal. Sigal. Whichever.

Mr. Guthrie. Sigal -- Dr. Sigal. I had a professor named Dr. Sigal, spelled the same way. That can be put in place so this could be -- this could work? Or is this just something you don't think can work at all?

Dr. Bateman-House. Well, the thing that I don't think has been said today is that it's not supplanting the FDA. What it's doing is it's doing an alternative pathway.

So if you want to go through expanded access you still can. If you want to go through Right to Try, you can't. I mean, you can also do that if the federal bill were to pass.

Honestly, as Ken Moch said, I have said all along I don't think any reputable company will give access to drugs this way. So I really think it's a moot issue.

Mr. Guthrie. Well, thank you. My time has expired so I yield back.

Mr. Burgess. Chair thanks the gentleman. Gentleman yields back.

The chair recognizes the gentleman from Texas, Mr. Green, five minutes for questions.

Mr. Green. Thank you, Mr. Chairman, and having served 20 years in the Texas legislature and had a number of issues when I was serving there, whether it be cancer treatment with peach

pits -- laetrile -- DMSO, who had -- and states have an ability to do that, whereas on the federal level we have an FDA since 1906 and so it may be easier for states to say well, you have the right to try.

And, basically, I agree with that. If I was terminally ill or needed, I would want that. But I also know we have this agency that has tried to protect us for over a hundred years and to do it.

Dr. Sigal, I know that you've spent considerable time and effort on working with researchers and sponsors to help enroll patients in clinical trials.

I represent a district in Houston and we have some great clinical trials whether it be at MD Anderson, Methodist Hospital, any of ours. In fact, our chairman actually went to medical school in Houston.

But I am greatly concerned that the Right to Try legislation would confuse families and patients on what role the FDA plays and how they can access the FDA, and let me give you an example.

A couple of years ago when we had the Ebola scare, I was concerned that there was something on a lab table that would treat these patients, and I checked with them and I was told that they did that and the FDA gave 24 hours' notice that they could give that.

These patients were U.S. citizens. They were doctors. They were cognizant of what they were doing, and the sad part is

we don't know whether that medication helped them or not because, you know, it wasn't a trial. It didn't have a comparison.

So but what would the impact on increasing access to investigational drugs through Right to Try legislation outside the clinical trials have on clinical trial enrollment and do you believe it would endanger or delay clinical trial enrollment?

Ms. Sigal. The answer is yes. The clinical trial system is not perfect but it is the gold standard and we do need to work on exclusionary criteria on it.

However, if patients think they can circumvent it and get this drug off a clinical trial through Right to Try, clearly, they are going to try to do it.

Unfortunately, there will be probably no company -- reputable company that will allow their drug to be used that way. But I do think we can do a lot about clinical trials and we can do a lot more in informed consent.

But in fact the clinical trial system is the best we have. We need to have more patients enrolled in it. We know that. We need to look carefully at exclusionary criteria, and also we are doing a lot about innovation.

FDA now is -- we are working on lung cancer master protocols. There are single arm trials. There's seamless drug development. There's a lot going on in this field to expedite drug development so patients can have the benefit of these treatments earlier because that's what we want.

Mr. Green. Okay. And another concern I have is that pediatrics -- we also have the great hospital, Texas Children's, and those facilities all over the country, and just because, you know, children are different than adults and we need to have trials with children, and I know Congress over the years has encouraged that. Would that also impact pediatric clinical trials?

Ms. Sigal. The answer is yes. We need more of them. We know we need more. Twenty-first Century Cures just have really important provisions to expedite that and to really handle with drug development on it.

But, again, the same issue -- if people think that they can access a drug through Right to Try, they are going to circumvent the clinical trial process and then we won't know the data.

We won't know exactly what happened on it and, again, the ability for the patients to access these trials is -- or this Right to Try is going to be highly limited and really very worrisome.

Mr. Green. Okay. Thank you, Mr. Chairman. I yield back.

Mr. Burgess. Chair thanks the gentleman. Gentleman yields back.

Chair recognizes the gentleman from Virginia, Mr. Griffith, five minutes for questions, please.

Mr. Griffith. Thank you. Thank you very much. I have to say I don't think that patients who are dying are going to be confused particularly if we say this has not yet been approved by the FDA. Would you agree with that, Ms. Lopez-Bauman?

