Event ID: 2050875
Event Started: 11/15/2012 1:48:12 PM ET

Please stand by for realtime transcript. Please stand by for realtime transcript. welcome and thank you for standing by. At this time all participants are in a listen-only mode your during the question and answer session please press star one on your touchtone phone. Today's Congress is being recorded. I will turn the meeting over two Mister Steve warning.

We have a good lineup for you today. We will start right off and that way you can ask questions at the end of the session. Just a few ground rules -- rules, we will be talking about patient advocacy and better ways to communicate and work together. Unfortunately at this time we will not be able to discuss any new drugs or drug development or definition of disease. We will be presenting all of the presentations at the very beginning and at the end we will open the forum for your questions. First we would like to start out with Sandra Greider, she works here at the office of new drugs at the center of drug evaluation and research. I will not waste your valuable time going over her wonderful career, I will start right now.

We have a list of speakers who are on? Unwelcoming page.

Good afternoon everyone. My job today is to speak about and give an overview of FDA and drug regulation with consideration with chronic fatigue and my logic encephalon myelitis. Why don't we go to -- per peer one -- this is important because in order to really understand the whole landscape of how trunks make it to being available to patients it is important to understand the important role that different hearties play. Having been at the agency for a long time I can speak pretty well to what our role is at FDA. One of the most important things to keep in mind is that the FDA does not do the research to develop drugs.

We know a lot about the research but we don't actually conducted ourselves. with that in mind I will say a few things about our role in the U.S. and how we got to where we are. Give you some basics on what the regulatory screen works is an high level basics and try to put that into context so that you can understand how information about that can be brought to bear in your work and hours to further develop products to treat chronic fatigue syndrome and M-letter E-letter. As a secondary background, I think I will come back to the point that maybe within this framework and the larger framework of joint development, there are not more or any trucks currently proven for treatments for these conditions.

Let's go to history. Ari while he was the first commissioner of the FDA. I have some pictures on the slide here of what is called his gallery of nostrils. He is in the photo over to the left, a board of countless. That is what they were really, countless. You'll see some products that were widely popular at the time this photograph was taken in 1883.

You can see that off, you can see snake oil, people make jokes about that but it was a wildly popular over-the-counter widely available medicine to treat just about anything you could think of.

All of these products were completely unregulated. Until 19 oh six when Teddy Roosevelt signed the food and drug act. That historically was in context and brought a lot of attention to old ulcerated wounds, poisons influence, botulism, all of the God awful things around canned foods and rotting meat. Some of you are -- have read the jungle that talks about that. Another boat called the great American brought by Samuel Hopkins Adams.

One that times times what that acted was actually from it interstate commerce of adulterated foods or drugs. It basically laid down the gauntlet that drugs had to meet certain standards cost strengths and purity. Really it was more of a declaration rather than what we understand laws to be today. The FDA really didn't have any authority other than we had to demonstrate that there were problems.

And the government's work in establishing the FDA really didn't end there. from between 19 oh six and 1938 there was still a lot, it was still a wild West marketplace out there for medicine. Not as bad, but it still existed. On the slide you will see a bottle, two bottles, elixir of Sultan alone my. It was a very popular medicine to treat upper respiratory -- upper respiratory symptoms. It was a precursor of what we know as antibiotics today. There was 107 deaths reported.

Those were the ones reported from the elixir of Sultan dolomite, the truck went under drug seizure by the FDA. It was the first drug Caesar. What was in it was giggling clinical, it was in their to make it palatable. Ethylene, or add a breeze as we know it, is very sweet and it was used to make it sweet and palatable for children.

Again, clearly the FDA did not have enough authority. So in 1938 track length Eleanor Roosevelt signed what is the -- drugs had to be shown to be safe before marketing. and they had to have adequate direction for safe use and companies in order to demonstrate that, were required to submit application to the food and drug administration. You can see a picture of Franklin there on the next slide. If you could change it? Statement that one.

No. But that's okay. But what about effectiveness? Franklin Roosevelt never said anything about effectiveness. the food and drug and cosmetic act of 1938 only talk about safety. This is a picture of a machine called an Automator.

This gentleman is demonstrating how it is used. You had to wear this special hat and special shawl and the machine would crank and you would praise him through that device, its original purpose was to treat schizophrenia. It was claimed to be highly effective to treat that and a number of other illnesses. Did it work? I don't think so. It is certainly not on the market today and never got approved by the FDA.

That was the effectiveness piece. In 1938 act nobody had to show that drugs did what they said they would do. They just had to be safe and not toxic. The next slide you'll see that the cofounder Harris amendments of 1962 was the first time that companies had to show that drugs worked. That they did what they said they did. That they were efficacious. It had to be shown before the drugs were marketed the FDA would review the applications for marketing and make a decision on whether the sponsor of that application had truly demonstrated that the drug was safe and effective.

---

the pharmaceutical industry and science, science and particular has grown a lot since 1962. I don't know about many of you, but a lot of us were actually around in 1962 and it is amazing to me to think that before that time all of the drugs that were on the market did not have to be shown to be effective. Since that time we have learned a lot about how to study drugs, understand what they do and drug discovery and manufacturing are so much more sophisticated today the network then that it is just impossible for us to even imagine the rudimentary nature of science at that time.

In parallel, I think this is a good thing, as science has grown, so has societal -- societal expectations. In particular the importance of understanding patience perspectives on what the appropriate balance of risk or safety and benefit may be. Those were really not things that were factored into discussions back to me 19 sixties and not even too much in the seventies.

Today, they are front and center. So on the next slide I can show you what our centers mission is with that history in mind and that is to promote and protect the public health by assuring a safe and effective drugs are available to Americans.

---

the next slide I will say a little bit about what we mean by safe and effective. These are important. by safe we mean that the risks are managed, it doesn't mean risk-free. There is no such thing as a drug or treatment that is risk-free. We also mean the quality of the product as manufactured will assure its safety. If you take that tablet, and it has 50 milligrams of something in it, it really does have 50 milligrams of something and it and not other stuff that should not be in there. Safe is that the advertising is appropriate and what is advertised to consumers or healthcare professionals is truthful.

---

and that information on how to use the drug is widely available to help your practitioners and patients. by effective we mean that this drug has been studied with proper endpoints and standards and that someone did not just wave a magic wand and say, looks good to me. Really in general, the standards of how the drugs are tested our current and generally agreed to be scientifically sound. The drugs of today, what the standards are are not what they are a century ago. with the current status of science must be considered in our current assessment of effectiveness. Science is a part of this, as well as safety.

All of it is written down in this amazing set of volumes that have -- it is a cure for insomnia, I'm kidding. It is really tiny print. But they are really well spelled out in the Code of Federal Regulations. We have room to adapt them for critical areas of medicine and we do that through guidelines and times and two judgment.

Of the standards, everything has to come back, has been deferred marketing, has the drug been shown to safe and effective for its intended use in the intended population. On the next slide you can see, a lot of people say, if the FDA does not do the research, who does and what does times does the FDA do. Who makes the decisions?

