FOOD AND DRUG ADMINISTRATION (FDA)

CENTER FOR DRUG EVALUATION AND RESEARCH (CDER)

SICKLE CELL DISEASE PUBLIC MEETING ON

PATIENT-FOCUSED DRUG DEVELOPMENT

Friday, February 7, 2014

Food and Drug Administration

White Oak Campus

10903 New Hampshire Avenue

Silver Spring, Maryland 20993

Reported by: Natalia Thomas

Capital Reporting Company

M E E T I N G R O S T E R

FDA STAFF

Jonca Bull, MD
Director, Office of Minority Health
Office of the Commissioner
FDA

Sara Eggers, PhD
Office of Strategic Programs (OSP)
CDER, FDA

Ann Farrell, MD
Director, Division of Hematology Products (DHP)
CDER, FDA

Lisa Faulcon, MD
Medical Officer, Division of Hematology Products (DHP)
FDA

M E E T I N G R O S T E R

(Continued)

FDA STAFF

Theresa Mullin, PhD
Director, Office of Strategic Programs (OSP)
CDER, FDA

Anne Pariser, MD
Associate Director for Rare Diseases
Office of New Drugs, CDER, FDA

Kathy Robie Suh, MD
Division of Hematology Products (DHP)
CDER, FDA

Pujita Vaidya
Office of Strategic Programs (OSP)
CDER, FDA

M E E T I N G R O S T E R

(Continued)

FDA STAFF

James Valentine, MPH
Office of Health and Constituent Affairs
FDA

Nicole Verdun, MD
Division of Hematology Products (DHP)
CDER, FDA

PUBLIC PARTICIPANTS

Kamilah Bailey
Lakiea Bailey
Tara Bailey
Olga Barnwell
Terri Booker
Anthony Braxton

M E E T I N G R O S T E R

(Continued)

PUBLIC PARTICIPANTS (Continued)

Velvet Brown-Watts
Adam Bundukarma
George Harris Carter
Bill Cummings
Farron Dozier
Marquita Gaines
Margaret Hadnott
Lewis Hsu, MD
Tina Kay Hughes
Edward Donnell Ivy, MD
Judy Gray Johnson
Ibrahim Kargbo
Mary Bentley LaMar
Marybeth McAfee
Alana McClinton
Sameka McNeil
John Moore

M E E T I N G R O S T E R

(Continued)

PUBLIC PARTICIPANTS (Continued)

Gwen Morgan
Theresa Murphy
Dawn Nelson
Tosin Ola
Maisha Pesante, MD
Nikki Peterson
Nancy Rene
Mattie Robinson
Gloria Rochester
Helen Sarpong
Jocelyn Stinson
Jay Sweet (ph)
Johnnie Tidwell
Francesca Valentine
Marqus Valentine
Andrea Williams

M E E T I N G R O S T E R

(Continued)

PUBLIC PARTICIPANTS (Continued)

Sitrena Woodson
Teonna Woolford

Amorilla (ph)
Carol
Cassandra
Jonathan Posiko
Nicole
Nikki
Yomi (ph)

T A B L E O F C O N T E N T S

T A B L E O F C O N T E N T S

(Continued)

T A B L E O F C O N T E N T S

(Continued)

T A B L E O F C O N T E N T S

(Continued)

P R O C E E D I N G S

Welcome

DR. EGGERS: Good morning, everyone. We're going to get started in a few minutes, so if I can ask everyone to start to move to their seats.

All right, I think we can get started, and as people join a little bit late, they can just feel free to come on in.

We are very excited to have our fifth Patient-Focused Drug Development Meeting on Sickle Cell Disease. We have done a lot of preparations, and Ann in a few minutes is going to give a welcome and do the proper thinking.

But my name is Sara Eggers, and I will be the facilitator for today, and I just want to go over a few housekeeping and agenda items. Before I do that, I do want to have my colleagues, my colleagues from FDA, introduce themselves. They'll be sitting up here throughout the meeting today.

DR. FARRELL: My name is Ann Farrell. I am the Division Director of the Division of Hematology Products in the Center for Drug Evaluation and

Research.

DR. VERDUN: My name is Nicole Verdun, and I am Hematologist and Medical Officer here in Dr. Farrell's office.

DR. ROBIE SUH: Good morning. I'm Kathy Robie Suh. I'm one of the Medical Team Leaders in the Division of Hematology Products.

DR. FAULCON: Good morning. My name is Dr. Lisa Faulcon. I'm a Medical Officer in the Division of Hematology.

DR. MULLIN: Good morning. My name is Theresa Mullin, and I direct the Office of Strategic Programs in the Center for Drugs.

DR. BULL: Good morning. My name is Jonca Bull. I'm Director of the Office of Minority Health in the Office of the Commissioner.

DR. PARISER: Good morning. I'm Anne Pariser. I'm the Associate Director for Rare Diseases in the Office of New Drugs.

DR. EGGERS: Thank you very much.

I'm going to ask for the agenda slide.

So let me go over briefly what our day will

look like today. We have a jam-packed day, and I'm happy to say that most of it is focused on listening to patients, caretakers, and advocates of people with sickle cell disease. After some opening remarks and some brief background context setting by my FDA colleagues and myself, we're going to go right into the discussion.

Our first discussion topic is on the health effects of sickle cell disease that matter most to you, and we're going to start that discussion by looking at the pediatric and young adults, so people about 22 and under, and then we're going to have a discussion, the same discussion, with adults, about 23 and older.

Then we will go to lunch, and after lunch, we'll come back and have Topic 2 discussion in the same format, and I'm going to go over the panel format in my talk later on, so you'll have an idea of how that will run. The second talk will be on your perspectives on treatments for sickle cell disease.

Following that, after we have those two discussion topics, there will be an Open Public

Comment, which will give anyone here, not just patients, but everyone, a chance to talk about other topics that may not have been within the scope of the first two topics.

And then following that, Kathy will give some closing remarks and we'll be done.

This meeting is being recorded today, both it will be on the webcast, and I want to give a huge shout-out to all the people who are on the webcast today, you play an important part of this meeting, and so we welcome you as well. The webcast is going to be streaming today. It will put up, it will be archived and posted on the website some days after the meeting. There will be a transcript, so everything that is being said is being captured today, and that will also be put on our website.

There are restrooms about as far away as you can be in this building, but they are just down this hallway, and then if you go to the end wall and go to the right, you'll see the restrooms. There is a kiosk where you can buy basic food, sandwiches and stuff, during lunch or anytime you want. And I do encourage

you, if you need to get up and move around, please do so. This is a very informal setting. There is a hallway, there are some tables out there, if you need to stretch your legs and walk around or if you need to go use the restroom or get a snack.

I think that's it for the agenda and housekeeping. If you have any questions, my colleagues with name tags are sitting up here and around, just come find one of us and we will be happy to help.

With that, I will turn it to Ann, who will give a few opening remarks. Thank you.

Opening Remarks

DR. FARRELL: Thank you, Sara.

Good morning and welcome to this meeting on Patient-Focused Drug Development for Sickle Cell Disease. This is an important meeting, and we are delighted to hear today from patients about how they think about sickle cell disease. My name is Ann Farrell, and I am the Division Director for the Division of Hematology Products in the Center for Drug Evaluation and Research. Previously, I was in

university practice where I was a hematologist oncologist taking care of patients with sickle cell disease.

I see we have a full room today, and I would like to thank all our panelists, patients, their families and caregivers, the advocates, and the numerous advocacy groups who are here today, the pharmaceutical industry, health care professionals, academia, NIH, CDC, and other government partners, the press, and interested observers for dealing with the recent Washington Metro weather and coming to today's meeting.

I would also like to thank all of those who are joining remotely via the web. I'm delighted to see a high level of interest from those of you who play a very important role in the drug development process. Thank you very much for coming here today and being part of this meeting.

A major mission of the FDA is to ensure the availability of sfafe and effective medicines to the American public. The FDA is also responsible for advancing public health by speeding innovations that

make medicines more effective, safer, and more affordable, and by helping the public get accurate science-based information that patients need to use medicines effectively. While we at the FDA are part of the process, we are just one part of the process. FDA does not develop medicines or treatments for sickle cell disease nor do we conduct clinical trials.

CDER's Division of Hematology Products oversees the development and approval of medicines like hydroxyurea, which are used to prevent or treat the complications of sickle cell disease. While we do not develop medicines or treatments, we work with our partners to facilitate the research and development of safe and effective medicines.

Can I have the next slide, please?

Drug companies or manufacturers work with academic investigators and research and patients to conduct the necessary studies and clinical trials to submit applications for new drug products to the FDA. It is then the FDA's responsibility to ensure that the benefits of a drug outweigh its risks. The path a drug takes travels from the laboratory testing to your

medicine cabinet can be a long period and each drug takes a unique route.

Typically, this work is done through a process that involves formal application to the Agency and investigational New Drug Application. Sponsors or manufacturers typically do initial preclinical animal testing. This is followed by manufacturers showing the FDA the results of the animal testing and making an application for human clinical testing. After considering the results of the animal studies and negotiating with the sponsor, the FDA decides whether it is reasonably safe to allow the conduct of human clinical testing with various agents. The testing in humans is a gradual process of the accumulation of safety and effectiveness data sometimes involving different populations, different dosages, and using different drugs sometimes in combination.

During this process, the FDA will meet with sponsors on several to many occasions as they test their product to discuss the findings and the path forward and hopefully the path to eventual approval for widespread use.

How often the FDA meets with a sponsor varies. Along the way, some drugs are found to be too toxic and are never tested in humans. Other drugs are discovered to be toxic in early clinical trials and are never fully developed.

When enough data has been accumulated, the manufacturer submits an application to the FDA, this is called a New Drug Application to the FDA, to ask that the FDA consider approving the new drug for marketing in the U.S. The submission will contain all available animal and human data as well as information on manufacturing. The FDA then will perform a rigorous evaluation process looking at all aspects of the drug, including the manufacturing, inspecting sites, and the clinical trial data and analysis of trial conduct, and then we'll make a decision about the application and whether the manufacturer has submitted enough information to allow the marketing, which would be the widespread distribution and sale of the product.

All of us attending this meeting have different but very important roles in the development

of effective treatments, from manufacturers who develop the product and perform the initial preclinical testing and work with the researchers and investigators and the Agency to design the clinical trials, the patients who participate in the clinical trials of experimental therapies, and the FDA, who will eventually make a decision regarding the product.

Pharmaceutical companies synthesize and manufacture agents to target those effects or identified causes of those effects, working together with clinicians and manufacturers, develop clinical trials to test the promising agent in patients. And patients, their families, and advocacy communities engage and participate in those trials to allow the verification of the beneficial effects of the drugs and help to understand what side effects can be expected.

We're very happy to be having this meeting today on sickle cell disease. As many of you know, we have a scarcity of products approved to prevent or treat the complications of this disease. Sickle cell disease is a chronic and debilitating disease that

affects patients all of their lives. The treatment of sickle cell disease remains an area where there is a large unmet medical need. Hydroxyurea is not 100 percent effective and transplant is not an option for everyone. Hence, we are interested in getting patients' perspective, patients' families' perspective, caregivers' and advocates' perspective on the aspects of their disease that affects their lives the most. This is an area where we really want to hear more from patients about how you experience the disease and how it affects your life, and what you would like to see in a potential treatment that might be approved.

Can I have the next slide?

Having this kind of dialogue is very important for us. Hearing about what you care about can help lead the way in figuring out how to best facilitate drug development for sickle cell disease. You can help us to better understand how endpoints that reflect the aspects of the disease that bother you the most can be incorporated in clinical trials to better define the benefit. And I'm talking about

patient-reported outcomes, how you feel, your symptoms, those items that are most important to you.

We hope this meeting will be the first of many successful collaborations leading to the development and approval of effective therapies for sickle cell disease. Thank you very much for your participation here today. I will now turn it over to Theresa Mullin, who will provide the background on the FDA's Patient-Focused Drug Development.

Thank you.

Overview of FDA's Patient-Focused Drug Development Initiative

DR. MULLIN: Good morning. I'm going to take a few minutes to tell you about this broader initiative, and we are very happy that sickle cell disease is one of the disease meetings that we're including in this initial set of 20.

And we can go to the next slide.

So this initiative began with FDA making some basic observations, that really patients are uniquely positioned to give us a better understanding of the clinical context of the disease, and that would

be critical to our benefit-risk assessments of new drugs. And we would also benefit from a more systematic approach to getting that kind of input on the severity of the condition from the patient's point of view and also the impact on their life and how they feel about the treatments that are currently available, and do that outside of the decision making around a particular drug, really try to do this in advance of and as a separate effort where really it's all about hearing what patients think because that would provide us a wonderful reference point for any applications that come in, including new drug investigational applications, as Dr. Farrell was describing, early on in development. It would help us in a much broader way. And the mechanisms we had available before this initiative really only gave us an opportunity to get that input in the context typically of an advisory committee meeting or when a particular product was under consideration, and there are all sorts of constraints that that places on our ability to get input. So we thought this was really an unmet need that we had.

And so in 2012, as part of the reauthorization of the Prescription Drug User Fee Act, FDA committed to and built into our performance goals for ourselves development of a more systematic approach and one that would allow us to get that patient perspective and inform our understanding of their view of the context of the disease, which is, after all, it's the drugs are being made for patients, they are going to the experience the benefits and also the risks. So getting that input would really help us to do a better job of protecting patients and reviewing those applications.

And so we committed to at least 20 diseases in different specific disease areas and that we really view this as the first 20 where, quite honestly, we're really learning how to run these meetings or how to get this kind of input, even getting remote input and making the best use of those technologies to hear from as many people as possible. Not everyone can be here today. We're thrilled that you were able to make it to this meeting and that the people on the webcast are able to join. And so we're learning how to do this,

and that's what these meetings will do, giving us this opportunity.