Ms. Lopez-Bauman. Thank you for your question. Yes. The Right to Try Act, Senate Bill 204, actually works in tandem with the current updated process and that is why Right to Try is only available to patients who have exhausted approved treatments, who are unable to participate in the clinical trial, and why Right to Try only applies to medicines that are already being considered by the FDA and are continuing to be evaluated by the FDA, and I would like --

Mr. Griffith. So by the time a patient has gotten to that point, they are fairly well educated on the issues, at least related to their condition and disease?

Ms. Lopez-Bauman. I think that's true, but I think that the words of Dr. Razelle Kurzrock, who at one point ran the nation's -- one of the nation's largest clinical trials actually at MD Anderson, explained that the process was so burdensome that they only submitted one application per year.

This was a clinical trial of more than 1,000 patients, and to quote Dr. Kurzrock, that there were so many barriers that even at one of the best places in the world and one of the largest apartments that this, as their day-in and day-out job, it was still very challenging.

Mr. Griffith. And I appreciate that.

I also think that it's important that we do have informed consent. Both House bills have that, and so any language that you might want to provide to make that stronger for us I would

greatly appreciate that, Dr. Sigal.

But I would appreciation any language that you could provide. Unfortunately, time is of the essence so I can't get that language right now later if you could provide us with some opportunities.

Lieutenant Commander, again, thank you for your service. You mentioned that you hoped that it would be different when your children were grown up. How old are your kids?

Lt. Bellina. All right. I have a six-year-old, a four-year-old, and a seven-month-old at home -- three boys.

Mr. Griffith. Well, I know that's got to be a great joy for you.

Lt. Bellina. It is, and I do want to also throw out there and I hope everybody hears this. There's this notion floating around that expanded access isn't getting used because people don't know or can't figure it out.

I find that deeply offensive. I would say the ALS patients I know are bright, well informed. A lot of them know more than the researchers and the doctors they work with, and the idea that they wouldn't apply because they can't figure it out, I don't even know what to say.

Mr. Griffith. Yes, I appreciate that.

Back to you, Ms. Lopez-Bauman. What legal protections to patients in the 37 states that have passed Right to Try laws have that patients in other states do not? And we are running out of time so if you could keep it as quick as possible.

Ms. Lopez-Bauman. So, really, what it comes down to is that in the states where Right to Try is now law, it's about allowing terminal patients more freedom to access the right treatment at the right time.

And it's not a guarantee but it is an assertion that patients have a right to medical autonomy and that bureaucratic and administrative barriers shouldn't be standing in the way.

And I'd like to point out that earlier this year, this very own legislative body implicitly endorsed the right to try for terminal patients to seek investigational treatments to save their own lives.

Remember little Charlie Gard, who was granted residency to seek an investigational treatment here in the United States. He was granted residency after the U.K. blocked his parents' right to seek an investigational treatment.

And so this has already been implicitly endorsed by your legislative body. We have the vehicle and it has been vetted and stakeholder concerns have been addressed. It's time to act.

Mr. Griffith. I am going to open this up for anybody to send me a response afterwards. But I do want the lieutenant commander to respond to this.

In your article -- I believe it's your article -- in the Washington Post you indicated that in 2014 nearly 25,000 people in France were using investigative treatments through the government's equivalent -- through the French government's

equivalent program and yet we had less than 2,000 -- I think that was your reference earlier -- to the food stamp program.

Lt. Bellina. That is correct.

Mr. Griffith. And what are the differences in the French program that allow them to get access to them, even though they have a much smaller population than we do that we don't have?

Lt. Bellina. Well, I think Dr. Gottlieb was 100 percent correct that it's a supply issue. It's not a demand issue, and I think their legislation allows for the demand -- the market to drive this supply is what we see there and we don't have that here. I think this bill is a big step in addressing that.

Mr. Griffith. I appreciate that.

My time is up, but if anybody else would like to give me a written response to that of what they see as either pros or cons with the French law versus the American law I would appreciate it.

And with that, Mr. Chairman, I yield back.

Mr. Burgess. Chair thanks the gentleman. Gentleman yields back.

Dr. Sigal, let me -- let me ask you, and thank you for bringing the case of your sister to us. It was very powerful.