Remember the FDA regulation started out being intended to regulate interstate commerce. Really interesting part of the history. Some companies who intended to market drugs are usually the ones who fund the research on the safety and effectiveness of drugs. They themselves again don't necessarily perform the tests, at least not the clinical trial test.

But they work with academic researchers and community researchers who will conduct those studies. FDA piece is that we actually have a rule in overseeing not at a high level. Our job is to assure that one most studies are done, times, network is going on, that patient's and safety is protected. If the regulations are in sight -- in place are being followed, by the company and investigators. So we work with the company, we call them sponsors, every step of the way as the product is being developed in going through clinical trials.

To make sure that patients are protected, to understand where they are in the program, to advise where we cap times can and understand the strategies. How they intend to study a drug. What the standards are. And whether they are going in the direction that is likely to lead to them being able to say a drug is safe and effective. When a company has done times times is done with the research study, they submit an application to FDA for review for a new drug application.

As we are doing all this work, both during the investigational phase of drug development and as part of the new drug application for FDA review, our job is to employ the best most current scientific knowledge we have available and judgment. You can see in the box there, this is the discipline listed, they are the kinds of expertise that we bring to every single investigation program and drug application. Every time the company submit something it is subject to potentially, for review by scientific experts. We have 3000 people in the center of drug evaluation alone. My opposite of the clinical part, the clinical and animal toxicology, we have over 900 people, mostly all scientists that work on this every day. We also bring branded drugs in particular, particularly different questions we have, we utilize a whole system of public advisory committees and we will meet and present data to times times it to them and ask them questions.

We do include on those as ritual talk about later, patient and consumer representatives depending on the issue at hand. You will see him the next slide, what are we doing all day? I mentioned the investigational phase of drug development. Every experimental drug him into a human must be have oversight by FDA to ban investigational new drug application. It is basically a pile.

We get in our center about 1800 new IND is submitted to us every year. About 500 of those are from commercial drug development companies. The rest are by small or independent researchers. the ones that usually result in a marketing application or commercial. Even at 500 a year, new drugs, new start ups, that is a lot. Once you have an IND it stays active for a long time. Once you do a clinical study on the new drug in August put in the file of the IND. At any given time we have about 12,000 IND's on the book. Stuff is coming and going every single day, depending on how active the development program is at the time. I am sure you are interested in what is in the box on the slide.

What we have been able to ask the team we have in house for an E-letter or CFS, IND is that her studies for Emmy or CFS. We have been able to locate evidence of nine IND is. Only four of those have had any active research I'm going within the past four years. Only four of the nine. One of those is commercial IND, it is for the drug and bludgeon, that is the open since 1990. for that IND, the last controlled trial ran from 1998 through 2004. So that is one of the four activists, the other three are much smaller research IND's was very small local times medical trials.

Again, very small. That is investigational, what about new drug are getting applications? We get several hundred new drug application submitted to FDA every year. the 200 through 300 contained not only approved -- new drugs or new doses of already marketed drugs. The number of absolutely brand-new drugs, they get approved every year, is about 25 or 30. We have been up to 30 and a little above that and the past two years. On average it is 25 or 30. Generic drugs are copies of drugs that are already approved. We have had one new drug application for CFS /-slash MV treatment. Just one.

So what do we look at when we look at these drugs, we are looking at chemical composition, we are looking at animal studies, particularly before clinical trials start, when an investigational drug is and how we are talking about the company about what the plans are for the clinical trials. Would they want to include. What does they will study, and how they know they got the best does the study. How did they come up with that number. What are they to compare the study two. How are they going to measure if it is effective. What is your study input your what are the measures that matter and how do you know that. There are multiple opportunities for us to work with companies along the course of their drug development to address some of these things.

Every clinical trial that is proposed and addressed by us in detail. Our goal is to make sure that all the light is shutting so at the end of the day and the trial is a success and has at least been a really good scientific endeavor. It shows up, everyone has confidence in whatever the results are. Whether it shows a good outcome a not so good outcome. The study was done as best as could be.

I will say a little bit about the end of the day, the gold standard in the regulation, a new drug that is being marketed, must demonstrate substantial evidence of effectiveness. Does not save perfect evidence it says substantial evidence. It has to have shown substantial

evidence of effectiveness as demonstrated in an adequate and well-controlled study. It is plural, more than one.

There are circumstances that we may of drugs on a single trial. But the definition of an adequate and well-controlled study is on the slide. This is really important. the study has been designed well enough so as to be able to distinguish the effect of a drug from other influences, such as spontaneous change, placebo effect, or biased observation. In order to be able to meet that criteria, this is the crux of why we work so closely with the clinical investigators in designing this trial so at the end of the day adventure appears to work, we can say, yes, we can distinguish the influence of the drug from other influences.

That is the most vertical thing. Next slide. The clinical trials matter a lot. They are essential in order to assess the effect of the drug. Here on the slide are the kinds of things that get measured in clinical trials. We might be, if we are luckily, there are objectives, easy to quantify signs. Quite blood pressure, kidney function, blood counts, viral counts in blood. Those are out there and used for a lot of clinical trials. We also take into account what we would call subjective measures. Those that measure symptoms. Pain, fatigue, weakness, headache, depression, things that aren't described but harder to measure. But they all involve how the patient feels or functions.

They are just a little harder to measure and quantify. I would say that the most challenging clinical trial are those that must rely on only measuring symptoms. For example, we know for clinical studies of depression, if you look at any drug that has been approved for depression, only about half of the clinical trials actually show a benefit. The other half don't show anything, they don't show a benefit, they just can't seem to show a benefit because of the symptomatic conditions of the disease.

Researchers have learned better how to measure depression. How to quantify it and get and help patients report the symptoms. This is because, there are variations, pain and fatigue are often described as different for patients with cancer or patients with arthritis, or patients that just had surgery. They describe it differently and may times they have different characteristics. They matter when you try to quantify whether pain is getting better or staying the same. If you have the wrong tool, you are not going to be able to get a very good measure. The tools have to be targeted at specific to the symptom of concern. to take that back, to take it further to chronic fatigue syndrome and I will include Emmy in this. One of the challenges that has existed to date and trying to do studies in these conditions are, the heterogeneity or multiplicity of symptoms. Even complex over decades of what the criteria for disease are, what is a case of CFS, there have been a lot of questions, a lot of uncertainty. This is really hampered research in this field.

Objective measures are limited. We don't have a marker in the blood or a marker and a muscle biopsy or something of that sort that is clearly been shown to predict the clinical course of disease. There are some that are subjective, but none are widely accepted or definitive. There is no question that the clinical trials and drug development have been constrained by these vectors.

Because the clinical trials have been constrained by these factors, I think this is why there are so few applications. In the IND stage, or the MDA -based, that the IND has to proceed. Companies are likely to be reluctant to invest in developing a treatment that is surrounded by all of these uncertainties. I would say from my Monarch during of this, there is controversies that have arisen from this.