And so how do you choose that 20? There are so many diseases where patients have told us they need better treatments. And so to come up with an initial set of 20, we developed some criteria, we asked our review divisions to consider these criteria, we put these in a *Federal Register* Notice as well to ask for public input, and here is the set that we came up with to help us shape up an initial 20: diseases that are chronic, symptomatic, and affect functioning and activities of daily living; ones where there are important aspects of the disease that are not being formally captured in clinical trials today; ones for which there are no therapies or very few therapies, and the therapies don't address all of the most critical symptoms or perhaps any of them in terms of how patients feel, function, or survive. We wanted to get diseases that reflected a range of severity across patients affected where possible to understand that better and also look at diseases where there might be a particular subpopulation that is more affected by

the disease. And, finally, as a set of 20, we wanted to capture a range of diversity in terms of the size of population affected and the kinds of input that we might get.

And so we came up with our initial list, it was almost 40. We published that FDA-generated list in a *Federal Register* Notice in fall of 2012, and we had a public meeting in October of that year and it was very well attended. We received about 4,500 comments from the public docket. In those comments, 20 -- or, rather, 90 diseases were identified as being ones we should be considering and of interest. And so we very carefully sifted through all this input, went back to the review divisions, talked to them about, "What should we focus on first here?" And what we've come up with so far is 16 diseases for the first 3 years. We're going to come back and try to determine which diseases to pursue in the final 2 years of this 5-year program later on.

And so on the next slide we have the diseases that are identified so far for the first couple of years, and as you can see, sickle cell is

our first disease for this year, this calendar year. And so for each of these meetings and looking at the range of diversity of those diseases on that slide, you can see we really need to further tailor each of these meetings to some of the particular issues and the experiences of the population of patients who are experiencing that disease.

And so we have some standard questions that we'll be asking you today that we ask at each of these meetings related to the severity of the condition and your experience with it and how it's affecting your life and how the treatments available are working for you. We also bring in questions that the review divisions have asked to have included because there may be particular concerns or things that they see coming up in their reviews and they would like us to try to take the opportunity, the sort of unprecedented opportunity we have here, to get your input on those as well. So some of the questions or probing that we do today is to help the reviewers. And the review division staff come to these meetings to hear, it's a wonderful opportunity for them, and so we'll have

questions of that kind as well.

And as I mentioned earlier, we're also trying to figure out how to use technology to poll and get responses to different questions, using interactive questions for the webcast to be able to get input from those who are participating remotely as well.

But common to all these meetings, we have found that patients and the caretakers and the patient advocates who come provide us with very insightful and powerful messages about what it's like to live with this disease and really have been invaluable to us. And the stakeholder involvement, even in preparing for these meetings and planning for them and gathering the input and outreach to other patients with the disease has been critical to the success of these meetings.

And, finally, what do we do with the information that we get in these meetings? Well, one thing that we will do is develop a meeting report which tries to capture very faithfully what we heard from the patients who come to the meeting or provided us with input and who have sent information into the

docket as well. We keep a public docket, which is an electronic docket, so people can submit comments to that as well. And we analyze all this information and really try to make sure we keep the input in the words of the patients, use the language that they use, because we don't want to try to translate it, that's really authentically the input that we received, and that's one input. And the other, as Dr. Farrell was saying, is an opportunity for us to get a start on perhaps development of the patient-reported outcome measures that can be used to help capture more information in clinical trials to see if drugs that are being tested are addressing some of the symptoms and the concerns that we've heard from patients. So that's another longer term result that we can get from these meetings.

And with that, I'll stop and I'll turn it over to our next speaker. Thank you.

Background on Sickle Cell Disease and Treatment

DR. VERDUN: Good morning, everyone. My name is Nicole Verdun, and I'm a hematologist here in the Division of Hematology Products in the Center for

Drug Evaluation and Research. And I also have treated several patients with sickle cell disease. Many of my mentors are either in the room or participating via webcast. And so we welcome you and we look forward to this discussion. And with that, I will get started.

I have been tasked with giving a very broad overview of sickle cell disease in 10 minutes. You can feel free to laugh because that's, of course, difficult to do, but here we are.

So the next slide. Thanks.

So I will be giving a definition of sickle cell disease, discussing a little bit about the genetics, the complications, and treatment.

So sickle cell disease truly is a global health problem with 100,000 people affected in the United States and millions affected globally. Sickle cell disease occurs in 1 in 500 African American births and in 1 in 36,000 Hispanic births. And sickle cell trait affects 1 in 12 African Americans.

Our goal here at the FDA is the development of safe and effective treatments for preventing and reducing the complications of sickle cell disease.

Sickle cell disease is a multisystem disease associated with episodes of acute illness and progressive organ damage, and red blood cells change to a sickled shape in the presence of decreased oxygen and inflammation. Sickled red blood cells and white blood cells then become trapped in small blood vessels or the microvasculature.

Normal hemoglobin consists of two alpha-globin chains and two beta-globin chains, and hemoglobin S results from a point mutation changing the 6 amino acid in the beta-hemoglobin chain from glutamic acid to valine.

Sickle cell anemia, or homozygous SS, accounts for about 70 percent of sickle cell disease. There are several other forms of sickle cell disease that result from a coinheritance of hemoglobin S with other abnormal beta chains. So for example, sickle cell disease SC, S beta 0, S beta plus, SO-Arab, and SD.

There are significant differences in severity and complications that are somewhat attributable to genes and the type of sickle cell

disease. So, for example, sickle cell disease SS and S beta 0 has a different phenotype than SC and different from S beta plus, but even within the same family or mutation, there are differences that exist. We do know that there can be coinheritance of other genetic factors that modulate the disease such as alpha-thalassemia and the hereditary persistence of fetal hemoglobin.

There are many sickle cell disease complications that can occur as early as infancy and continue through childhood and adulthood, and this is a not comprehensive list but does list several of those complications. Some of them are a direct result of the disease and others are a consequence of needed therapies such as iron overload.

Recurrent episodes of blood vessel occlusion and tissues not getting enough oxygen can result in progressive damage involving most organs: so the bones with complications such as avascular necrosis, the lungs can have restrictive lung disease over time, hepatopathy, kidneys, brain, retinopathy, cardiovascular system problems. Chronic hemolysis can

result in varying degrees of anemia, jaundice, fatigue, gallstones, delayed growth and sexual maturation, and progressive damage to blood vessels. And increased rates of hemolysis can predispose people to pulmonary hypertension, priapism, and leg ulcerations.

One of the most common causes of stroke in children is sickle cell disease, and damage to blood vessels in the brain can start in infancy. Some people can have a progressive vasculopathy with recurrent strokes despite a transfusion program. Silent brain infarcts are recognized to have problems with neurocognitive deficits. Intracranial bleeds can begin in the twenties and thirties with moyamoya-like syndrome, cerebral aneurisms. Treatment is largely neurosurgical and limited in its scope and its ability to have an effect.

Acute chest syndrome is a form of acute lung injury with significant morbidity and mortality associated, and it's the second most common cause of hospitalizations in sickle cell disease outside of acute pain crises.

People with sickle cell disease can have chronic damage to blood vessels in the kidney that can start at a very early age, and adults can develop chronic renal failure and require renal transplants.

Sickle cell disease can also affect pregnancy. The manifestations can vary. Some people with sickle cell disease will have an increase in acute painful episodes, an increase in the risk for thrombosis or clot formation, infectious complications, cardiac complications, and low birth weight.

The current treatment paradigm for sickle cell disease is a combination of preventive and supportive with each patient falling a little bit different in terms of where they are for prevention versus supportive based upon what's available for their individual disease.

Hydroxyurea was FDA approved in 1998 with an indication to reduce the frequency of painful crises and to reduce the need for blood transfusions in adult patients with sickle cell anemia with recurrent moderate to severe painful crises. The uses for

hydroxyurea continue to grow in scope and are much broader than the indication. It works very well for some to decrease complications and has actually been shown to increase survival. The mechanism of action is not completely understood. Several people have an increase in hemoglobin and a reduction in hemolysis. There is an increase in hemoglobin F production, which allows red blood cells to live longer. Decreasing inflammation has been seen, and dilation of the blood vessels as a result of nitric oxide metabolism.

Other preventive treatments that are commonly used are penicillin prophylaxis, timely immunizations. The pneumococcal vaccine has really revolutionized infections in sickle cell disease. The influenza vaccine is very important. Folic acid is often used due to increased red blood cell turnover. Chronic red blood cell transfusion therapy can be indicated for some patients. All of the indications are beyond the scope of this talk. And although not a treatment, ongoing education of caregivers and patients is essential. And a lot of surveillance is done: Transcranial Doppler ultrasounds,

echocardiograms, eye screening, urinalysis, et cetera, and monitoring for growth and development.

Stem cell transplantation can be curative, but there are significant risks during and after transplant that have traditionally limited its use to those with significant complications. There are also problems with finding match donors. Continued improvements in immunosuppression in the management of transplant-related complications are ongoing, and the criteria for consideration of a transplant are constantly changing.

I had to mention some of the limitations of the preventive treatments that are used. For example, chronic transfusion therapy, although effective for some, can have problems with iron overload, antibody formation, transfusion reactions, and infections, which limit their use in some patients.

And hydroxyurea, although a wonderful drug for several people, is not universally effective. There is laboratory monitoring that's required, you can have myelosuppression, and it can be harmful during pregnancy to an unborn baby. So we really do

need further development of safe and effective treatments for sickle cell disease.

Treatment of complications is often a combination of antibiotics. Blood transfusions may be indicated in the acute setting. Surgery. Pain crisis management is largely supportive with a combination of hydration management, oxygen, anti-inflammatory agents, and pain medications. And acute chest syndrome management is also quite supportive. So really are here at the FDA interested in continuing to switch the treatment paradigm from supportive to preventive or curative.

So what is the future of sickle cell disease treatment? Well, there are several clinical trials that are in the planning stages or in process, but it really is not enough. We really do need more development to have the global impact on sickle cell disease that we need and that we feel is overdue.

Thank you.

(Applause.)

Overview of Discussion Format

DR. EGGERS: Thank you very much, Ann --

Nicole, and Ann, and Theresa.

It's my pleasure to get the discussion started. I'm going to go over a few basic things about what our discussion will look like today, and to put you all at ease about how the day is going to run.

Can I have the next slide, please?

We have our two topics, we've gone over those a bit today, but I'll just briefly go over those again. The first topic is the health effects that matter most to you, and by "you," I mean patients, and if you're here as a caretaker, if you can speak on behalf of your loved one. What matters most to the person who lives with sickle cell disease?

We're going to discuss the pediatric and young adults first, and then we'll have a separate discussion on adults. So during the pediatric discussion, adults, please stay silent. And then during the adults, we'll ask the pediatric and caregivers to be in listening mode as well. Hopefully, you'll learn a lot.

We want to know what the specific ways that sickle cell disease affects your health. We want to

hear about your average days with no acute pain crisis and your worst days when a pain crisis hits, and we're going to split those two topics up a little bit.

And then in the afternoon after lunch we'll come back and talk about your perspectives on the treatments that Nicole just described. What are you doing to treat your disease that includes those prescription treatments that she mentioned but also the range of other lifestyle -- other therapies that you do to try to manage your condition? How well do these treatments work for you? What would you look for in an ideal treatment? And what might you think about if you had a chance to participate in a study for an experimental new treatment?

So for each of those topics that I just discussed, we're going to first hear from a panel of patients and caregivers, and the purpose here is really to set a good foundation for our discussion. We have asked each of the panel members to give 2 to 3 minutes of their story. Now, we all know that our stories could take hours to tell, but we've asked them to limit it to 2 to 3 minutes, and I'll nudge them

along if they start to give a little bit more than that. But they're all very willing and they're happy to oblige so that we can get to our facilitated discussion, and that's the discussion where I'll come out and in more talk show style engage the rest of you in the audience, those who have sickle cell disease, care for someone with sickle cell disease or are an advocate of sickle cell disease patients. The purpose here is to build on the experiences that were shared by the panel.

So we're going to ask a series of questions and invite you to raise your hand to respond, and we have Andrea and Soujanya will be coming around with microphones. This is very talk show style, it's quite novel for FDA. So we're going to invite -- please raise your hand to respond. We have a huge crowd today, and I love to see that. It makes it a little bit of a challenge to try to get to everyone, but we will, we'll try to do our best, if you want to contribute, to let you do so. Please state your name before answering so that we can capture that. You just need to state a first name, that's fine.

You'll also have a chance to answer polling questions, and here I'm talking about the people on the web as well as the people in person, and I'm going to ask that the little clickers that we're going to use in person to be handed out. So if you are a patient or a patient caretaker, please raise your hand, and we're going to have these little clickers come out. We're going to practice in a bit on how to use these. You just have to click the button to respond to the right answer.

On the web, it's a little bit easier for you, I think. There is Adobe on your webcast. You'll be able to click on the response that you want. Sometimes you'll have to scroll down because the responses won't always fit on the screen, so just scroll so you see all the possible choices.

So, web participants, you can also add comments through the webcast, and although we may not -- we won't be able to read all of the webcast comments today, but we will summarize those as best we can. They are important. We review all of them. And we will incorporate them into our report.

We'll occasionally go to the phones to give you another opportunity to contribute, too, on the web. The phone line information will be provided on the webcast screen at the right time.

You can also send us your comments. We very much want to hear what you have to say. If you have friends who weren't available to join in the meeting, have them send a comment. If you heard something today that really you want to comment more about, you want to describe in more detail, please send in a comment. If you want to describe your story, like the panelists are, your full story, please send in a comment. We review all of them. They're very important to us. The comments will be included in our summary report.