I was actually in practice in the 1990s so I remember that controversy very well, not with your sister but with the high dose chemotherapy and rescue with stem cell transfer for metastatic breast cancer.

And it was quiet controversial and there was a sense, perhaps relating to what Mr. Moch encountered -- there was a sense that, hey, here's something that will work when nothing else will but it's expensive and so therefore it's denied.

Can you tell us what has now happened with the therapy that your sister received? Is that still a viable clinical pathway for patients to follow?

Ms. Sigal. Well, the answer is no. I mean, at the time she had metastatic disease. There were no options. But she did go into that knowing that there was a 20 percent fatality. This was the decision that she made and, of course, she died from it.

Later on when we did do clinical trials we realized that that therapy was not effective. But because patients refusing to go into clinical trials at the time it took us a much longer time.

I mean, today our system is swifter. We are, at the FDA, approving drugs in single arms, some with 10 and 15 patients. When we have the breakthrough mechanism and when we see really good evidence early, it's all hands on deck and they are getting to market earlier.

We are the fastest in the world. We published at Friends five years ago EMA versus FDA because we were told that we were slower than Europe and we were shocked.

We went back and did the study ourselves and were shocked that it was -- that we were faster and when we told the FDA they said nobody will believe you -- you have to publish this in a peer

review journal, and we did. And, in fact, the importance is not faster but the issue is better and gold standard.

So we all understand the burden of disease and particularly for dying patients who have no risks. So the ability to get them on trials, to look at exclusionary criteria, and to look at treatments that work.

But most importantly, patients really need to have information -- informed consent. They may decide they want to take the risk.

But they can't make that decision with their doctor unless they have the data, and if they don't have the data in phase one where it is really only safety and no efficacy, and if that's not available to them what decision -- what decision that's informed will be made by that patient and the physician? You need data, and then it is up to the patient if they want to participate.

Mr. Burgess. Well, let me ask a question then, Ms. Lopez-Bauman. The case that you reference -- Diego, with the osteosarcoma who's now 17 and was diagnosed when he was age 10, and you said that medicine is still not available in the United States, is that correct?

Ms. Lopez-Bauman. That's correct. If I recall correctly, it has passed phase three and this summer there was an FDA advisory council that voted against approval. Of course, the ultimate approval has not been -- has not been made by the FDA.

But I think it is really important to point out that the

current process only serves less than one half of 1 percent of terminal patients in this country and it is only the well-connected and the affluent that are able to go to other countries to get this kind of treatment.

And Right to Try is about making this available to everyone to at least pursue. I mean, obviously, there are no guarantees.

I would also like to point out something about safety. We've been talking about safety quite a bit, and Dr. Gottlieb explained how he was treated off-label for his own cancer, and I'd like to just explain to the committee that doctors can prescribe FDA-approved treatments for off-label uses, where medicines that are used to treat conditions other than what the FDA says it is approved for.

So what that means is that these are -- these are prescribed and this is completely legal and lawful and it is actually very common, particularly in areas where there's a very serious disease, where there's -- without proof of efficacy.

And this -- and this is done very frequently. About one-fifth of all off-label prescriptions are -- about one-fifth of all prescriptions are written off label and in cases where -- in cases where there aren't a lot of options, particularly the more serious types of cancer, it is the majority of the time.

And so this idea that we can't allow doctors with their patients to make decisions about what might be an appropriate treatment and, really, just run roughshod over patient autonomy

is, I think, is really the wrong --

Mr. Burgess. I am just going to interrupt you for a second because, again, I am getting such a completely different story, Dr. Sigal, and your story on Diego's osteosarcoma medication. Is there a question of the efficacy of the medication and that's why it hasn't been approved?

Ms. Lopez-Bauman. Well, I mean, there certainly isn't in other countries. It's available in Mexico, Israel, all over Europe. He went to the U.K. and in, I believe, 2014 it actually won the --

Mr. Burgess. But let me -- I am going to stop you for a second. There really wouldn't be an off-label option for Diego.

Ms. Lopez-Bauman. Not in his case. But what I am saying is that this idea of talking about risk or that you can't prescribe something without knowing the efficacy is actually not true.

In our current system, it is perfectly legal for a physician to prescribe off-label and it is actually very common.

Mr. Burgess. Sure. Very common.