Companies like certainty, they like a sure thing here that said, I think there is a path forward. There are other conditions that have had exactly these same challenges and are saying the fruits of collaborative work between patient advocates and researchers and funders. Some of them are listed on the slide. I mentioned depression, irritable bowel syndrome, functional dyspepsia, fiber myalgia and even prostate cancer when it is chronic and long-term.

Progress has been made by establishing definition of the clinical trial populations and targeted reliable measures to assess their symptoms and functioning your I think this to me makes me very optimistic about CFS and MTV. Times times M-letter E-letter. In my final thought going or were consummate general advice.

We do have more time in an hour, so you can be sure we will not run out. We have reserved conference time in Adobe after that. One of the critical things for patient advocates is to find common ground.

Partner with organizations times organizations or times are centers equipped to conduct clinical trials, buying parties that have expertise and interest in the condition. Don't get stuck, when a -- when you develop a research definition for the clinical trial, keep in mind that it is a research definition and that we understand, everyone understands that the brother use of a drug is likely to be a little broader than the research definition and the clinical trial.

Once a product design and market you can go back and look at one of those gaps and find ways to build up with additional information here the science does matter, it does not have to be complicated stick to the basics. Finally, be creative and learn from advocacy groups.

Enqueue break much Doctor Cuyler. That was a very good presentation and we need to remember that this and permission is important for all this to be on the same page and two be able to move forward and how to -- next would have Richard Klein from the office of special health issues.

I will times was handed over to Richard.

Thanks Steve and thank you everyone for joining us, very good to have you here. I want to talk a little bit about advocacy and the role that patients play in advocating FDA and outside FDA. FDA has for many decades and put from patients and patient advocates, but traditionally it has been through advisory committee meetings through open public hearings or written submissions. We have had public policy meetings where the public is

invited to speak. Public town hall meetings and written comments that are requested to the Federal Register.

In 1988 there was a gentle knock on the door from the AIDS community who wanted to have the agency moved faster on approval of treatments that they also wanted a more direct role in drug development because they thought they had a lot of information that wasn't being reflected. The FDA office of special health issues interface of the community and became the office -- together with the is community looked for ways to work to improve development and to get good therapeutic -- we met times met often to discuss various developments.

Is advocate began to talk with the FDA they began to learn that the agency did not really hold all the cards. There were a lot of moving pieces outside of the FDA that were involved in the development of therapeutics. They also learned a lot about the statues of the FDA works in and that the agency has limited authority and limited role in drug development. I think not everyone is completely familiar with that and Sandy went over quite a bit of that in the historical perspective. FDA essentially is a regulatory agency. Much of his role in drug development is reviewing empirical data in very tightly controlled studies.

One of the doors that the advocates of open is giving the patients in active and meaningful role of the FDA a problem -- process. AIDS advocates love the way for patients to join FDA advisory committees and participate polling in discussions about quality and data that was generated by drug sponsors and controlled studies.

They took a very active role in the discussion and they brought a unique perspective of the patient directly to the advisory table. They also did something back to the community and that was a better understanding and appreciation of the drug development process and the challenge of making safe the -- safety determinations based on available data. Once they have reviewed the entirety of the data that was submitted to the agency for review. Today there are more than 175 patient representatives who are part of the patient representative that started with HIV and AIDS. The special government employee set represent about 70 different diseases and conditions and meaningful -- and their understanding of disease community at large. That has been a very important addition to the discussion by the advisory committee and for FDA consumers. Patients bring a perspective to the table that reaches well beyond the science to understanding a complex and ethical social issue that is faced by the patient. They bring diversity of perspective to the discussion and talk about the different points of views and needs within the patient community.

Patients have an interest in clinical trials. They are interested in how they are conducted, quality of data generated, so that they can add advice on designs that would increase recruitment and retention.

They also bring a real world experience to the table that highlights the problem and the issues that are faced by individual patients. They help set the mark for balancing possible

risks against benefits of looking at new products. the best patient advocates have become knowledgeable about the disease and have taken time to understand the FDA and drug development process. So there are certain facts to keep in mind for patient advocates that are working directly with FDA. One of them is that FDA works with a relatively tight regulatory framework.

It really doesn't have just jurisdiction or authority outside of that framework. I think that was the thing that HIV patients did not really appreciate until they started working with this. FDA can't encourage and guide sponsors but does not have the authority to direct their research and development. You can really only encourage and advise. FDA provides expert technical assistance to facilitate new drug development but really doesn't have any direct control over drug developers. the FDA does not have all the answers or solutions. One the HIV advocates came in and times in the beginning they thought the FDA had all these things on the table, all the answers sitting here and working together I think they really saw where the challenges were.

---

the other issue is that FDA operates under strict confidentiality rules concerning proprietary information related to product development so often it seems that the FDA is a black box because it is not free to talk about applications. Therefore what advocates do is go to the source to find out where the development process was and they started working much more closely with sponsors.

Drug development involves many parts that need to come together, it is like pieces of a puzzle that need to come together to form a whole and in that picture becomes more evident. FDA does not have the mission, resources or jurors diction over most of the drug development process. It has a very defined role that comes in in the oversight and review of the rest of the process.

FDA does not hold or control all the pieces. It is just one component. So this little illustration, all of the pieces that together, people who work or get involved in the process of drug development really need to work with the FDA but also researchers, Congress, which can direct funding and two a certain extent can call the shots on how research is pushed forward in a particular direction. Mostly with industry, because industry is the one who makes the decision about what products they want to pursue. What diseases or conditions they want to look into. and how to make decisions about the risk and convinced of their business and developing certain therapeutics.

Outside of that puzzle of course there are more pieces that would fit into it. So it is not limited to that but there are a lot of pieces I go into the puzzle of developing new therapeutics. There are other important channels aside from just working with FDA and I think working directly with researchers and industry is one of the most important directions that advocacy can go in getting involved in joint and product development.

Also, convening broad-based meetings to develop consensus and address errors -- barriers and product development. One really good example is the collaborative research on HIV and AIDS. That is the organization that pulls together patients, physicians,

clinicians, industry, as well as the FDA. They created for him or everyone can talk about where the barriers and problems are and look for commonalities and ways to approach those which are very helpful to FDA.

Not the sort of meetings that the FDA can easily pull together under its regulations. I think my final point and probably would be, the FDA, the patient's sponsors, everybody is really pulling in the same direction and everyone wants the same thing which is to bring innovative new therapy to market as quickly as it can and it wants adequate information about the benefits and risks that doctors can use to guide not only patients who are making decisions, knowing how to manage the use of those drugs and successful treatment.

So, we have for the rest of the program are two very important speakers, they are members of disease communities that have had very good success in helping to guide drug development and research and sort of pulling together all of the moving arts. the first one is Mary Dwight and she was with -- is with the cystic fibrosis patients times on nation.

Thank you very much for having me and I'm glad to join you guys today. I think what would be most helpful, I know this is about advocacy is to talk a little bit about the CF foundation model. How it works and in particular, I will give you guys a quick overview of the comprehensive model and focus and towards the end about ways we particularly work with the FDA.