And I've just shown the website here that you can visit to go find it. It's on our meeting web page, and if you click on the "Comment Now" button, it will take you to a spot that you can upload your comment.

There are a few ground rules that we have to make sure the discussion is as beneficial as possible

and as fair as possible. We very much encourage patients to contribute to the dialogue. Caretakers and advocates, you are very welcome to contribute as well. I hope you feel comfortable to contribute. You're in company of your fellow patients and in people who find drug development for sickle cell disease very important, so please feel free to contribute.

FDA is here to listen. My colleagues are up in the front and I know we have other FDA colleagues scattered in the room. Because we're in listening mode, we probably can't answer all the questions that you may have for us. There are a number of other topics. If we can't answer a question -- we won't be able to answer very many questions, and in that case, if you want to put your question in the docket comment, we might not be able to answer that directly either, but at least we know what questions you have and what things you want us to be thinking about and addressing when we talk to you in the future.

Our discussion today will focus on health effects and treatments. We know that there are many

issues related to the care and support of people with sickle cell disease, and this doesn't mean that these issues aren't important. We do want to focus on the things that FDA can really manage and think about as we continue our role in drug development. There is an Open Public Comment period, and if you haven't signed up already, you can sign up at the registration table. It's first come, first served, so if it's filled up when you want to register, again that public docket, send us that comment with your comment, with your thought on another topic, too. We will be glad to read those.

The views today expressed here are personal opinions, and we want to respect everyone's personal opinions. I know that they are each individual persons. So maybe you hear something that doesn't quite reflect what you think about sickle cell disease, just remember that's their personal opinion. And, of course, respect for one another is paramount.

Also let us know how we're doing. There are some evaluation forms at the back, and we find those important. They will really help us as we continue to

improve on our patient-focused meetings.

With that, I think we get to go into some of the clicker questions. I want to remind you, these clicker questions are going to be some demographic questions and then later on some discussion questions, and they are not scientific. This is not a scientific survey that we're doing. This is just to aid in discussion. It's completely voluntary, but again we encourage you -- it's anonymous, we encourage you to contribute to the polling questions. If you need any more clickers, just let us know at the front.

Can we go to the first clicker question? Okay. The first one is easy, we hope. It's: Where do you live? Do you live within the Washington, D.C., area, including our suburbs? You would click "A" if you are in the room, you click "A" on your clicker, and on the web, you just hit the right choice. Or do you live outside the Washington, D.C., metropolitan area?

(Answering question.)

DR. EGGERS: I see we still have some clickers going around, so I'm going to give a few

minutes.

And I will say, there are some empty chairs toward the front over here. If you're a patient or patient representative and you want to move up, it does make it easier for us to see you if you raise your hand to participate, so feel free.

Okay, let's go to the responses. If you missed that first one, it's our least important question. Okay. So it looks like two-thirds of you came from out of the area.

(Applause.)

DR. EGGERS: It's difficult to travel around D.C. if you live in the area, but if you came from outside of the area, our special thanks.

Okay, the next one, please.

Which of the following best describes you? Choose all that apply. A, I have sickle cell disease; B, I am a family member or caretaker of someone with sickle cell disease; C, I work for a sickle cell disease patient support or advocacy organization; D, I'm a health care professional who works with sickle cell disease patients; and "Other."

And while you're getting that, let me just remind you, I'm sure not all of the health care providers, professionals, and advocates got a little clicker. Don't worry. This is not going to be seen as the be-all, end-all of who is in the room.

(Answering question.)

DR. EGGERS: Okay. Can we go on? Okay.

So does this give us the numbers at all? Okay, that's fine. We have a lot of people here who have sickle cell disease and so many family members and advocates. It's just wonderful to see you.

(Applause.)

DR. EGGERS: We're going to be doing a lot of applauding today, I think, I have a feeling.

Okay, let's move on. Now, for the rest of these, we really just want the patients and caretakers, and by "caretakers," I mean someone who is here on behalf on someone who has sickle cell disease, they aren't here and they aren't answering for themselves. So if your son or daughter is here answering for herself, please don't answer the polling questions. Okay.

So what is your age or your loved ones age? Zero to 5; B, 6 to 12; C, 13 to 17; D, 18 to 22; E, 23 to 49; or F, 50 and greater.

(Answering question.)

DR. EGGERS: Great. It looks like we have a diversity in the younger ages. We split those out because we wanted to know a little bit more in detail the ages. A lot of adults 23 to 49. But I want to give a special shout-out to the folks 50 and greater. It is fantastic to see you here.

(Applause.)

DR. EGGERS: Pujita, can I have on the web? Do we have -- or James.

MR. VALENTINE: Can you hear me?

DR. EGGERS: Mm-hmm.

MR. VALENTINE: Yeah. So we actually have some similar numbers for ages, except we have a little bit higher for 23 to 49 were almost 60 percent of the participants, and then we have about the same, about 27 percent, for 50 or greater.

DR. EGGERS: Great. Thank you. Okay. Moving on. Okay. Is your loved one male or female?

(Answering question.)

DR. EGGERS: Okay, we won't give as much time for this one. Okay, two-third female and one-third male.

And on the web, similar? It's similar on the web. Okay.

Where do you or your loved one receive most of your sickle cell care? A, at a sickle cell treatment center with a hematologist; B, not at a sickle cell treatment center but with a hematologist; C, a primary care center, for example, a pediatrician or family medicine; D, only in emergency rooms and hospitals as needed; or, E, you're not sure. And we're asking you to choose just one in this case.

(Answering question.)

DR. EGGERS: Okay. At a sickle cell treatment center. So it looks like we have a mix of everything. Most people are treated by a hematologist in the room.

Similar? And it's similar on the web.

Okay. I think, is there -- yes. I think this is the final one.

In the past year, how often have you or your loved one had to go to the hospital or the emergency room because of sickle cell disease? A, no times in the past year; B, one to two times; C, 3 to 5 times; D, 5 to 10 times; or, E, more than 10 times. And this is just approximate numbers.

(Answering question.)

DR. EGGERS: Okay. So we've had it looks like again a mix of experiences with those who are fortunate enough to not have to go to the hospital very often, but we are also represented by those of you who do go to the hospital quite often.

I forgot to ask at this point, but can I ask the panel members to come up, the first -- the pediatric panel members? So that would be Nancy, Dawn, Andrea, and Alana, if you're here. Okay.

Are there any more polling questions? Okay.

MR. VALENTINE: Well, just to note a difference on the web.

DR. EGGERS: Yes.

MR. VALENTINE: You do see a similar spread, but then for more than 10 times, a quarter of the web

participants.

DR. EGGERS: Okay. I don't know if you all heard that, but a quarter of the participants on the web have been to the hospital more than 10 times. So those of you on the web who are in that position, your input is extremely valuable.

Pediatric (Infant and Young Children) Perspective on Topic 1: The Effects of Sickle Cell Disease That Matter Most to Patients

DR. EGGERS: Okay. So we are ready to begin our first topic. I'm going to have each of them introduce themselves as we go along. And they have come up. We have three caretakers and one person living with sickle cell disease, and they are going to be speaking on the pediatric perspective of the effects of sickle cell disease that matter most to you.

If I can have the next slide.

Nancy, Andrea, Dawn, and Alana. I don't know if you're in that right order, but that's okay.

And if we can go to the next slide.

What we've asked them to do is to talk about

-- to give us again that brief summary of the ways that sickle cell disease affects their health, the one to three things that matter most to them, how sickle cell disease affects life on an average day and how it affects life on the worst days and what worries you most about how sickle cell disease could affect your health in the future.

So with that, I am going to -- let's start with Nancy. Oh, push your little red button.

MS. RENE: Good morning.

DR. EGGERS: Good morning, Nancy.

MS. RENE: First of all, I would certainly like to thank the FDA for selecting sickle cell disease as the topic of this patient-focused initiative. I know a lot of us are very excited by this event.

I'm the grandmother of a 10-year-old boy, Joseph, who lives with sickle cell disease. I'm also the Chair of the Board of Directors of the Sickle Cell Disease Foundation of California.

The greatest impact of sickle cell disease. In December of 2003, Joseph was 9 months old. He had

just celebrated his first Christmas and was reaching those milestones every parent looks for. He was interacting with his family and pulling himself up to hang onto the furniture. All of that good progress stopped. He started crying and refused to eat or drink. His mom and dad realized that he was having his first sickle cell crisis.

His parents rushed him to the hospital, and although he was under a doctor's care, Joseph had a stroke when he was 9 months old. It left him partially paralyzed on his right side. He now walks with a severe limp and has difficulty grasping things with his right hand. He is on regular blood transfusions and had his spleen removed last year. He learns well, but his speech has been affected by the stroke, and he's hard to understand.

On an average day, Joseph has a hard time keeping up with his peers physically. He cannot run, climb, or play ball like the other kids do. Many times Joseph gets tired during family activities like going to the beach, walking, or hiking. Sometimes he can't even keep up with Grammy.

On the worst days, Joseph had several very bad pain episodes. A child who is generally happy and curious began to slow down and ache with pain. He would become so weak that he couldn't manage the stairs in his house, couldn't climb into bed. Although he was taking morphine, he was so weak and in so much pain that he simply couldn't move. As his blood counts dropped, it was decided to remove his spleen. For many of you, I know this is a familiar story.

I know Joseph would like to take part in team sports. It's hard to watch from the sidelines or be ignored by the other children. Whenever I pick up Joseph from school, the other kids are running around the track and Joseph is limping after them or sitting on a bench.

Currently, he is getting great treatment from his hematologist and nurse at Kaiser, who see him every 6 weeks for exchange transfusions. Their experience has made a real difference in his life. The family trusts them and knows that we're working together with the medical team. Not every family can

get this kind of expert care.

In infants, newborn screening has really made a big difference, and it's good that his parents knew the Joseph had sickle cell disease because they were able then to understand what that first crisis was all about.

Luckily, Joseph only has to go for his transfusions once every 6 weeks, so he doesn't miss a lot of school, and, of course, he missed when he had his spleen out, but school issues are a problem for other children when they have to miss more often and always get caught up in the makeup assignments.

But I want to say that since this is the FDA, thinking about these problems has given me new insights. If there were a drug that stopped a pain crisis in its tracks, children wouldn't miss so much time from school, young adults could spend more time in college and beginning those first jobs. A drug that could stop or reverse the damage a stroke can cause would really help children like Joseph. A safer drug for pain control would be key to effective treatment and keep many out of the ER. And, of

course, a drug that prevented red blood cells from sickling and causing organ damage would be seen as a cure.

According to data gathered by the CDC in California, 50 percent of people with sickle cell disease die before they are 50 years old. I don't want that to happen to Joseph. I hope that the interest by the FDA and the development of new and effective medications will help improve the lives of those with sickle cell disease.

Thank you.

(Applause.)

DR. EGGERS: Thank you, Nancy.

And we have Andrea next. Oh, push your red button. Yep.

MS. WILLIAMS: Good morning. I'm Andrea Williams, and I'm the Founder and Executive Director of Children's Sickle Cell Foundation, but for today's conversation, I'm Jonathan's mom. Thank you for the opportunity to share some insights from the parent perspective on the effects of sickle cell disease that matter most.

As the mother of a 13-year-old boy, I'm faced with the realities of this debilitating, often fatal disease. Jonathan is living well with sickle cell while we hope and wait for a cure. It is my hope that as I share today and we share in this event, that we learn from what we do here in the coming report to be enriched and empowered by the voices of patients and families.

My son had acute chest syndrome eight times from the time he was 2 years old until we started him on hydroxyurea at 6. Since starting hydroxyurea, he has only had one episode of acute chest syndrome, as recently as this past December.

He has experienced sickle cell pain crisis more when he was younger, from 10 months to 6 years. These unpredictable, severe painful episodes have interrupted his education. Missing days of school at a time, he missed critical building blocks that caused him to have difficulties of reading early on. We were able to provide him with a professional tutor who worked with him, and he began reading proficiently in the fourth grade.

Jonathan still has trouble concentrating at school and has a 504 support plan in place to assist him in succeeding academically. Other aspects of the 504 plan include having him seated near the teacher so he may be redirected easily if he gets distracted or off task. He has an elevator pass and door-to-door transportation to assist him with getting to and from school and to and from class.

We are proactively addressing the areas where sickle cell disease affects his daily life by managing the areas where he has the most difficulty and by preventing sickle cell pain crisis by avoiding the triggers that usually cause the pain.

His participation in outdoor activities is limited when it is cold. He finds this frustrating when the weather is borderline and he is told to stay indoors. This can be difficult for any teenager, but it's more so for Jonathan because his passion is basketball. He tells me that he wants to be the first NBA point guard with sickle cell disease. I tell him I want him to be the one that used to have sickle cell disease.

(Laughter.)

MS. WILLIAMS: He loves to play basketball in the backyard daily, and when the weather changes in Pittsburgh, we have to place time restrictions and sometimes complete restrictions on him for this. At times, he perceives this difference as a weakness because he can't do what he loves for as long as he would like, so we work on this perception, treating it as an adjustment that must be made so he can do what he loves and stay healthy doing it.

He is very in tune with his body and his limitations. He even knows the difference between sickle cell pain and other pain, so from muscle soreness from working out or injury. During pain crisis, whether at home or hospitalized, Jonathan describes this as an annoyance that once again has interrupted his life. Sickle cell has interrupted it with pain, fever, or an infection. The pain can be anywhere in his body, but usually in his back or his limbs. The severity ranges from mild dull aches to sharp and excruciating pain, but until it's well managed, it can be difficult to perform the tasks of

daily living.