Ms. Lopez-Bauman. And so this is -- so Right to Try -- you know, so Right to Try, I don't believe, poses, you know, additional risks and burdens on the doctors and the patients in terms of a lack of information or not having perfect information because -- because we are already using off-label treatments in a lot of different areas of health care and, in fact, one-fifth of all prescriptions are off-label.

So this idea that you have to have efficacy or you have to have more data before you can give a patient permission to use it is, I think, absolutely unacceptable and that the default should be that patients should have the right to try to save their own lives.

Mr. Burgess. And I -- and I don't disagree. But, again, as Dr. Sigal so eloquently pointed out in her sister's case that perhaps was premature to be utilizing that type of therapy.

And I agree, we're -- we are much better now and the -- put the United States breast cancer statistics up against anyone in the world. It is -- that is truly one of the bright spots in -- as far as developmental therapeutics is concerned.

Mr. Moch, I just have to ask you, sort of the last -- the last tier about the issue of the legal liability, and what is -- you're the one who served as -- on the -- I guess on the board or the CEO of an actual company that had to deal with this.

So that's a real concern for a company, is it not, that someone will come back after the fact and say, I was harmed by your product?

Mr. Moch. So I've been on the -- I've actually been CEO of five companies involved in this space.

The answer is no. I think that's not the argument and it is not one of the reasons that I've looked at for not making an experimental medicine available, and I've done it in multiple companies.

You have -- you have informed consent. There are going to be side effects. I think everybody knows in these cases these are terminally ill patients.

Again, I think -- and I just -- I have no other way to say this. As being right between this debate, I cautioned everybody that you're looking at a specific issue in a vacuum.

I see -- the plural of anecdote is not data. I can pull out lots of examples of people who survived or died or have been problems and you're looking at a particular case that's made -- a statement that's made to make a point.

You have to look at the totality. That's not being done in this discussion in a way that really is -- for me, as a -- as a drug developer in five companies is frustrating -- I will be clear.

There are lots of reasons that people will make or will not make a drug available under expanded access. The Right to Try laws address none of those reasons, and I think that is -- I wrote -- in my original statement I said this is feel-good legislation for legislators, and I am sorry to say it so bluntly.

The percentage of legislators who voted for Right to Try legislation is about equal to the percentage of FDA approvals of expanded access applications. It's not a relevant comparison but it is a very relevant comparison.

The problem here is that drug development is very complex. You do now know, in most cases -- in fact, you do not know the safety of a drug after phase one. I have taken drugs through phase

three and had them fail for safety issues in phase three.

Hundreds of millions of dollars are spent. You think you know the answer and 40 or 50 percent of drugs still fail in phase three.

The drug development process where you're trying to alter a biological system that's evolved over how many hundreds of millions of years and you're trying to alter one system in a human being.

Doesn't happen that way. You get side effects. You get issues. You don't know what the number is or percentage is.

So the argument that Right to Try legislation is going to make more people have access to experimental medicines does not exist in my mind as a drug developer nor in anybody I know, and I can't say it more bluntly than that.

I know it is a very emotional thing. I know we all want -- look, I've done more expanded access than most drug developers with a biotechnology company.

I want to see it happen. This doesn't do anything. If you want to talk about at some point how to do things that are helpful then you've got to get a group of people in a room and have a meaningful discussion.

This discussion really doesn't address those issues.

Mr. Burgess. Well, I actually look forward to having that discussion. So you have set the stage for our -- perhaps our second hearing in this regard.

But this has been fascinating today and, clearly, we haven't heard the end -- this is not the end of the story. But very powerful panel, and I thank you all for spending time with us today.

I don't see any other members who have not yet asked questions. So, again, I will thank you for being here today. We have received outside feedback from a number of organizations on these bills.

So I'd like to submit statements from the following, for the record: Right to Try, the National Conference of State Legislatures as well as a letter from our Senate colleagues -- Senator Johnson and Senator Donnelly.

Without objection, so ordered.

[The information follows:]

**********COMMITTEE INSERT 11**********

Mr. Burgess. Pursuant to committee rules, I remind members they have 10 business days to submit additional questions for the record and I ask the witnesses to submit their response within 10 business days upon receipt of the questions.

And without objection, the subcommittee is adjourned.

[Whereupon, at 1:34 p.m., the subcommittee was adjourned.]