Let me start at the very beginning with a super brief CF one oh one. Assistant fibrosis. It is a rare disease, there are only 30,000 patients with CF in the U.S. Well below the definition of rare disease which is 200,000 patients are you are. It is a genetic disease, both parents must be a carrier of the CF gene and they have a one in four chance of having a child with cystic fibrosis. What it is is a mutation of CF CR which basically, and a layman's definition results in an inability for the body to transport salt in and out of cells. What happens as a result, is a lot of damage and a whole host of the organs in the body, most particularly the digestive tract or patients are not able to digest or absorb their food or nutrients from it.

Particularly concerning the lungs. Were thick and sticky mucus clogs the lungs leading to chronic infection, significant loan -- lung damage and eventually death. When they found -- the foundation was founded by a bunch of parents, life expectancy for a child with cystic fibrosis was less than five years old. So it really was a Beatle pediatric disease. We have made enormous gains in net times that diagnosis and prognosis for the disease and the average median life expectancy for somebody with cystic fibrosis is in their thirties, forties and beyond. What I am going to focus on in a little bit in some. Promising new drug therapies. One of which was approved by the FDA.

At the beginning of this year. Which is really a game changer for cystic fibrosis patients. Hopefully, while it is early stage, we really do expect this new range of treatments could result with somebody with cystic fibrosis could live a normal length of life. It is an

exciting time in the world of cystic fibrosis and I wanted to share with you sort of how we have gotten here and how we have made these changes and it really is through a very vigorous drug development and discovery program.

I want to point your attention to a topline overview of our drug discovery model which you can read a lot about it on our website, but there was a great headline in a recent pics, me story, X-letter, me.com and it says the new power players in drug research and development are wearing bright T-shirts.

It talks about the power of patient advocates and pushing drug discovery and development. In being their own spokespeople for fighting new treatments. I think times think the CF story is absolutely one that illustrates that. So I want to walk you through what the components are.

Because as I mentioned in my CF one oh one, cystic fibrosis is a rare disease. There was not a lot of profit motivated investors, drug companies, that were really excited and times in getting involved in the cystic fibrosis drug discovery space. They did not see the profitability for a disease that impact and so few people. So it was very frustrating for the CF community because we hadn't lot of great science behind us.

We were really unlocking the keys to cystic fibrosis and me ever Tory -- in the laboratory. We had -- Discover the CF gene way back in 1989, all of this promising signs but not a lot of folks flocking to us to help you find your new drug because -- the CF foundation develop a model which has now been dubbed venture philanthropy in order to spur on drug development for cystic fibrosis.

I think a lot of the stories around the land survey focus on the money -- philanthropy that focus around the money, -- wearing those bright T-shirts and spending the money. We had raised through walkathon's and hikes and dinner dances, two by science. But I think what is important about the conversation today, is that it is really about so much more than that. Am I driving my slides? That is the one. I want to hit not just on the money which is the first bullet on the slide but on everything else that is in here.

This is the information that we as the patients brought to the table and drug discovery and development and this certainly is applicable for interactions with the FDA, but it is also applicable for the components that have already been talked but in this webinar today. About working with the drug discovers and the companies who are moving forward in clinical trials. How can we as advocates really bring some expertise of our own disease to the table as clinical trials or designs as struts or developed? Really what the foundation's model is about bringing human capital, financial capital certainly, but also clinical drug development expertise to provide drug development companies and the FDA with a host of information with data to drive forward drug development.

So what does that look like? It certainly looks like an understanding of what cystic fibrosis is. an understanding of being able to paint the picture of disease. It is hugely help via the second bottle -- bullet, the patient registry, really what this provides drug

discovers and the FDA is a robust natural history -- history of cystic fibrosis. We also have a network of basic research centers. Which provide centralized access to clinical trial participants through a nationwide network of foundation credited care centers.

Which really streamlines clinical recruitment and implementation. In its very essence, we know whether patients are are for these drug companies who are interested in researching drugs for cystic fibrosis. That was critically important with a rare disease like CF. Quickly quickly identified for a company that was interested in studying a product or a new antibiotic to treat lung infections, ear of the patients you need to look at. Again, for a rare disease it was very valuable. Also the patient registry really enables us to keep data that was gathered in a clinical trial against what we knew about the disease.

What we really came down to was as Doctor Kreider discussed, being able to demonstrate efficacy in a drug or not. Which is equally as important when talking about drug development. We also, as I mentioned, bring experts to the table. Certainly those folks with S-letter F-letter are the consummate expert, also expertise times times and also expertise in trial design preclinical research and clinical applications of the disease. We bring the physicians and other providers to the table to say, this is what she should times times you should be looking at when you think about cystic fibrosis. This is anchored out of our therapeutics development network. It is a network of clinical trial centers that administrate their a coordinating center to Seattle Washington. There is 50 people working in it they provide expertise to industry collaborators, those drug companies we work with and others that are interested in clinical research into cystic I process. They are able to provide best practices and clinical trial design and help interpret CF outcome measures.

What does that really mean? It means telling a drug company that is interested, for example, ushering one function one times times function one way, it means being able to tell them that that actually is not the way to measure lung function and cystic fibrosis. You need to use this method instead. Or these are the endpoints that you should be looking at and how to effectively and efficiently collect that data.

This is critically important in our collaboration with the FDA as we work to develop and validate new outcome measures with the disease of all. I will say that while we have had our bulbs in the road, you can find. You bigger fans of the FDA and patient advocacy world than the cystic fibrosis world and that is because we know we have a partner that is there to talk to the data with us. But I will say that it is our job as a patient group to bring that data to the table. It is not as Doctor Kreider discussed, the job of the FDA to create the data, it is their job to analyze times analyze and effectively. Were we would save the FDA has been a remarkable part, is an understanding how they need to look at the data of cystic fibrosis. Let me give you a couple of good examples. a couple of years ago, we had before the FDA a new inhaled biotic -- and Dave biotic for the treatment of CFS lung infection. It would be the second antibiotic used in the treatment of CF. Remember I said the most automatic outcome of cystic fibrosis is chronic lung infections. That really is what proves fatal in the disease. So you better believe our patient population had a very vested and just in the second inhaled antibiotic to treat these infections. Particularly as

our population aged and many times times in many of our patients were starting to become resistant to the other alternative on the market. Initially, the FDA, are pulp in the road looks at the primary and -- and point, the objective and said, we don't know, we don't think that the change, the improvement in this lung function measurements of this antibiotic is good not.

We don't think it really demonstrates that the drug is efficacious. This is early in the process. When we started getting data back there we were able to go in because of a good relationship with them told on a long-standing trust of knowing each other had each other's best interest at heart. and the shared goal of bringing safe and efficacious drugs to the CF people. Let me tell you as a patient wife this lung function improvement in this drug is actually incredibly enabled when you think about it from the cystic fibrosis perspective, rather than just anyone with a lung infection. That the change was actually significantly clinically meaningful in this patient population. That meant adding years of life, in fact. We were able to show that through a bunch of data both from the trial itself and also from the natural history in the patient registry. We were able to come to a consensus about that and how to interpret the data being shown. We were able to provide the data and history and provide the experts including the patient voice saying, this means something to me, two my day-to-day and what I am able to do.