What worries me most about how sickle cell disease could affect Jonathan's health in the future is that a cure won't come in time and that the more severe complications of having sickle cell disease will begin to affect him or that he may start to experience the side effects of hydroxyurea.

Of the many different complications that can happen during adulthood, I tend to worry about pulmonary hypertension the most because he has had so many episodes of acute chest syndrome. They are all very scary. When I looked at the list that she put up this morning, I cringed in my chair like, yeah, I would want to forget about those, but you can't forget, and that's why we're doing all we can to preserve his health now while he's still very young and very healthy.

This disease is not only physically devastating but emotionally draining for the child and the family. The unpredictable nature of sickle cell disease weighs heavily as we make plans for vacations, holidays, and more. We address every situation on a

case-by-case basis and live our lives knowing that we're doing the best that we can by getting him the best care, taking his medications, and teaching him to understand his body and limitations. A few months ago, Jonathan said to me, "Mom, I sometimes forget that I have sickle cell until you remind me to take my medicine." In that moment, I smiled because I knew that we were on the right track. However, I couldn't share with him that I never forget. I don't forget the pain of watching him writhe in pain, of watching him in his early hospitalizations for acute chest syndrome or others, the idea that sickle cell disease is affecting our lives even when we can't see it.

I remain hopeful that there will be better treatments -- (begins crying) -- more effective drugs, and a cure. When Jonathan was born, I was told that a cure could come in his lifetime, maybe even in the next 20 years, and we're 13 years in, and I'm looking forward to getting on to that, looking forward to contributing in any way we can to find a cure for this dreadful disease. Thank you.

(Applause.)

DR. EGGERS: Thank you, Andrea.

(Applause.)

DR. EGGERS: These are hard stories to share, and we appreciate it.

Dawn?

MS. NELSON: That's hard to follow. Let her go first. (Begins crying.)

DR. EGGERS: Yes. Alana, can you go first?

MS. McCLINTON: Okay, I'll try to follow that. I'm Alana, and I'm 23, and I am living with sickle cell. First I would like to thank everyone for coming. (Begins crying.)

DR. EGGERS: You know what? I'm going to suggest something -- while we let the panelists -- this is very emotional. We have a polling question, and I am going to ask to put up that polling question.

MS. McCLINTON: I wasn't expecting that.

DR. EGGERS: Because what we want to know in the panel, we've heard a lot about a lot of these symptoms, let's put this up and have you start to think about it. If you want to start to answer it, you don't have to answer it now, it's going to be up

here, you can put your choices in. What we want to know about is for those pediatric and young adults, other than acute pain crisis, because we imagine that would be one of your top most significant health effects, so putting that aside for the minute, what health effects of sickle cell disease currently have the greatest impact on your child's life, or if you're the adolescent answering, on your own, on your life? Please choose up to three effects. And while you think about that, I'm going to let Dawn start her comments.

MS. NELSON: One good thing about being here is that I'm a mother of a sickler, she's 16, but I realize being here that I'm not a crazy person, that there are a room full of people that feel exactly the way that I do, and so it's actually comforting to be here to know that I'm not alone in the way that I feel.

As we're sitting here, my daughter -- we live in Michigan, and she goes to a boarding school, she's in 10th grade, and she's actually having a pain crisis and my husband is taking her to the hospital as

we speak, and so this is very important for me to be here, and while I feel like I should go back home, I think this is -- she would want me to be here. All that I'm going to say are her answers, and I told her I would represent her well, so --

(Applause.)

MS. NELSON: She wanted to come, but she has already missed a week and a half of school since Christmas, and I told her that I can't explain her leaving school for anything other than health reasons, even to come to a meeting on sickle cell.

So when asked the question, "Of all the sickle cell diseases that affect your health, which one to three effects have the greatest impact?" I could give my answer, but I'm going to give you Maya's (ph) answers. She said nausea. Maya takes 1,500 milligrams of hydroxyurea every day, which is a lifesaving thing for us. She was hospitalized over 60 times, she has been in her life, and since she's been taking hydroxyurea since 2005, we have gone from being in the hospital seven or eight times a year to about one or two times a year.

So she would say that nausea is her biggest debilitating symptom as well as chronic leg and arm pain, and she has chronic fatigue, which is often confused with laziness, but I'm happy to hear today that that's one of the symptoms that's up there, so I can stop getting mad at her.

(Laughter.)

MS. NELSON: When asked, "What are the activities that she can't do?" she answers this, she does not participate in physical activities like other kids her age. She experiences instant fatigue that may lead to a pain crisis. Other activities that are limited are swimming unless the water is 84 degrees. I've had swimming pools change the temperature to 84 degrees just so she could learn to swim. Any kind of manual labor, raking leaves.

Because of the intermittent nature of sickle cell, her concentration during prolonged school work can be a problem. I'm actually going to do a research project soon on hearing loss and fatigue in sickle cell patients. Frequent breaks are often necessary. Playing in the snow is definitely out of the question,

and we live in Michigan. I really could go on, but I'm sure many of you could to.

I'll go to the next question. When I asked her, "How does sickle cell affect your life on the worst days, such as days when you have a pain crisis or are hospitalized?" guess what her answer was. It surprised me, and I checked up on her just last week to make sure this is still her answer. Maya feels that the anticipation of going to the hospital, the anticipation of the pain crisis, and the enormous amount of back work that she will have when she gets back to school. She missed, as I told you, a week and a half of school in January, and she just started to cry, it was so overwhelming, and I had her write down all of her work so that her algebra teacher could see it. She had missed about 5 tests, 10 quizzes, and I said to the school, we have a 504 plan, you know, "This is the law, you have to help me with this," but for her, she says, "I'm going to the hospital this weekend, Mom, and I'm going to take my work with me." I said, "No, you're not. You need to get better." They just need to deal with it, it's not a choice that

they have. But I said, "Are you sure it's not worse than the pain?" She said, "No, they have medication for the pain." That back work is what really gets her, and, of course, the social issues that we could all speak about.

Maya feels that when a pain crisis is brewing, she's very edgy, she's very nervous, she's very tearful, and it's been brewing for a while. There are also social consequences with the hospitalization. She is in boarding school, as I say. When she returns to school, her friends have moved on with life. Who you went to the cafeteria with last week, they've found another friend to go with this week. She's constantly trying to stay up with those friendships and stay in the loop.

Are there activities that she can't do? Her answer was this: she reported that she would like to be able to ski, to snowboard, hike, swim in a lake. She does not like to be an indoors person, she would like to be an outdoors person, however, they would evoke a pain crisis.

When Maya was a little girl, 4 years old,

she fell at a skating rink, a roller skating rink, and her arm turned black and blue, and all the other mothers said, "Why are you catering to her? Everybody falls at a skating rink," but I knew that bloodcurdling cry was a little bit different, and that turned into a 2-week hospitalization for Maya because a mother reported to me when she played with her kids at home, "Is something wrong with Maya's arm? Because she won't catch the ball, she won't use that arm." She had fallen at the skating rink. When she was in gymnastics as a child, she did a forward roll like all the other children during the second class, I had already paid for it, and her back started to hurt the next week. To this day, she will not skate or do other strenuous physical activity. So I would say that avoidance is her method of pain reduction.

In the future, when asked what worries her most about sickle cell disease, she says that she worries most about having a healthy baby, getting cancer, or dying early. That's very painful to hear your 16-year-old say that. So I just confirmed that with her, and her answer hadn't changed.

What specific concerns did I have about her? When she was a baby, some of them you have already stressed, not knowing when she was in pain or distressed, you would have to wait until she started to limp or something like that. I was also very afraid of fevers even though she took penicillin.

I'm a college professor, so anytime I know that she is going to get sick, I know that my entire schedule for the next 2 to 3 weeks is going to be messed up and I can't answer e-mails and that kind of thing. And so I would really like to talk to some other mommies around here.

(Laughter.)

MS. NELSON: I don't know how everyone else does it.

As an adolescent, what are we worried about? Maintaining social relationships is really a big one, as I said before. Getting the transition between reminding her to take her medication to now her transitioning to reminding herself to take her medication and training her to listen to her body and to know when back work is not as important as taking

care of that, taking a day off of school.

I now begin to worry about depression. She had a breakdown on Sunday. I had never seen her as dismayed in my life. Her eyes were glazed over with pain. Worrying about her future. Transitioning from a pediatric wing to an adult wing, so I'm going to be very anxious to hear from the adult patients here. It's a fear of mine, but I guess I have to face my fear, which is why I took a plane to come here.

In older adulthood, she worries about organ failure, premature death, people believing that her pain isn't real and providing adequate pain management. When we've been hospitalized outside of Michigan, getting people to give her enough of a drug has been a big problem, and I'm like, "Don't just give her enough medication, I need you to kill that pain," you know, but when I'm in Michigan with my doctors, they believe. I could call them from here and say, "Hey, Maya is going to show up today at 3:00, and they believe me."

So those are some of the issues that we face, and I look forward to hearing from everyone

else.

DR. EGGERS: Thank you very much.

(Applause.)

DR. EGGERS: We'll be getting into the topics on treatments in the afternoon, so let's everyone remember what's been said.

And, finally, we have Alana.

MS. McCLINTON: I will try to keep it unemotional.

DR. EGGERS: You're okay.

MS. McCLINTON: Listening to your story -- (begins crying).

MS. NELSON: You're okay, honey. You're okay. Just tell it. Yours is the most important story.

MS. McCLINTON: I so wasn't expecting to be this emotional.

MS. NELSON: It's okay.

DR. EGGERS: Let's give her a round of applause.

(Applause.)

MS. McCLINTON: This is why I don't wear

makeup, Ma.

(Laughter.)

MS. McCLINTON: Hearing your daughter's story, I feel like it's mine. (Crying.) At 23, I'm still going through the same things. My average day consists of now of a Level 7 of pain at least. There is this constant pain I can't get rid of. Tuesday night, after speaking to Sara on the phone, I went into a pain crisis, having to take 15 milligrams of morphine just so that I could sleep for at least 2 hours. I haven't slept through the night since I was in elementary school, and I'm 23 right now.

It's difficult to have to live with this pain everyday. And your average day is your worst day sometimes, most of the time.

I wish that I could -- thank you. I'm okay, thank you -- it's just this is the first time I don't feel alone. Seeing everyone and hearing everyone's stories, I wasn't expecting to be this way. I had this big speech planned, and it so did not turn out the way I thought.

(Laughter.)

DR. EGGERS: Alana, could I say one thing that you told me in your thing? So I think -- correct me -- you have completed your bachelor's degree.

MS. McCLINTON: Mm-hmm.

UNIDENTIFIED FEMALE SPEAKER: Amen.

(Applause.)

DR. EGGERS: And you have completed your master's degree.

UNIDENTIFIED FEMALE SPEAKER: That's wonderful.

DR. EGGERS: And in education of some sort?

MS. McCLINTON: Adolescent education.

DR. EGGERS: Adolescent education.

MS. McCLINTON: Before my 23rd birthday.

UNIDENTIFIED FEMALE SPEAKER: Wonderful.

DR. EGGERS: And what Alana told me what she wants to hear, she can't wait to hear the adults speak today. What she told me is that she doesn't know how people -- she is scared coming out of school. She has finished school, she has her degrees, how is she ever going to be able to actually live up to those degrees and have a job and be able to function in her day? So

I'm going to say that one of your summary for you because --

MS. McCLINTON: Thank you.

DR. EGGERS: -- I think that's a very important part and I personally want to commend you for finishing school.

UNIDENTIFIED FEMALE SPEAKER: Amen.

MS. McCLINTON: Thank you.

(Applause.)

DR. EGGERS: Is there anything else, Alana, that you want to say, or would you like us to go to the discussion?

MS. McCLINTON: I just want to say thank you for coming. You guys look so beautiful. (Crying.)

DR. EGGERS: Let's give Alana a thank-you.

(Applause.)

DR. EGGERS: And I'm going to ask you guys to stay up here. You're still part of the discussion.

Is that a microphone over there? Is there a -- do we have a floating mike for -- okay.

MS. McCLINTON: Oh, there is a microphone in front of me.

DR. EGGERS: Okay. So now we're going to have the facilitated discussion. I'm going to come around to the front if I can find that there is a microphone for me. We're not a professional talk show up here, so bear with us as we go through.

I'm going to let my colleagues ask some questions as you need, and you feel free to join. I have to actually stand this way. Sorry. I need to be able to see this alarm clock over here, it's the only thing that keeps me on any kind of track. Okay.

Can I have a raise of hands of who is a caretaker or a young person in the audience today? Raise your hands. Be proud. Okay.

(Show of hands.)

DR. EGGERS: All right. Great. We're going to be focusing on you for this morning's discussion. And I really, I truly want to thank all the panelists. It does, it takes a lot of courage to be up here, and so a very sincere thank-you.

What we wanted to do is now listen more, let you build on Alana and Dawn and Andrea and Nancy's stories. And we wanted to put up a polling question

that would help us get a sense of what you think are the most significant impacts, effects, of sickle cell disease on you or your loved one. So we put up this question, and we wanted to know, was chronic pain, that's A, if you haven't selected yet; B, multiple infections; C, stroke; D, acute chest syndrome; E, growth problems or a delay reaching puberty; F, priapism or painful erections; G, problems with the spleen; H, difficulty concentrating; or, I, something that's not listed here. There are a number of other symptoms. We couldn't capture them all. So if you could take a moment and pick up to three. And raise your hand if you need a clicker. And on the web, I hope that you're answering as well. Okay.

(Answering question.)

DR. EGGERS: Is everyone ready? Okay. Can we go to the results? Okay. Lots of "Others." Okay, we'll get to those.