That collaboration was very important. I know certainly, I am sure you are thinking about, what is meaningful and chronic fatigue or empty and that is such an important role of the patients is to be able to articulate what is meaningful, what does make a difference and also demonstrate it with data. Being able to show what that change will look like.

I also want to point out that this there. Development network utilizes a collaborative approach to clinical trials that includes a centralized review of trial design. Common policies to protect patient safety, standardized research procedures and shared expertise by top CF researchers and training for staff.

So we don't have to reinvent the wheel every time I start something new in CF drug discovery. We also have a great partnership with scientific consortium that is coordinated by the cystic fibrosis foundation times foundation that brings scientists together to tackle key questions in research that really fosters unprecedented levels of sharing a pre-published data and two really accelerate the understanding of our disease. I would say that the FDA again has been a great partner, joining us in many of these consortium meetings possibly also have a very robust understanding of the research that is going on and see him so that by the time it gets to them, and the form of the drug, a new drug application, they really understand what is going on. They see the science and the understand where it is coming from her that is a great partnership. Finally, research tools for finding new resources that can help an investor discovery. We are fortunate in that the cystic fibrosis has changed significantly in the past 40 times four years and even in the last five or six. It also means that we have new challenges before us. Especially in working with the FDA.

At the point of bringing a new therapy to be reviewed, what might have worked in years past or being able to demonstrate that a drug was efficacious, it might not work anymore in the future because unfortunately we have the high-class problem of patients doing better.

How can we demonstrate something as efficacious. We cannot rely on what we have already used, what we used 10 years ago. We have to keep working together to be able to validate the tools that the FDA will use to measure the efficacy of the drug. I think that is very important, that is the kind of thing where the foundation has played a vital role in sharing that information. Inviting the FDA to come and hear the basic science. To hear the clinical discoveries that we are making as the cystic fibrosis changes how it manifests in the patient. That they are partners in listening to the sum of the basic components that may not times times might not be an immediate day-to-day but will form a critical back bone for understanding the disease as we move forward.

If you can switch to my next slide because my computer went to sleep. Hopefully the rest of you have not as life rattle on. I want to point out to you guys, great new tool that is the most recently passed prescription drug user, in the world of Washington. It is an unpronounceable acronym. Since the rule for how the FDA works in conjunction with drug companies. What are the rules that the drug companies must follow as they review drugs. There is a provision in their cottage is something that we worked really hard with the FDA to get in. Which is the expert. External experts on rare disease, targeted therapies working with the FDA. Remember I said CF is a rare disease, 30,000 patient. The new drug that I'm mentioned at first, treats only 1200 people. So, my girl rare if there is such a term. How do you really understand the implications of a drug that treats all you times times of you people. Robustly paired frankly, I think this is where a lot of medicine is going, it is -- has been referred to as the very first personalized medicine. As we slice and dice ourselves as patients into smaller and smaller patient populations based on our times edgings and maybe even having a therapy that only works for a handful of people. How can the FDA possibly have a robust enough understanding of all of these different iterations of disease by themselves.

So, what this provision, which is now times now law is designed to do is to facilitate the FDA ability to contact and reach out and understand disease through experts. So that they can have the most robust understanding of something as they review it. If I could go to the next slide. Let me be very specific that times by what this means. The topics of consultation which I think we as patient advocates should consider bringing to the FDA of the following. Understanding of the disease and the disease severity what does it mean, in other words to have this disease. Unmet medical needs, what is going on in the clinical treatment of the disease, remember I talked about a second inhaled antibiotic picks somebody could've said, you have one of how when you need another one? It was our job to explain why we needed another one here we had patients that were becoming resistant circuit did have different implications for lung function, but this can be used in conjunction with the treatment that was already on the market. That was up to us as the patience to explain and to demonstrate why that was necessary.

---

the benefits and risks of proposed therapies, certainly that has been discussed by previous speakers. I think one thing that you hear a lot when you talk about patient advocacy is patients are not consumers and that we have very different views of risks and benefits. What we may be willing to accept looks different than somebody that is just evaluating their toothpaste. Obviously those of you on the phone understand that.

Clinical design and patient participation. This -- what is a patient able to do in a clinical trial? What are they willing to participate in and what they are not? for example, one thing that is hotly under discussion right now, for cystic fibrosis, I will give you an example. Most patients take between eight and 15 drugs at a time. Most of our patients would be unwilling to come off of all or most of those drugs in order to participate in a clinical trial for a new therapy. That is something that is important that must be taken in to account when designing a clinical trial. the FDA has to approve those in the times times of the need to understand that patient respective.

Finally, the demographics in the clinical description of a patient population. I would say natural history, so essential. Are CEO usually talks to patient groups, if you do nothing else, times times else can't do your natural history. Make sure you are talking to patients and collecting data on what it means, what the clinical description of your patient population is.

These are provisions that have always been in place of the FDA. But the reason we ask for them to be put into this new law was because we saw them used sporadically. In different ways depending on which reviewed division he went into. to have a drug reviewed. We wanted to basically have a list of all those and be very clear that the patient role in consultation with FDA could be very robust but also needed to be specific to these topics. and that that was really going to raise the ball faster.

So, I will stop there because I think I have gone over my time. I look forward to questions later on.

Thank you Mary. We have one more speaker who is an other person who represents a disease area where they have had successful advocacy and that was Pat Furlong, with.

Thank you very much and thank you for inviting me to participate, I can appreciate where the chronic fatigue syndrome folks are, peer I will start talking about it he couldn't -- ecosystem of and humidity and all this lived together, the role of the patient in a patient times times the patient organization is really incredible and is incredibly important. It is really important for you to understand that working with others and borrowing from others is really going to be important as you move forward. Because there is no sense in reinventing the wheel and you certainly have no time in terms of the needs of our patients to waste time.

They have developed amazing models for all of us in advocacy and illness. One of those we have our own visit patient registry and we have heard this from the CFO about routine occasions and one of the things that we have done as an organization is developed

participation registry I will start off and back of a times times up a little bit and tell you about muscular dystrophies or you can understand in concept.

It affects primarily boys, one in 3500 boys will have muscular dystrophy and interestingly enough the same amount of web and are carriers. I am a carrier, I had four children, I had two girls and two boys. the boys were diagnosed with mosque pillared dystrophy.

They walked about 14 months of age, progressively muscles were losing function and the reason for that is muscles, muscle -- structural protein called -- without -- over time they can completely to hear times hear your and die. As boys grow they are trying to rebuild this times this muscle but essentially it is missing this trope in and therefore muscle cannot survive. Which times times. When GC is a typical progression, the boys walked, although delayed, they learn to walk, they never run, they never jumped, they cannot write byte.

Between 10 and 13 years old, it is followed by degeneration, loss times laws of armed function, usually they need respiratory health in terms of not invasive, sometimes invasive ventilation. In 1994 I started -- the concept that we needed an organization that was focused on muscular dystrophy, we wanted to of course have drug therapies approved and none times not work in their are still not improved in this area.

---

the only treatment we have is prednisone and I'm sure, perhaps some of you are familiar with prednisone. It serves and disgracing the immune response, it really brings along so many side effects such as behavior issues, delayed growth, delayed puberty, it causes a host of things that impact on metabolism that these boys really supper and don't have much benefit at all in terms of long-term.

So our role was, how can we promote drugs, how can we play a role in what we do to accelerate the development of drugs for muscular dystrophy. I'm happy to say there are any number of clinical trials in progress and many new drugs in the pipeline coming before the FDA so we feel an amazing place for -- nothing can come fast enough for our patients. We really knew we had to develop several things -- to look at eight clinical infrastructure so we times it have her patient registry in there so we registry initially confined his patients. We also needed to understand and point to what is account what is the measure, the primary outcome that we can say to the patient had improved. That shows us on some significant challenges. We do not have a marker that will -- disease progression. We can look at -- even in that we cannot correlate the Tropez puzzles or even suggest what that means in terms of data bits. There are significant challenges and the times times in the understanding of the impact of a given drug is on these patients.

We knew that we had to be involved in advocacy. the FDA could not know the intricacies of this disease -- the number -- it is really impossible, what is the important challenges is to educate the FDA so that we can see the common pathway to work together.

Which is called the M.D. care act, it gave some attention to muscular dystrophy. MIH would invest in them and we would partner with them to leverage additional funds -- that exist in United States and around the world to Excel those developments -- galvanizing research with industry and -- and I am happy to say today that we have about 15 companies that are quite industries -- interested in the area of muscular dystrophy. We, looking at the model of the foundation. We advocate for drug development so we can see together what strategies we should utilize and accelerate the development of drugs. We look at the trial development and interact with the patient to understand what is important in their lives, what will prove the quality of their lives. and what would make a difference in their lives. So that we can bring to our drug developers in the industry as well as -- to understand what might be useful and what we need to think about to validate -- the other thing I wanted to mention, I think the good news about advocacy is that we are here and it is certainly a lengthy process and we all need answers now or yesterday for that matter. As far as expectations, a therapeutic dose of hope, we have to be specific and strategic as we work through -- I don't know if I have control or you do marks.

So I just wanted to throw this out here, it is a little bit complicated and I have learned about it over time and I feel like the -- we as an advocacy organization invest in research, there is a whole E-uppercase-letter Schmidt has to be in this effort and we learn about this overtime that includes not only developing the drug but delivering the drug to the patient and what that means to patients. This is something we have to consider --

---

Because you had a really terrific explanation of this file, we have adopted to our own. I thought I would drill down and look at specifics -- our drug development strategy involves better, faster and now. It is because it is the kind of things we would like to happen. In terms of better, we try times tried to identify and directly support a drug candidate with the greatest likelihood of success. This really does validate --

---

We really want to make sure that we are offering the best candidates, -- within that M.D. enterprise they developed what we call the -- review process. We encourage the individual or academic centers that we support to -- further review process. They can times times the company is only comprised of academic, regulatory academics, with the help of those two, to understand what are the issues whether they are in the development process or --

---

From that patient perspective, not enough -- around it, or enough confidence for the patient, it really comes down to it and what we know about this drug and should go to the clinical trial, are you willing to give this to your child. This is how we try to advance compounds that have a high degree of success to go through the clinical trial office. How can we do things faster? In the case of clinical trials, by applying a block grant -- this might be expanding our survey to ask specific questions that is relevant to a given topic. This is to validate surrogate markers, this is really to look again for a marker that might provide -- given compound in. the MRI in Florida, without that MRI might be a -- we wanted to look at -- we knew that the images changed with progressive and wondered if they would change in reverse if we had a drug that had an impact. We initiated that with a small grant and ask them to a plied to the NHS, they were not successful in the first round

so we bridge them. They were friendly successful and now this is a three site study that is looking and validating MRIs, this is part of our concept.

Then we continue our advocacy efforts to as boys why they would participate in clinical trials. There is an increase in social isolation, when some of the boys get comfortable in high school, they have an organization plan and activities organized, the idea of participating in a clinical trial is not useful to them. I really don't want to is the time and usually the response to us is -- which is not a problem, we went -- and trying to find -- which really look at outcome levels, we cannot look at markers across the spectrum, it is meaningful to the population we serve. Alcott this is really about advocacy, really about trying to educate FDA to understand the process and will that together we might be effective. In one of those ways we need to look at -- before I talk about my survey and research funding, we are not a large organization, our expenditures have been about five .-point $6 million. We have five .-point $1 million in active research projects. We have about 28 projects. What it clinical process, we really look towards strategies on how to advance drugs so that patients would have access to them. One of the challenges for all this overtime is going to be the access to assistive therapies and combined therapeutics in these populations as mentioned earlier. The idea of testing once you have an approved drug, presents challenges that are different and new certainly exciting in terms of having an approved drug but certainly different and new challenges overtime.

---

and our FDA advocacy we have now met with our Board of Directors and have drafted a policy statement. the board really wanted to get behind the statement an outline that to the degree that we provided the statement to the division of neurology -- really talk about the policy --

---

We have had to successful meetings, very open to discuss and talk back and forth ideas and challenges and risks. We have educated on -- talked about the clinical variability as well as the variability of care. There are many centers cross the country, in times and in fact the MDA has clinical centers, but within the centers is highly bearable. really looking to stratify and -- clinics. We are moving towards the methodology. So we then looked at, in our discussion with the FDA and how we can be helpful and one of the conversations that has come up of many is the risk -- and to share with the community. Clearly the patient. It is perhaps different than the FDA, perhaps different from the industry view. We wanted to understand that without we have some idea about this but we thought it was not useful unless you have concrete data going forward to share with the FDA. Spoke with people in neurology, what is what they would like to see, what is the rigor that is needed to accomplish, what are some the questions they would ask. They said, very forthcoming.

It is still under review, we have engaged the entire muscular dystrophy community and this may go out to five -- follow-up which we also tried to devise novel ways to reach those patient. We spent the survey to go out to about 5000 patients. This is to look at benefits, I should separate here and say part of it is because we are dealing with the pediatric population, children under 18 years old. The survey will be taken by the parents. So what are our parents willing to risk, we believe that we are going to see more

willingness to accept the risk, just prior to the loss of ambulation and perhaps less willingness to accept risks, because of the increasing complexities of -- so we really -- commissioned by the FDA and suggested with the natives of their neurology division. We use the peer choice methodology and will distribute through MDA channels, about 8000 potential responders. We will analyze what they help of a statistician -- this is just a little bit about the design, the screening questions, demographics. Optimism questions. The degree of control over disease perceived by the parent. Patients really feel that they're out of control of some of of the symptoms we describe your days will be two separate survey so that we can separate parents view from a adult patients. We want to talk about side effects and delivery questions. Some of the compounds that are in process and clinical trials, some of them are --

---

There is a difference in how drugs get delivered and how they look at that. We went to look at the side effects, we need to talk about in terms, but you get all the way up to death and we want to understand where their mind is being drawn. We have -- and risk as well. You want to look at, you also want to give the patients an opportunity to tell their story. In year of his religion to the young adults and ask what is important in their lives. That seemed to really help us understand what the outcome measures we might look at in the adult population if they were no longer ambulatory. This is a little more about the benefit and risk, my time is getting sure, I will go through quickly. I showed higher and lower and we looked at the side effects. We made it very simple, hopefully very easy to understand the patient's in the present we are asked to look at the survey and respond to it. This is an example of some of the questions that you can see. We feel that understanding risks and a community is another way to interact with the regulatory -- better understand the risk tolerances so when FDA is reviewing, they will have a better way to understand the community and make other choices or -- as they make decisions.