So I know this is extremely hard to see, and my bifocals aren't doing me justice right now, but it looks like the number one thing, at 46 percent of you in here, is difficulty concentrating. We're going to

delve into that a little bit. Chronic daily pain, I think we've heard a little bit about that. And then, D, acute chest syndrome.

Okay. So let's go into those topics a little bit more. Let's start with the difficulty concentrating. We heard that. I think, Dawn and Andrea, you talked about it. And, Nancy, you talked about it I think some. Does anyone else want to share some specific way how you would describe what effect difficulty concentrating has on your child or you in school or in some other reason? Raise your hand and then we'll come to you. Okay, over here. And if you could just state your name.

MS. BROWN-WATTS: Good morning, everyone. My name is Velvet, and I am from Oklahoma. I am a social worker, and I have a 9-year-old son who was diagnosed with sickle cell in 2004, and since his birth, Jeremiah has had a significant difficulty in retaining what he learns in school. He has IEP and he had significant problems with reading and language development. He's had three sets of tubes put in his ears, and even after having the tubes placed in and

his hearing restored, he still struggles in speech. He's been in speech since he was 2 years of age, and he's in the third grade now.

And what I am hoping is that there can be some drugs that maybe deal with the difficulties in the cognitive abilities of the patients. I wake up every day wondering what's going to happen to him if he cannot maintain what he's learning in school. How will he be able to sustain a job or graduate from school or even when you look at college? He takes tutoring. We have him in everything that we can think of, but most of the days after school, the only thing he wants to do is take a nap.

We recently learned 2 years ago that Jeremiah's lung function was at 72 percent, so he had major problems with his lungs. When he was born, he had four pneumonias, two ileuses, and we kept kind of wondering what was going to happen. His lung function had never dropped. When we found out that his lung function had dropped, they took him out of gym, and the only thing he wanted to do was be normal. He says, "Mommy, I hate this disease." And he says, "I

don't want to die." And I said, "I understand that and we're working to do everything we can to keep you healthy." He's on 5 liters of oxygen at night, and consistently we are working to maintain him getting educated.

And so there needs to be drugs to deal with the lung issues and concentration, and there needs to be a concentrated focus on education for individuals with sickle cell disease.

DR. EGGERS: Thank you.

(Applause.)

DR. EGGERS: Jeremiah's mom, I didn't get your first name?

MS. BROWN-WATTS: Velvet.

DR. EGGERS: Velvet. Thank you.

How many of you with the pediatric perspective or even as adults, that that was a problem for you, the concentration and difficulty with school? Do you want to raise your hands if you feel comfortable?

(Show of hands.)

DR. EGGERS: Okay, significant. Okay.

Then I want to touch upon something else that you said, Velvet, which is you said -- let's go to fatigue. Fatigue wasn't on the chart, it should have been, it didn't make it up here, but you mentioned that when your son gets home from school, the only thing he wants to do is go to sleep. I think we heard some of that up at the front.

Can I just get a show of hands, about how many of you even when you were a young child, had that problem with some sort of fatigue?

(Show of hands.)

DR. EGGERS: Okay. Does someone want to describe the fatigue a little bit? Does it happen at a certain point of the day or did you notice some sort of triggers? Does someone want to share that? We'll go the -- let's see. Okay.

Yes. Go ahead. There is a microphone coming. When you were a child.

MS. STINSON: So for me it was literally my body felt like it was weighed down. Actually -- I'm sorry, I'll stand up. My name is Jocelyn.

DR. EGGERS: And what's your -- yes.

MS. STINSON: I am 28 years old and I have sickle beta-S thalassemia -- sickle beta-plus thalassemia. It literally felt like I couldn't get up, like my -- it would just -- just my arms and limbs would just drop. I could not roll out of bed, it would just -- I couldn't move, it was that kind of fatigue, and I would sleep all day. My mom would try and wake me up, and she would wake me up to get food and water, and I would go right back to sleep, and I would sleep for hours straight. So it was really tough. I don't know if anyone else --

DR. EGGERS: And what was your name again?

MS. STINSON: Jocelyn.

DR. EGGERS: Jocelyn. Thank you, Jocelyn. And was this every day, Jocelyn?

MS. STINSON: No, it wasn't every day. I'm not really sure. It didn't have like a consistent pattern. It didn't happen around -- it was definitely when I considered myself 100 percent, meaning I hadn't been in the hospital for a couple of months or I hadn't had any pain crisis, but it was just considered those day-to-day issues that you have with sickle

cell, I would classify it under that.

DR. EGGERS: Okay. Does anyone else feel what Jocelyn described, do they also experience it? Just with a show of hands, how many?

(Show of hands.)

DR. EGGERS: Okay. Yeah.

Yes?

MS. HADNOTT: That percentage is so large, and I'm wondering if fatigue is there.

DR. EGGERS: Okay. Margaret raises a good question. And, Margaret, do you want to do co-facilitation with me? You can.

(Laughter.)

DR. EGGERS: How many people had put fatigue in their "Other" as the young people?

(Show of hands.)

DR. EGGERS: And we'll have the same type of question for the adults. Mm-hmm.

Anything that my colleagues want to ask about fatigue?

Jonca.

DR. BULL: I was just wondering if fatigue

had any relationship as a warning sign for crisis, or was part of the outcome after a crisis? Did it have any relationship, any predictive value, in terms of your clinical course? I see a lot of heads nodding no.

DR. EGGERS: Yeah. Okay.

And I think, Alana, you had something to say about fatigue?

MS. McCLINTON: I was just going to touch on Velvet's concentration point. An experience I had all during college is the difference between being physically present in a class and mentally present. And, you know, in college you have a certain amount of absences you can have before they just fail you. But I was very up front about what I had, but a lot of times I had to just be physically present and not that mentally present where I'm actually there concentrating and focusing on, you know, whatever is at hand.

And then the fatigue point is a lot of times, you know, I did mention when I spoke of not being able to sleep at night. A lot of times when I'm

in a crisis, especially an acute crisis and I'm in the hospital, I just want to sleep, it's that moment of peace you get when you're sleeping that you're not in pain. So I've never -- I mean, the people that I've spoken to with sickle cell, I've never heard of fatigue as a kind of a, I would say, coming attracting of a sickle cell crisis.

DR. EGGERS: So we're going to actually have to move on to -- oh.

DR. FARRELL: One question. I know this is the pediatric and young adult session, but when we get into the adult session, we have concerns that some of these what we call patient-reported outcomes may change over time, and so there may be the fatigue that you experience as a child or adolescent, and does it persist into adulthood? Because when we think about these measures of the effects on your life, we need to come up with good measures, and so if they're different from the pediatric and the adult perspective, we would like to hear about that.

DR. EGGERS: So I'll put a shout-out. We're going to have to move on from fatigue and

concentration, but, please, it sounds like this is a very important and maybe not discussed, very much discussed, topic. So in the docket, when you submit your comment through our website, please address your issues with fatigue.

We have so much we want to cover, so I'm just trying to see where we should go. I think we'll save talking about chronic pain for the comments. Send us a comment through the docket. And things like strokes and acute chest syndrome, we heard the panelists, so if you have a story that's different from that.

I want to hear if there are any symptoms that are not on this list that maybe were in your "Other" category that you would say are in my top two, my top two symptoms, the effects that affects my child or the young person. Anyone want to comment on something?

Yes. We have --

MS. McNEIL: Hello. My name is Sameka. I have a 6-year-old girl, Jasmine, and one of the biggest things that I have, which is acute chest, but

coming with acute chest is asthma. And Jasmine can get a cold that triggers acute chest that then goes into an asthma attack that just -- you know, so for me, understanding, as a parent, how the lungs are really affected, and she's had a total of four acute chest syndromes and has been hospitalized for each one of them, but since she has been on the asthma medication and has now been placed on the Singulair and we have a very aggressive treatment plan, if I hear a slight cough at all, I start the aggressive plan. It's because if I don't, the acute chest kind of rolls right into it. So the biggest thing is understanding the relationship between sickle cell and also having that asthma, which then goes into the acute chest.

DR. EGGERS: I see a lot of heads nodding. A lot of agreement with this statement?

Any other symptoms? Any other effects?

DR. FAULCON: Sara, perhaps we can hear from those that are on the webcast?

DR. EGGERS: Oh, yes. For -- mm-hmm. Can we have the webcast results of that polling?

MR. VALENTINE: Okay. So moving through the list, very similar. We had about -- except much higher percentages, similar trends. We had about 83 percent of the participants say that they had issues with chronic daily pain, about 25 percent with multiple infections, 12 percent with strokes, 29 percent with acute chest syndrome, 12 percent with growth problems, 7 percent with priapism, 7 percent with problems with spleen. We did break out fatigue on the web, and we had about 80 percent confirm that they have fatigue, 44 percent with difficulty concentrating, and then about 32 percent with other effects not listed above.

DR. EGGERS: Okay. Thank you. That's very informative.

We're going to tee up the phones. We're going to go to the phone lines in a few minutes, and that will give a chance for a few web participants to call in. Now, if you're on the web and you want to go to the phone, there are going to be some instructions here. And what we're looking for is if you have a symptom that hasn't been mentioned that you want to

talk about, this is our second time doing the phones, so if it doesn't go exactly smoothly, then my apologies in advance, but we're going to try. So that will be in a few minutes.

Are there any other symptoms that people want to talk about? Restrictive airway I hear. Okay, so that kind of goes with the breathing problems.

Okay, we have someone in the back? And while Andrea goes to the back, I'm going to come over here. Raise your hand in the back if you wanted to -- okay.

MS. BAILEY: Hello. My name is Tara. And as a child in primary school, I had a lot of issues with stress causing sickle cell crises, so for me, during pretty much every year final exam, I missed every final exam for like 3 years in a row, just stressing about studying about it.

DR. EGGERS: I see a lot of heads nodding. Anyone had a similar experience? Do you want to show your hand?

(Show of hands.)

DR. EGGERS: Okay. It looks quite common.

Okay. Sorry. Yeah. Let me go over here. We have Francesca?

MS. VALENTINE: Hi. Good morning. My name is Francesca Valentine. I live on both side of the fence. I'm a registered nurse for 35 years. My son is 30. He'll be on the adult panel with sickle cell disease hemoglobin SS. Silent symptoms that can be ignored during school plagued us. I didn't know he had his first silent infarction because they switched school nurses and the new nurse hadn't looked at anything about sickle cell, so he was deemed being unruly when he was in the nurse's office with black spots in his visual field rearranging things on the desk, sent back to class. Then he was deemed as being not paying attention and lazy when he was tired and he could no longer spell in fourth grade what he learned to spell in second grade. And he couldn't tell the time on a watch face, a regular clock, and he could before. So in the midst of labels, they missed the symptoms.

DR. EGGERS: Thank you, Francesca.

Did someone want to say something up here?

I thought I saw a hand.

Go ahead, Nancy.

MS. RENE: Yeah, as I work with the Sickle Cell Disease Foundation of California, I'm a former school principal, so I get a lot of these educational issues, and I tell you they are simply awful, just awful, from school people not believing that the child has sickle cell disease and not believing that the child is in pain, only believing the worst about a child, and even when we would go in to have the conferences with documentation, those beliefs did not change. And so that's the added stress on the child and on the parent. One of our parents was sent to the district attorney because they were accusing her of truancy for her child, and the child is in the hospital with a sickle cell crisis.

DR. EGGERS: Thank you, Nancy.

I heard a lot of murmurs, a lot of hands raising.

There is one comment back here, I think?

DR. PESANTE: Hi. My name is Maisha Pesante. Our "Other" is very different because my

children have only AS, and we were told that it doesn't have any symptoms. I have three children with AS and two of them get very many of the same problems I'm hearing today.

My first child had got those symptoms when she would come home from school and go to sleep for hours, I thought she was just being unruly and lazy, me, as a parent, I thought that, because that was what I was taught, that sickle cell didn't cause a problem, sickle cell trait AS didn't cause problems. It took years, and until she had what was pretty much a sickle cell crisis at the age of 16 that I understood that you can have that happen with trait.

And it's been very, very painful when my son, who just turned 12 yesterday, had his first crisis at the age of 10 and was sick for an entire month, and we couldn't get anybody to believe us or even just give him fluids. And they gave him like a little bit of morphine, like 1 milligram, and he said, "Mommy, the morphine doesn't seem to help as much as the fluids," and I could get the morphine for him easier than I could get the fluids. It was an

extremely painful experience.

He had 12 hours of priapism. They both have terrible fatigue. They get bone pain. They have trouble breathing. In school, when he was passing out from the crisis, they said he was faking it. EMS was called to the school. The EMS person told me that, "Oh, I don't think anything is really wrong with him," but his blood pressure was like 70 over palp. And then when we got to the hospital, he couldn't walk, and the doctors and nurses there told him, "Oh, you can walk. There is nothing wrong with you."

So I am very sad listening to all of the stories. I'm thankful that they aren't as severe, but it's also scary because we can't get treatment because we're told that AS doesn't have any problems. He missed a lot of school that April. And by the way, he's a straight-A student, top of his class, skipped a grade. And my older daughter, who is here with me, she was doing great in school and then all of a sudden stopped doing well in school, and I missed it because I was told AS doesn't have symptoms.

So the fatigue and the priapism were the two

symptoms that were scary to me because my son didn't even tell me, and then the pediatric hematologist said, "Well, this isn't sickle cell. I don't know what it is, but it isn't sickle cell."

DR. EGGERS: Thank you very much. Thank you for sharing your story.

(Applause.)

DR. EGGERS: We're going to have one more comment and then we're going to go -- oh, two more comments and then we're going to go to the web.

MS. HUGHES: Hi. My name is Tina Kay, and I'm from Birmingham, Alabama. One of the things in "Other," I have sickle cell disease, and I'm 40, but I mentor young ladies who are transitioning into college, and bullying is a serious issue. I've had one young lady who actually was put off of the basketball team by the coach, they wouldn't let her play volleyball, and the young ladies that played the sports with her were threatening to beat her up. So bullying is another issue, and that's why children with sickle cell disease want to hide it, because they don't want other people to know that they're

different.

And another thing is loss of hearing. A lot of people don't talk about the loss of hearing. It's not just vision, but the small veins that go to our eyes also go to our ears as well.

DR. EGGERS: I think we'll hear about loss of vision in the next topic. Thank you for your point, Tina.

We're going to have to keep going if we want to make sure we get to the adults, to hear from the adults in the room.

Any quick follow-up questions from my colleagues?

DR. VERDUN: I was wondering if anyone here has experienced a stroke or silent infarcts as a child and want to speak about that experience.

DR. EGGERS: First before we do that, we've got someone in the back who has been waiting a long time to talk, so we'll let that person. Yeah.

DR. IVY: My name is Donnell. I wear multiple hats. I'm the Program Director for the Hemoglobinopathies Program at HRSA, I'm an MD, and I

also have sickle cell disease. I was just going to say in the "Other," one thing you might want to talk about, because it does go to concentration and fatigue, is sleep disturbance. I know that even as an adult, I haven't had a crisis in years, but I still find it hard to sleep sometimes at night, so that could be a part of that "Other" that needs to be considered in terms of difficulty in concentration and fatigue.

DR. EGGERS: Thank you very much.

Okay, now, going back to Nicole's question about stroke. Okay, right here, go ahead.

MS. BAILEY: You asked about stroke as a child and silent infarcts, and I did experience that when I was younger. My first -- I was living in Indiana where I was the only patient with sickle cell disease in the entire city I lived in, so there wasn't a great deal of education, and also I did have a silent infarct. We've learned that the teachers, as you all have mentioned, are the best ones in a position to notice these changes, but for me it was just considered unruly, it was considered you're just

not trying hard enough going from being a straight-A student to bringing home C's, and my mom didn't know, so no one understood, and I did have a stroke. I fell, I bumped my head, and they were saying that I was faking, and all of it was -- wasn't. And fortunately, I had a physician that was confident enough in herself and her ability to acknowledge that she didn't know anything about sickle cell and to contact those that did. So even before the study came out that blood transfusion helps with -- the chronic transfusion will stop a lot of that stroke, they put me on chronic transfusion to slow down some of those infarcts I was experiencing.

DR. EGGERS: Thank you, Lakiea.

So I think we get one more and then I really want to get to the folks on the phones. This is our last comment in person for the pediatric.

Go ahead.

MR. CUMMINGS: I'm speaking on behalf of my son, who is 25, when he was young had a series of silent infarcts culminating with a pretty major stroke when he was 9. One particular time it was

precipitated after traveling on a plane, and he arrived and was having slurred speech and weakness on one side. We had a bad experience. We were going to an ED. We weren't believed that he was suffering from anything, and after 45 minutes when hydration had not started, we stormed out and went to another hospital where he had an exchange transfusion for 6 straight hours. He ultimately to this day still has difficulty with walking without assistance and takes a number of meds to control seizure activity. And he was eventually treated for the moyamoya condition with a type of surgery to correct the occluded carotid arteries with replacing I guess temporal veins so that they would reperfuse his brain from the top down.

DR. EGGERS: Thank you.

Are you John? What was your name?

MR. CUMMINGS: My name is Bill Cummings.

DR. EGGERS: Bill, Bill. Okay.

So as we go -- I'm so sorry we can't get to all of the pediatric. We will be able to touch upon the treatments for pediatric patients in the afternoon. But I hope this gives you motivation to

write to us and submit a comment telling us your full story.

As the people -- we're going to have a web summary first of some of the comments, and I'm going to ask for the Panel 1, the pediatric panelists, to step down and for the adult panelists to come up.

Again, pediatric panelists, thank you so much.

(Applause.)

DR. EGGERS: And, James, can we have just a sampling of, or Pujita, a sampling of the symptoms mentioned?

MS. VAIDYA: Hello. So we've gotten a lot of comments on the web, and I'll just summarize and mention a few of them. Some participants have mentioned jaundice, fatigue related to menstrual cycle, so she has noted that it's gotten worse during her menstrual cycles, which we have not heard in the room. And some problems with eyesight and avascular necrosis.

DR. EGGERS: Thank you very much.

MR. VALENTINE: And I'll just note one other

thing, that many comments have talked about the stress and depression resulting from pain and symptoms.

DR. EGGERS: Thank you. Okay.

We just have time for one or two phone comments. I'll just ask on the phone just in the interest of time just to keep your comments brief and focused on a symptom, a health effect, that you haven't heard mentioned today or one that very much bothers you.

Do we have the phone? Do we have our next caller?

MS. GAINES: Hello. Can you hear me?

DR. EGGERS: Yes.

MS. GAINES: Hi. My name is Marquita Gaines. I'm tuning in from Georgia right now. And I'm 21 years old, but I did want to just let the parents of the adolescents know that it is important to address the physical symptoms, and I actually talked to my doctor about how you can change the hospital environment, but because the turnover with ER doctors and students in training is so quick that the sensitivity and the care that you receive in the ER is

I guess a little more cold and not as caring and comforting as it could be.

When I was younger, I did have the problem with being in advanced classes but missing so much school that, like Alana said, the seat time hours that you were required, you could have an A, but they would fail you because you weren't there. And I think it's important to talk to the children about how they're not alone and it's okay to feel confused because you know you're capable, but your body won't let you. And even now that I'm in college, I deal with that. And it's just important that we support each other and listen to the younger kids because as far as sports and socially, I would just want them to know that it does get better.

DR. EGGERS: Marquita, thank you. You might not be able to see this, but I see a few moms nodding their heads in agreement here on your excellent point.

MS. GAINES: Thank you.

DR. EGGERS: You're welcome.

One more phone comment? Pardon me? Oh, Operator, can we have the next caller?

They have to train me for this.

(Laughter.)

OPERATOR: Sorry. It's Amorilla?

AMORILLA: Yes. Can you hear me?

DR. EGGERS: Yes, we can. Amorilla?

AMORILLA: Hi. How are you? I'm Amorilla, and I'm calling from West Hartford, Connecticut. My daughter has -- my daughter, Annemarie (ph), is 4 years old and she lives with SS. As I've been waiting here listening to everything, so many people have brought up the points that I wanted to kind of just touch base on, which were asthma and its relation to acute chest syndrome, which my daughter has been diagnosed with asthma, as well as the sleeping patterns, which affect her as well, and because of them, she has had her -- because of her irregular sleeping patterns, she has had her adenoids and her tonsils removed, and it has helped, but it has not helped enough because it's still affecting her.

And also some people have brought up the loss of hearing, which I'm a little bit amazed to start hearing about. I didn't know that could happen

at such a young age, and I feel like recently my daughter has kind of been complaining about not being able to hear.

But, you know, I think that it's totally amazing that this public meeting is taking place because so many of us have such diverse stories, and to kind of hear them all in one, I myself, listening to like the panelists and listening to everyone's personal stories, have been taken aback with so much emotion, and I'm sitting here crying because I'm just like, "Oh, my god, I'm not alone."

So I do look forward to hearing more about the relationship between asthma, breathing, sleeping patterns, and as well, my daughter has a lot of allergies, so that kind of makes it difficult as well. So I'm just looking forward to hear more about the relation of them all.

DR. EGGERS: Great. Thank you so much for your comment.

AMORILLA: Thank you for allowing us to be able to call in. This is amazing. And I wish that I could be there physically, but I'm glad that I was

able to call in.

DR. EGGERS: Okay. We'll call this experiment a success.

(Laughter.)

AMORILLA: Absolutely. Thank you.

Adolescent and Young Adult Perspective on Topic 1

DR. EGGERS: Then with that, we're going to move into the next topic and focus on adults. I think there has been some overlap, so what we're going to do is focus on things, how your condition has changed since your adolescent and young years. We have four excellent panel members to share their stories. I think I'm going to step back at the podium. I'll relinquish my microphone.

I'm going to come back up here and let the panelists speak for themselves. Again, keep it to a couple minutes. We are going to be running short on time, so 2 to 3 minutes, and I will nudge you along if I have to. And I can't wait to get started.

Who wants to start first? Marcus or George? Let's have Marcus start. Let's let the young ones start. Just hit the button.

MR. VALENTINE: Hello. Hi. My name is Marcus Valentine. I am 30 years old, and I have been living with sickle cell hemoglobin SS for all these years. This is my mom next to me. I'm from Illinois, and we came out here for the panel discussion on the topics that are concerning the sickle cell.

And for the first half of my life, I was just kind of wanted to be normal, and I heard the stories from the mothers of the pediatric people who were just up. They were similar, I was similar to that. And so if your children, which they sound like they have the strength I do, will get to where they should be, and that's doing something for the illness to further advance our survival and well-being.

But I am very happy to talk about the effects that sickle cell has on me and my daily life, which I have heart failure from it and the fatigue. And there is something that just developed with me, which is wounds, sort of like diabetic ulcer wounds, that I just developed on my ankles and --

UNIDENTIFIED FEMALE SPEAKER: Similar to that.

MR. VALENTINE: -- at first it was new because all the treatments that I get when I go into a crisis usually help, and I have a set plan, and it helps out, but the thing that I'm going through now, it's kind of like a different -- the treatments that usually work, are successful, are helping, but only to a certain point. And so I was happy to hear that the FDA is having this conference about sickle cell and possibly developing new drugs so that we have something there, something extra, and something that's kind of our own that can help us get through life, our daily life, without these long pauses because when you get sick, it's like you have to just stop everything you're doing just to recover. So I hope that some of what I say today can help further getting us to that next step.

My mom is going to talk for me because I'm not feeling too well today, so --

MS. VALENTINE: I'm going to help him if that's okay.

DR. EGGERS: That's fine.

MS. VALENTINE: He developed wounds. We had

a flood, he got a scratch. It developed into a chronic non-healing ulcer, swelling, sickling to the surface of the skin and his tiny vascular, microvascular, system. So what you would do for, say, a diabetic or someone with other types of wounds, those don't all work for these sickle wounds. We learned early on you can't put him in compression, that's going to make it worse and cause more constriction, and the standard wound care, we have excellent wound care, but it's not enough. Everything has to go together, and there is no quick fix to this. The right one healed, it reopened. The left one developed cellulitis and had to be surgically debrided.

So wounds are a very debilitating complication on the lower limbs because you still need to walk to get where you have to go, and they're painful and require a lot of care. And I think he said to me right after he got out of the hospital, he says, "You know, I would almost rather have them take these feet and be done from above the wound and call it a day because," he said, "I'm so tired of this, of

all my complications," which have included acute chest ventilation exchange, osteonecrosis, the list is very long, I won't even try to give it to you. But of all of this, he thinks the wounds hands down have really caused the biggest problem for him.

MR. VALENTINE: And then I started a film about sickle cell, and I just wanted to put that out to let people know that you're not alone and you should never be afraid to talk about this illness because that's how we advance and progress, we have to talk about it, because for such a long time I hid all of that. I didn't want to talk about it, I didn't want to deal with it, but the moment I started, it's what made me feel better and I was able to see the change in other sicklers. So, you know.

MS. VALENTINE: And one more thing, he -- and I applaud him, his wound is on Facebook, and with the progression and what we're doing, and anything we find to help make it better, it will be on Facebook with a website for evidence-based proven from the physicians. Our physician is at Edward Hospital in Naperville, and our colleagues from the University of

Illinois in Chicago, the collaboration is phenomenal. I'm a registered nurse, so I'm hoping through teaching future nurses, we need to bust a hole in the stereotype wall because while they're worrying about stereotypes and faking, we're dying.

(Applause.)

DR. EGGERS: Thank you. Thank you, Fran, and thank you, Marcus.

We've got a great young advocate on your hands here.

I think we're going to move on and have Helen. And Helen has -- oh, no, I'm sorry, we'll have Anthony.

Anthony, nice to meet you.

MR. BRAXTON: I do apologize.

DR. EGGERS: No, push your little red button.

MR. BRAXTON: I'm sorry. I apologize. I'm actually speaking on Topic 2, I kind of jumped the gun a little bit. So --

DR. EGGERS: You can come in the after -- can you come back in the afternoon?

MR. BRAXTON: Yeah, yeah. I'll be here all day, but --

DR. EGGERS: Okay.

MR. BRAXTON: So I'll pass it up for now and talk to you later.

DR. EGGERS: All right. Then we'll have Helen.

MS. SARPONG: Hello. I'm Helen. I'm 38 years old. I have sickle cell SS, I'm sorry, sickle cell disease SS. I'm a mother to two twin girls, ages 5. So I had this speech here that I've been working on, but after being here and hearing all the stories, I feel like there is so much that I can add and not go over time.

So I'm just going to say that I've had my spleen removed, my gallbladder removed. I have avascular necrosis in both of my hips. And I am now starting to feel symptoms in my two shoulders. I've had problems with my eyes. I have a herniated disc. And I am also being followed for pulmonary hypertension.

The symptoms that matter the most to me is

the things that affect adults -- sickling cells, bone damage, obviously death -- but I'll tell you a little bit more about the road to becoming a mother. I never thought I would be a mother, it never crossed my mind. They told me that I would not live to be an adult, but at 32, I decided to give it a try. I stopped taking hydroxyurea. I had seven major crises. I also had sickling of the placenta -- sorry -- intense bone pain, and the swelling made it difficult for me to make it to my doctors appointments as well as birthing.

My doctors came to me and said that I needed to have a selective abortion. Obviously I was devastated because the idea that my body was malfunctioning and causing my babies to stress was too much for me to bear. My doctors decided that I should have an exchange transfusion, and I was able to carry my babies to 36 weeks and 4 days. The day that my daughters were born I felt that I had conquered sickle cell disease.