I will stop there. Because I think we are going to just 3:30 p.m. That doesn't leave much time for questions. We will stop there.

This is Steve, I have not got to speak much longer because we do have a little more time than what is allotted. the presenters are all able to stay and answer a few questions. I will open it up to everyone on the phone call. If you would like to ask a question please press star.

---

to withdraw the request, press star than two. to ask a question press star one. One moment please.

Patricia Carter you may ask your question.

I am a patient with my logic encephalomyelitis, I have been more than 27 with this. I do appreciate the FDA interest in this illness, however I am puzzled by the fact that you have provided us with speakers from two rare diseases, and E-letter is not rare. In the United States millions of people suffer from this illness. and, our problems are so different in from these people who spoke, that their presentations are practically useless.

For example, although I am a lawyer and I practiced for years, I have now homebound and virtually bedbound. I cannot attend these meetings, I can barely take care of my own needs. Our problem is not that this disease is rare, our problem is that it is rare for healthy people to even know that we exist. We get no help from our government and no help from our so-called patient organizations who exist solely to provide their own that salaries.

Now we have the FDA saying what we need is a patient registry. In my opinion this will lead to the seafood Association of America establishing a so-called patient registry where they will register all of these tired people who support them and the real disease of myalgia encephalitis myelitis. We'll be this or guarded as always. I would like to see the FDA times FDA or someone in our government take an interest in the real illness from which millions are suffering and people are dying every day.

Thank you.

Thank you for your comment, this is Doctor Greider. I disagree with you, I think the two speakers today are right on target, not because they were presenting rare diseases, but because they represent organizations that are advocating for disease for many years were invisible. Completely invisible. Just as you described your own situation. I actually would like to open it up to either pass or married, do you have any comments in response?

This is married to white, I would completely agree. Our model is not unique or uniquely applied to rare disease at all. In fact, any patient groups have copied our model that our -- represent significant populations. the applicability of this patient gender research model is about gathering the data that has to be there in order to demonstrate safety and efficacy and bringing the patient was to the very warm front to say what that means for your particular disease.

So I would disagree strongly that the CF model is only applicable to a rare disease.

This is Pat Furlong, I can imagine the frustration of having a disease that feels like no one is paying attention. In 1984 when my sons were diagnosed I felt exactly that way. No one was paying attention to the disease and it has taken now, nearly 30 years where we are. My sons died at 15 and 17. What is important for both of us to understand, with muscular dystrophy there is a population that progression is variable and the clinical care is variable. That makes it very difficult to understand. How is it that you understand with this drug is doing and if it provides benefits to the patient population. I think this is where the data is really required, the register is important because it is important to understand the natural progression. It is important to understand there are populations that are progressing differently and therefore when you design a clinical study to understand the effectiveness of the drug, you have the appropriate clinical outcomes.

I think there are so many common themes here, assisted at roses and rare diseases, we need to understand what we're looking at so we can gather the data so we can understand

how to improve the disease course over time. Otherwise we just keep, I can share with you in 1984, I felt like we had been -- we were just shooting arts trying drugs with no real scientific data and no real clinical trials, no care centers, no understanding of history and all of them failed.

This is variable, we need to study the natural history so you can understand how to impact the disease over time and how that will be useful. That is how I think we should, pathway.

Thank you very much for that.

Is there another question?

Our question is from Jennifer,.

Thank you very much for the presentation today. I want to go back to pants model of the ecosystem of drug development. Because I think there are a lot of players here, not just in the patient advocacy community, not just stakeholders in terms of drug development companies, but the other federal agencies as well.

CDC, NHS, the Federal advisory committee, also the various groups that serve people in this population, the researchers, clinicians and the individual patient advocates. So I would like to hear your comments on how all of these different players function together whether some of those players can have greater influence than others and in what step of the process and how we bring that whole landscape together to coordinate. Because that is what will speed up progress to the ultimate goal.

I love pats slide. I think it is fantastic. Sometimes our CEO, Bob Bell describes the CF model as forced collaboration. I think that is what he really can't when you boil it down to its very essence that that is really been the hallmark of one of the cystic fibrosis foundation has brought to the people, which is recognizing the players, as you so articulately said, the players are different in various stages of the game but the one player that is common throughout is the patient.

So our job is to make sure that the right folks are at the table talking to each other, sharing information, not being selfish and proprietary about it but moving the ball forward aggressively and no one knows that better than the patient. That has really been what is at the heart of our efforts and I would add that once you get to therapy than the players are at the table to, nobody is thinking about it holistically, really, except for the patient. So it is our job to subproblems, barriers and to bring the right folks to the table, hold their hand and jam them through the doorway until the solutions are identified.

I would echo those comments exactly. I think their are very different players at different times. They are all very important. the only way to ground of all is the patient's.

And is or another question? The next question is from Riley Silverman.

Thank you. It is not so much a question about much more of a statement. Over the years, the advocacy community, I am the founder of Pandora, we have begged, one can't complain, cajoled that patient registry be created for our community. Often I was told, not only by the CDC, that we are not there yet, it is not possible to do patient registry. I am delighted and grateful to see that both patient advocacy representatives are here today. It is so crucial how patient registries are and how important they are. Our community sees the immediate effect times effect of bullies and that context that there should be one boys, one cause, one community altogether pushing forward to get results and to get answers for our communities.

I am grateful to both of you patient advocacy representatives or being here and I'm delighted that the FDA is bringing this webinar to all of us today and I am eternally grateful to all of you. That is all I have to say. Thank you so much.

Thank you. I just want to give you a little insight as two how our registry was created. It was created I believe through clinical sites. So there's is a clinical registry. Just to give you a bit of insight on how that was developed, perhaps you're familiar with Jamie Heywood patients like me, his brother was diagnosed registry is times times, the registry is a recording registry, he thought these patients were not her well cared for. The disease was not understood in the progressive was not understood. His website is for them to communicate and two really share their experience, to share the progression and to share the things that they are trying. Jamie's idea was insufficient patients would get on this registry, and provide the data, then perhaps there would be some statistical relevance and they would be able to see where intervention was useful in these patients.

Muscular dystrophy involves children, that is not the sort of registry with the development we also wanted to have a much more robust registry because we had the use of steroids, cardiac meds, so there was a variety of treatments that some of the patients utilized and that is the clinical variability I spoke about. So we really look that a rigorous registry using that model and the CF clinical model and the patient reported outcome model. You are free to search that bottle.

Is available for searching and we want that to happen because we wanted not only academics and industry to search the data, we won the patients and families to be able to search the data and understand at a given age when times was a young boy might be taking, what interventions might be going on and one that of, might be. for instance you could look at a register and see how many boys were still walking and 14 years old. the ones that are what are they doing, physical therapy, etc.

Even beyond that we have prepared. Module for industry to use as the conduct vertical trials and that they can do that the base times phase for marketing study and at the end of the day, we want that data after a period of reasonable -- to be released to the patient because of the end of the day the patient stay to understand with the progression is, interventions, and what the interventions might be doing.

Thank you very much.

Our next question comes from Courtney Miller.

Hello, so I have a couple of questions, one is very specific. Which is who is the chronic fatigue syndrome representative on the advisory committee who is on the slide you listed where a whole bunch of illnesses have advisory committees. I would really like to know who that CFS representative is. On a broader point, this conversation has been quite frustrating in that much of this premeditation has been given in the past. I appreciate the time that the patient advocates for other illnesses have put into sharing their stories, but we need the FDA to move us to a process where there is give and take with experts in the room.

We do have experts, we do have data how we do have endpoints, we do have measurements and we have one drug which has been in studied for more than 1 meters -- 20 years. It is all in the hands of the FDA at the moment. It is a huge resource to be able to study what works in the truck that has yet to be approved.

Cooler works for, why it works for and what why. We need to add FDA process here that puts people who are those experts into a room so we can advocate from the patient side but we can advocate from the expert side as well. My question is, when are we going to move to that process?

I could tell you that first thing that will happen, this is Doctor Greider, there will be a public advisory committee to review the data and that is scheduled for December 20th. Secondly, we are in the process of developing a more scientific -based workshop that brings together experts to review the current date of this science and what the things might be to address the issues you have raised.

We are planning map of the times times for the spring of 2013. Early spring 2013.

Who is the patient representative?

Right now, the person is under the review for the SGE process. Until that happens, we can put not put that information out. Once times once it is finalized, we can. Once the background information goes out, a patient representative will be a public announcement at that time. That is usually two weeks prior to the meeting.

I am sorry I cannot give you more than information than that. But you can check out the FDA website under the advisory committee page and there is background information for each of the meetings coming up.

Richard, I wanted to point out, one of the things that happens for the advisory committee coming up, the public has complete access to all of the information that the advisory committee itself has access to. All of the reviews, all of the materials, all of the documents that were submitted, it is not the same as the raw data from the application.

That is a room full. It is information that is summarized for review by the experts on the advisory committee. The company provides impact is on the FDA also provides a background package that includes our scientific reviews that are relevance to the questions on the table. For instance, there may be some things that we reviewed the tonight discussed such as details of the manufacturing process. That would've been part of what is available are discussed. All of the things of clinical relevance will be a part of that. You will be able to see a day or two in advance of the meeting an opportunity to live times live through that so if you're at the meeting, I am not sure if it will be telecast marks.

I was wondering to.

If you are listening you will be able to have the same background materials that the committee does.

After the meeting all of the transcript of the meeting goes on the website on that same page.

Before we take our last question, this will be available as soon as I put the slides with the audio together for everybody to listen to. It will be on a website. I really do appreciate everyone taking the time and staying longer and answer questions. I hope this is helpful.

Hillary Johnson, your line is open.

Hello, I have a couple of questions. But I didn't want to say I think it is a shame that given your statement in how interested you are in patient participation that you have given patients so little time in the last two hours to comment.

A quick question, one issue with this disease over the past 30 years, there have been numerous small clinical trials conducted by scientists, clinical researchers, at academic institutions, such as Stamford and I -- yet these studies come and go. They show efficacy and yet days -- these anti- -- they are not approved by FDA in use for these diseases. Hundreds of thousands of CF patients I could benefit from these antivirals don't get any kind of option to take them because Medicare won't pay for them. This has become over the years, a disease relief for rich people. You have to have a tremendous amount of money to buy, to pay for drugs that are well-known to be helpful. The problem is most people cannot afford them.

I second question is why is FDA suddenly interested in this disease in interested in the people when they have been so uninterested for the last 30 years.

---

the way that drugs get approved by FDA is that someone has to bring data, usually the company has to request and make a decision that the research is substantial enough that they would like to bring it to the FDA to have what would be called a supplemental review and to add a used their product label.

So let's say the drug is aspirin. That is a good example because it doesn't have an HR application.

How about Bell site.

the company that makes Bell site would have delivered the data in CS, we think there is really something here and this is good scientific data likely to pass muster and we will bring it to the FDA and seek the addition of the indication of CF base -- CFS or whatever was studied for addition. That is the application then kicks the FDA review process into gear. Sometimes FDA earns about studies and independent of the company, will go to the company and say, we think this is an important study. Why don't shoot see if you can get more information, more data so that we can examine it and we will talk about whether you might submit an application or supplemental application.

That is the process. Why in the case of drugs you are thinking about that that has not happened, I am not familiar enough to answer that question but I think that is something for the researchers. What did the researchers take from that.

Should patients of five pressure to the researchers who did the study or apply pressure to the companies that make the drug?

Both.

Both.

That's all.

to answer question why FDA is suddenly interested. We, there is not a particular reason why, over time we have been working very hard to engage more and better in trying to bring attention to conditions that are important to patients. with that as background, and some of our newer staff who are more engaged in the department work in partnering with our colleagues in other government agencies have really, bringing people to the right place at the right time and saying this is a community we would like to engage better with because we think it is important and we would like to do what we can to help. As Richardson, we don't have all the answers, we can't turn things around on a dime, but we want to encourage patient groups to advocate for themselves and we will help as much as we possibly can for that is the honest answer. Nothing fancier than that.

If FDA approved and diligent, will Medicare approve it for use in the treatment of CF. and patients that don't have $60,000 a year to pay for our will we get access to that?

I cannot speak for Medicare or we -- Medicaid. What FDA approval means is that the drug can lawfully be marketed. We don't have any role in setting the price. Other countries that are equivalently do, we are specifically prohibited from doing so. While

CMF reviews our work, we don't have any ability to tell CMF what they must do or must not do.

This is Mary Dwight with the CF foundation. I will echo that last statement is one, they are two separate entities and as patients you need to think about both. But.

You mean Medicaid and Medicare as two separate entities?

I would just say, FDA approval does put a drug and eight different ones for most cares.

Absolutely.

Thank you very much.

Thank you all very much. I will be sending out an e-mail to everyone who registered with the link to where we will be putting this webcast as well as an e-mail address. If you have specific questions about advocacy or where to go, there are ways we can help direct you in the right direction. I will be sending that out horribly tomorrow. I want to thank all of the presenters, all the people on the call and all the questions we have hopefully this was helpful to you all. Have a great day and.

This concludes today's conference call. Thank you for attending. [Event concluded]