(Applause.)

MS. SARPONG: Five years later, I am still

reminded how happy I was that day. Present day, as their mother, it's very difficult for me. I have intense bone pain, not crisis pain, intense bone pain, that is due to the complication of sickle cell disease. Most of my days are spent lying in bed unable to get out of bed. My husband has to get me out of bed while my daughters help me put on my shoes. I am often late taking them to school because I'm just having a lot of pain, again not crisis pain, but pain due to complications.

Herniated disc came at a time when my career as a pastry chef was advancing. I was able to go to my dream school and graduate with a lot of difficulties, but I made it. So, of course, when I was told that I could not be a chef anymore, I said to my doctor, "What do you want me to do now because I nearly died getting here?" and he told me to get a new career. I tried the new career and it did not work for me. I went from not having a crisis in 3 years to constantly having a crisis. I am unable to work because of complications, I cannot sit or stand for a short period of time without experiencing intense bone

pain. It's hard because I'm a very outgoing person, I'm happy, but I suffer on a daily basis, again not because of crisis pain but because of complications.

I have a hard time concentrating. I should also say that I have a learning disability. I was diagnosed I think in junior high school, but I was diagnosed for sickle cell disease so that I could have accommodations, so that I could be able to take the cheese bus. So I never probably learned how to read until I taught myself at the age of 20. I always knew that I was smart, but I couldn't understand why I could not retain information or organize my thoughts or get my brain and my fingers and my eyes and my mouth to all kind of work together so that I could form a sentence.

When I decided I wanted to go to culinary school, I decided to seek help, and I got tested. I remember the psychologist who tested me said, "You know, your test scores does not reflect who you are because the numbers, they don't match. I have just met you, but I'm not understanding what's going on, and I don't know that much about sickle cell disease,

but I think that years of you having these crises and these little tiny blood vessels, that is affecting every organ in your body including your brain," because at the time I did not know the difference, "That could be the reason why you're having so much problem retaining information, paying attention in school, and just remembering conversations that you just had."

So that made a big difference for me, and that taught me that I had to, A, accept my learning disability, because it was a big deal for me, and, two, learn everything that I could about sickle cell disease and not hide. Once I did that, I learned how to study, I learned what my learning style was, and I learned to bring others into my life with sickle cell disease. In college, my father dropped me off and he gave me $100 and he said, "This is all the money I'm going to give to you, and you have to support yourself."

Within minutes after my dad left me, I had the worst crisis that I can remember as an adult, it was an acute chest syndrome. Mind you, I had just

been dropped off, so I didn't have time to make connections with roommates and friends, and here I was screaming on the floor begging for someone to help me. That was my first recurrence of acute chest as an adult. I really felt like I was going to die. I had never experienced anything like that before. So I had to, I guess, come out of my shell, not be scared, and invite people into my life with this disease, and once I did that, I got a lot of responses that I never thought I would get: the compassion, the willingness to learn, the support.

In college, I missed -- I think I X'ed out seven times, and that basically means that I would leave and stay away for 4 weeks because I missed 3 days of school because the program was so vigorous and intense, there was no way how you could miss days, but I kept on coming back. And every time I was paying for that.

I managed to graduate culinary school, I got my degree, held a job for 3 long years, the longest time I've ever held a job, and then was diagnosed with AVN. So when I was diagnosed with AVN and didn't know

what I was going to do with myself and couldn't get out of bed, could not work, I decided to start to have a family, and then I had my twins.

So as their mother, it's difficult. Like I said, they have to help me put on my shoes. You know, there are days when I hurt so bad they say to me, "Mommy, are you hurting today?" and I will say, "Yes." Okay, I'm so not going to cry. And I will say, "Yes." And they will say to me, "You know, I'm so sorry for you because you are the best mommy in the world, and tomorrow will be better." And tomorrow --

(Applause.)

MS. SARPONG: -- and tomorrow it does get better, but it gets better because I get out of bed, as painful as it is, I get out of bed. I have no choice, I have to function, I have to take care of them. On a daily basis, I cannot take pain meds because I need to be present and be able to raise my kids. My husband works to support us as well as support this endeavor that I have to tell my story and to help others. So I can't be on pain meds and not be able to function. So I've had to find other ways to

deal with the pain.

DR. EGGERS: Helen, we'll be talking about the treatments in the afternoon, so can I -- so make sure you hold those thoughts and share them then.

MS. SARPONG: Sure.

DR. EGGERS: Do you have any other final thoughts you want to say?

MS. SARPONG: Okay, so I talked about bone pain, avascular necrosis and how it affects me. Right? Okay.

DR. EGGERS: Mm-hmm.

MS. SARPONG: Pulmonary hypertension, sickling in the placenta. I have talked about problems with my eyes and my learning disability, that I really believe that it's because of years of sickling. Having a learning disability is so frustrating. You know, not being able to concentrate, not being able to organize my thoughts the way that I want to is really challenging for me. And just knowing that I have so much that I want to do with my life, and sickle cell really is not my story, you know, it's just this road, this obstacle, that's

getting -- it's keeping me from my story, my real path.

You know, I met these two women today, Margaret and Olga. They are like my dream because they are living their lives, and I want to live that long. I want to be able to do the things that they've done and just -- they talked to me for like 10 minutes, and I have more hope than I did yesterday.

DR. EGGERS: And, Helen, we will be talking to -- we'll be learning from Olga in the afternoon. I'm going to wrap it up then and move on to the next person, on to -- is that okay?

MS. SARPONG: Yes. Thank you very much.

(Applause.)

DR. EGGERS: And we'll have Terri, please.

MS. BOOKER: Hello. I'm going to try to stay on task with my little thing that I wrote here. I'm 30 years old and I'm living with sickle cell SC. I am what one would call a healthy sickle cell patient. I would say I'm healthy because I don't take daily meds and I'm not in crisis often. Most people who know anything about the disease are usually

surprised when I say I have it.

The effects of sickle cell disease that have the greatest impact on my life is the pain crisis, tiredness, and depression. I live with constant pain sometimes, and my tolerance for pain is significant. The meds given for pain have real side effects, and it includes possible dependency, hallucinations, and the dreadful detox, the sweats, the chills, the tossing and turning while trying not to take the meds in order to avoid dependency all while still being in pain. Once coming off the meds, I thought that a gang attacked and killed my mother, but later I found out she was alive.

I'm always tired, and it's like someone said earlier, it's this heaviness that comes over your body, and it's like you just need to lay there, you just don't want to move. And there are things I would like to do, but I just can't because I'm just too tired. I have to make myself get up and get ready for the day. Sometimes it just feels better to lay down in the bed and not do anything. Once I get moving, I like to keep moving, so there is no time for me to

think about me being tired. And unlike my colleagues, I'm an attorney, and so I'm like most of them, they can just keep working, working. I can't work constantly and forget to drink and just go, go, go. I always think about my health so I don't have a crisis. I stay hydrated.

Depression sets in after I've been sick and I'm not able to live normally. When I get sick, even a minor crisis, it just reminds me of the extra precautions I have to live by. Being a healthy sickle cell patient, quote/unquote, my life, my daily life is normal, quote/unquote, however, there are periods of time when I live with pain every single day. I'm constantly aware of the changes in temperature in the weather no matter what the season because it's always too cold. Summertime, it's too cold inside; wintertime, it's too cold outside.

(Laughter.)

MS. BOOKER: And when the cold gets into your bones, it's just like serious pain. One time my friend, she threw some cold water on my back as a joke, and I was hot, and it left me in the bed for 2

days with a pain crisis. So there is no such thing as a normal day, but I try to make it as normal as possible.

The worst pain crisis I ever had was in 2003, and it would be about 10 years before I had another one like that. I was a sophomore undergrad, and I was laying in my bed, and I couldn't move. I went to the hospital and got released, and I did everything real slow until a few days later I was back in the hospital and I didn't leave for a month. I received seven blood transfusions, I had kidney failure, lung failure, and I was intubated. I actually celebrated my 20th birthday in the hospital, all which was told to me because I was highly sedated and couldn't remember hardly anything.

When I left the hospital, I had to learn how to walk again. I was a semester behind in school. I was trying to get along with life and feeling okay for a little bit after the crisis, and then I was hit with the fact that I had osteonecrosis of the hip from the medicine that they gave me to help clear out my lungs, and I ended up having a full right hip replacement at

the age of 21, and I also ended up in therapy. I had been walking with a cane for a whole year before that. So I was upset because it was a whole year before a doctor would even -- wouldn't dismiss my pain as just a pain crisis and actually just gave me an MRI to find that my pelvic bone had crushed my hip bone.

Now, as I'm a mature young adult, my health has become more of an issue for me. I have a huge fear of living alone. I would like to buy a house and move out, but it's scary because I've never lived alone. I've always had a roommate, someone who could make sure that I was okay. And although I'm capable mentally, I'm afraid to work alone, which would be really nice to do when you can't get a job that you want. I don't want to work alone because I feel like I will leave my work stranded if I get sick. There is no telling how long I could be out. I feel like I'll set myself up for failure by the mere fact of being the only employee in my business. And furthermore, I don't like telling people about my illness even when I work with a company because, one, I look healthy on the outside, so people don't really get how or why I'm

sick. After explaining what it is, people still don't get it. And people treat you differently, and it's not always in a bad way, but it makes you feel a little uncomfortable, like, "Yes, I'm okay, don't --," you know. But the thing that's worst of all is that I need to tell people because there are days that I really just don't feel well, and it's due solely to my sickle cell symptoms.

I'm looking forward to having a family one day, and as I grow older, I'm worried about carrying a baby with sickle cell. I know I will be a high-risk pregnancy. I know healthy people who have had rough pregnancies, so being a sickle cell patient, the questions come to mind, will I be able -- will I be healthy enough to carry a baby? Will I be on bad -- high-risk pregnancy? How will it affect my baby if I have a crisis while I'm pregnant? Will I be well enough after I deliver to take care of my baby and do simple things like breastfeed?

My concern for infants and young children is the fact that I don't think people are being diagnosed early enough because doctors don't look for sickle

cell symptoms. I wasn't diagnosed until I was 11, and it was a total accident. And my first real crisis wasn't until I was 19. For all ages, I worry about the care that sickle cell patients receive.

In medical school, I have two friends who are doctors. There is not much told about sickle cell. And once you go to the hospital where they're unfamiliar with your case and your disease, doctors always ask you, "So what do you do?" and the sad fact is you probably know better than they do. However, when dealing with a pain crisis, it can be hard to focus on a plan of action for your care when you went to the ER to get help.

I fear that the meds will just get stronger and there will be no search for a cure. I fear that it will always remain that sickle cell patients will not be able to get health care or life insurance policies. I fear that young patients will get pressured into taking medicine that they're not sick enough to take. Like when I was having issues with my right leg, they tried to pressure me into taking hydroxyurea, which was way too strong for me and I

wasn't, quote/unquote, sick enough to take it. It would do more harm to me than good. So I can only hope that sickle cell patients are strong enough to know that they do have choices and to question and ask about their plan of treatment.

That's all.

DR. EGGERS: Thank you, Terri. Thank you.

(Applause.)

Older Adult Perspective on Topic 1

DR. EGGERS: And, finally, we have George.

MR. CARTER: My name is George Harris Carter. I'm 68 years old and I suffer with sickle beta thalassemia zero. Today I would like to represent myself and members of Sickle Cell Association of Richmond-OSCAR as its administrator, and to patients within the nine chapters of Sickle Cell Chapters of Virginia, where I also serve as administrator.

Problems associated with sickle cell disease include pain, which some people live with every single day; chest syndrome; anemia; fatigue; infections; breathing trouble; pneumonia; stroke; gallstones;

organ and tissue damage; spleen and kidney dysfunction; complications during pregnancy; jaundice; leg ulcers; hand-foot syndrome, swollen hands and feet that become hot, red, and painful; vision problems; blood in the urine; slowed growth and delayed puberty in children; priapism or pain erections in men. Sickle cell often creates a need for hip replacements and/or shoulder replacements. We may develop pulmonary hypertension.

Loss of hearing is not listed in most publications. I've lost 80 percent of the hearing in my left ear and 30 percent in my right ear. Some years ago, during a sickle cell presentation by Dr. Wally Smith, who is here today, he mentioned that hearing loss was a problem. I stated that I had hearing loss in my left ear. Two other males said that they had hearing loss in their left ear also. Only four studies have been presented on hearing loss, and two of those by Dr. Smith.

Dental problems also occur. A nurse practitioner recently wrote, "Sickle cell disease has a great impact on oral hygiene. We have patients as

young as in their thirties that have dentures, have lost all of their teeth, because of damage from lack of oxygenated blood and sickling in the microvasculature."

I have suffered from 14 of the above 23 problems. In addition to the sickle cell, I have asthma, bronchitis, a hernia, two stomach ulcers, deteriorating rotator cuff in both shoulders, and now an inflamed stomach, which in part is from the use of ibuprofen for pain and inflammation as a result of that sickle cell.

When I have a sickle cell crisis, the pain keeps me from sleeping. I'm too tired to do some of my activities. The pain also limits my concentration and my mobility. Where in my body the pain is also determines how much I can do and how mobile I am. On the worst day, a Level 10 pain crisis, I will be in the hospital trying to get enough pain medication, IV fluids, and oxygen to get through the pain. There is no other activity I can engage in on these days. The pain has complete control to the point that three times in my life I prayed to God to let me die because

I didn't think I could take any more pain.

Medical staff will often, on a scale of 1 to 10, want to know what your pain level is.

(Laughter.)

MR. CARTER: Using that same scale in terms of my activities, if I have a Level 2 pain, I can probably go about my regular activities on a scale of 8, or 80 percent. If I have a pain Level 4, then I can probably go about my regular activities on a scale of 6, or 60 percent. If I have a Level 6 pain, I can probably go about my regular activities on a scale of 4, or 40 percent, and so on.

I worry about further hearing loss and organ damage and having a stroke, but hearing loss is one because I virtually cannot hear in my left ear. I always keep my wife on my right side so I know what she's telling me.

(Laughter.)

MR. CARTER: Thirty percent I've lost on my side. If I lose any more, the TV is going to be up so loud, she's going to leave home --

(Laughter.)

MR. CARTER: -- so I can't afford that, but I'm 68 years old, and one of the ladies in our support group is 75.

(Applause.)

MR. CARTER: Thank you. We have overcome. So let that be a lesson to those who are younger who wonder. You can live, you can survive, you can enjoy life, and to God be the glory for all that we have.

(Applause.)

DR. EGGERS: Thank you, George.

And a sincere thank-you to all the panelists.

We are not on our schedule at all, so with your permission and the permission of the panel of everyone, we're going to go about 15 minutes into lunch. I don't think anyone here is going to mind too much, right? The lunch isn't that great anyway, the kiosk.

(Laughter.)

DR. EGGERS: Anyway, so we're going to have an abbreviated discussion, and our panelists, they had complicated stories, and it was really unfair to ask

them to stick to 2 to 3 minutes. But I hope -- I'm going to get a raise of hands, how many of you felt that your experience was shared by one of the panel members? They shared a wide variety. How many felt that you saw a lot of your experience in theirs?

(Show of hands.)

DR. EGGERS: Great. Okay. We might not be able to get into all of the topics that we want to for today, but share your stories through the docket.

Let's put up the one polling question that we have. This will help a lot. It's the same polling question that we asked the pediatric group. So this is only for the adults, if you're about 23 or older. Other than acute pain crisis, because we know that would be in your top probably, what health effects of sickle cell disease have the greatest effect on your life or your loved one's life if you're here for someone else? Chronic daily pain; stroke; acute chest syndrome; fatigue; priapism or painful erection; problems with eyesight from sickle cell disease; damage to heart or pulmonary hypertension; kidney disease or gallstones; or something else not listed

above.

(Answering question.)

DR. EGGERS: Okay. Has everyone had a chance? Can we go to the results? Okay. Yes. We thought that the "Others not listed above," would be pretty high, but it looks like our number one is fatigue. I don't think we're going to get into that because we did discuss it a lot for the adolescents.

So the second highest in the in-person is the chronic daily pain, and that has been eloquently shared by our panel members, so we won't get into that symptom as well.

Next -- I have to squint to see the problems with eyesight, but the next highest one is the problems with eyesight from sickle cell disease.

And then, of course, we have the "Other" category.

So I think my colleagues would really like to know now is to let's tease apart -- we have a limited amount of time, so let's tease apart that "Other." Other symptoms that you would put in your top three -- before we do that, on the web, does it

look about the same?

MS. VAIDYA: Hello?

DR. EGGERS: Yes.

MS. VAIDYA: It looks about the same. The highest on the web is actually for chronic daily pain at 85 percent, and then next we have fatigue at 79 percent.

DR. EGGERS: Okay. Thank you. And on the web, keep sending in your comments about your symptoms.

So that "Other," does someone want to share another symptom that they would put maybe even in their top two since time is tight?

Okay. Right here. I'm just going to -- over here. If you could state your name.

MS. MURPHY: Theresa Murphy from New York City. I'm also 68. And I was -- sometimes ignorance is bliss because I didn't even know I had all these problems.

(Laughter.)

MS. MURPHY: But I think my most defining problems, and lately it's been hypertension, is

extreme pain in my right calf, which is symbolic of getting embolisms, pulmonary embolisms, and I think that it is so striking it feels like someone has taken a baseball bat and just beat your legs in. But I tell you, ignorance is bliss because I had it for many, many years, and my youngest kids -- I have four kids. I was told I was going to die by the time I was 16. I tell you, ignorance is bliss. When I saw my hematologist after I had the twins, I said, "Thank God that I had tubal ligation because I would have about 20 kids now."

(Laughter.)

MS. MURPHY: So don't believe a lot of stuff. But it's definitely, I just see so many people in worst shape than me with other diseases that I am so glad I made 68 years old.

(Applause.)

DR. EGGERS: Thank you, Theresa.

We have another symptom here and over here. Okay. I've got a couple hands.

MS. GRAY JOHNSON: Hi. My name is Judy Gray Johnson, and I have the SC disease. I'm a retired

school teacher. And first of all, I would like to thank FDA and this panel over here for being here and attempting to do something for us. This is a very complicated disease. It's hereditary. Quite often when we go to the medical profession, we are made to feel like, well, maybe there is something we could have done or should have done before we got there. We didn't have any choice in this, and so we are here having to deal with whatever the symptoms are.

I looked around the room here. I'm 71 years old and --

(Applause.)

MS. GRAY JOHNSON: -- let me say this, and I'm thinking there are not many people in here that can relate to me because 71, having the experiences of a rural community in which there are very few blacks, and I was the lone child in the community with something strange. And have you heard of liniment?

(Chorus of yeses.)

MS. GRAY JOHNSON: That was what I -- the first 5 years of my life, I was treated with warm towels, just let the water run and all, and also from

a poor background. And then from 5 until 16, I was treated with liniment. The doctor would just say, you know, splash it on me and that's it. So how did I make it to here? The pain just wore off. Every year -- but I did not talk about -- I learned over the years not to talk about it, and until -- because I said, Who is going to understand? Who is going to know? Who is going to understand? Who can help me? I wanted so badly for someone to help me.

When finally I got -- as time went on, I started thinking I've got to write a book because nobody is going to believe me. I wrote one book, *Living With Sickle Cell Disease: The Struggle to Survive*, and that is our experiences here.

Afterwards, I thought, wait a minute, I've got more to say, and that is, I wrote my second book, which will be out at the end of this month, *Resilience*. And I would like to ask that FDA, at least the panel members, read the books and then I want you to put it on your desk as a reminder of every time you make decisions or you're planning activities or whatever for sickle cell disease patients, this

would be a constant reminder of what it's really like, and maybe, is there something else I should be doing? And my website is www.judygrayjohnson.com.

DR. EGGERS: Thank you very much, Judy. Thank you. Thank you.

(Applause.)

DR. EGGERS: Thank you. So over here we have symptoms?

MS. WOODSON: Hello. I'm from Richmond. My name is Sitrena, and I was diagnosed at 42, I have sickle thalassemia, but one of the symptoms that I really think I struggle with the most is stress, which increases my blood pressure, which increases my stress and mobility issues. I have a hip replacement and now I'm having a lot of pain my shoulders, and that just again increases the stress and the hypertension.

DR. EGGERS: Thank you so much.

Okay. All right, here. We'll go here and then we'll go over on that side of the room to the gentlemen.

MS. BAILEY: Hi. My name is Kamilah Bailey. I have sickle cell anemia with beta-plus thalassemia,

and the symptoms that I experience in the "Other" category are sleeplessness where my mind is awake but my physical body is extremely tired, and I cannot reconcile my mind with my body in order to get a good night's rest, and that prolonged, that going over several weeks or several months, puts me into depression because I feel like I'm never going to come out of this pattern. And so medications for -- sleep medications and pain medications, and on top of all the medication, it's difficult to function. So the sleeplessness and the inability to reconcile my mental fatigue with my physical fatigue and get the rest I need.

DR. EGGERS: Thank you very much. Thank you.

We had a gentleman over in the middle?

MR. KARGBO: Hello, everybody. My name is Ibrahim Kargbo, and I have been living with sickle cell for 27 years. And I'm going to be honest with the audience. Until about 2 years ago, I actually stayed away from sickle cell support groups. I stayed away from anybody who wanted to talk to me about some

of these symptoms of sickle cell disease because I was scared. Who wants to hear that they may not make it past the age of 18? Who wants to hear that they may not be able to have children? And who wants to hear that they will not be able to graduate college because they have sickle cell disease, something that they had no control over?

And one of the symptoms or consequences that I have experienced with sickle cell disease is not being able to spend time with my friends and family. I can deal with the pain. I go home, take my oxycodone, my Dilaudid, the Tylenol, call it a day. If I have pain that I can't handle, I go to the ER. I can deal with the pain. It's really the social aspects of sickle cell disease that's cutting deep into my soul. Not being able to take the person I am with to dinner because my joints are aching, that's really what I go through with sickle cell disease.

DR. EGGERS: Thank you. And I hope that you're hearing in here that you can make it past 18, you can go to school, you can have children, so it's an inspiring message from all of us here.

Are there -- pardon me? On the -- do we have web summaries? Okay. And I want to -- let's see. I'm going to tee up for one phone call, one lucky phone person, to talk about a symptom that they haven't heard. Please keep it to a symptom that you haven't heard described today. And we're going to go with a summary of what we've heard on the web.

MR. VALENTINE: Okay. So we've been putting together a list of symptoms that went into that "Other" category, and we've received a lot of comments, so we'll try to run through quickly.

DR. EGGERS: Great. Thank you, web.

MR. VALENTINE: One we received is issues related to pregnancy such as miscarriages and ectopic pregnancy, gout, sinusitis, allergies, mental effects, several comments about insomnia, migraines, teeth issues, severe ear infection leading to hearing loss, brain aneurism, ulcers, and jaundice.

DR. EGGERS: Okay. Thank you.

I heard a lot of echoes of that. Is that a list that you in the room agree with? Raise your hand if you heard your own symptoms in there.

(Show of hands.)

DR. EGGERS: Great. Okay. Before going to the phone, colleague. Okay, yes, yes.

MS. PETERSON: Hi. My name is Nikki Peterson. I'm 40 years old, hemoglobin SS. I've had four strokes and two TIAs. One of the symptoms that I've suffered with is compression fractures in my back. I have compression fractures T1 through T5 and L4 level, and that has really been a problem that I have suffered with for the last I would say 3 or 4 years, and each time I get a compression fracture, I have to start all over with rehab, with learning how to walk, and sometimes with speech.

And also one of the problems I've had as a female is going through menopause at an early age, being 20 years old and having a crisis in my ovaries and my uterus and going through menopause and not being able to have children, and that's been one of the symptoms that I've suffered with. It's not only physically, a physical problem, but it's also an emotional problem being a young woman who would love to have children one day, but I'm not able to. And so

that's one of the problems that I go through.

DR. EGGERS: Thank you so much. Okay.

Any symptoms that my colleagues wanted to -- any final questions from my colleagues?

DR. VERDUN: I just wanted to touch on retinopathy in particular since it was close to 30 percent, and if anyone wanted to touch on retinopathy.

DR. EGGERS: Okay. Back there. And if you could just state your name again, please.

CAROL: Carol from New Jersey. I have sickle cell retinopathy. Oh, I have so many surgery on my eyes. Actually, my mom lost her vision in her left eye, and she died from complications to pulmonary embolism. But about three times I almost lost my vision. I was at work one day and I saw this big black circle in my left eye, and the circle got bigger, I had no clue what it was, and because I work in occupational health, I went to the clinic to ask the doctor what was happening, and they rushed me to the hospital right away, and then they told me there was so much blood in my eye they couldn't do anything. The doctor sent me home and I slept upside-down for

like two nights, and then I went back and had surgery, and they had to coagulate so many vessels. At least it saved my sight, so I can see now, but I have tremendous problems with my eyes.

But one of the issues that was never discussed there, growing up in Jamaica, I did not know I had sickle cell. I found out I had sickle cell when I was 25 years old. And as a child, I had severe pains in my mouth, and it felt like neuralgia, and I lost half of my teeth because they were trying to figure out how my teeth look well, but I was having this pain, they didn't know where the pain was from, so they took half my teeth out. But I still have this pain up to this day, and the pain would start in my mouth, go up into my ears, into my head. So whenever it gets cold, like anything below zero, I do not go outside, because I end up with that pain.

So there are other things, too, but I won't go through that.

DR. EGGERS: Thank you. Okay. And do we have one phone comment, Operator?

OPERATOR: Nikki, your line is open.

DR. EGGERS: Hello?
OPERATOR: Nikki's line is open.
DR. EGGERS: Nikki?
NIKKI: Hello?
DR. EGGERS: Hi, Nikki. This is Sara. Do you have a comment?
NIKKI: Oh, yes. I'm sorry, I had the phone put down for a second. Yeah. I had a comment. By the way, I'm 25 years old, and I was diagnosed with sickle cell when I was 5 months old, which is pretty young. But some of my struggles that I have is I was diagnosed with pulmonary hypertension when I was around 20, and it has taken a devastating toll on my life period. I have to have oxygen all the time. With sickle cell, you're already oxygen deprived. So I have to have oxygen to breathe everywhere that I go all the time, and being 25 years old, that's really difficult in itself. I've had a thoracotomy of my lungs completely in the hospital. I also had my gallbladder removed and my spleen, and that was all before I was a teen. So my life has been a big struggle with it, so day in and day out I am extremely

tired, exhausted. Some days I can't even get out of bed. And I live in Florida and there is a lot of hostile weather. A lot of people think that it doesn't get cold here, but it gets really cold here. And our weather changes, so that was like the other day I went to a Super Bowl party and it was 80 degrees, and now it's -- it snowed. So my body, my body generally doesn't get to adjust to the weather change, so today I'm like having a lot of pain because the weather has drastically changed within, you know, a day.

DR. EGGERS: Thank you very much for that comment, Nikki. One final thing?

NIKKI: My final thing would just be I definitely have fears and worries in regard to my sickle cell, but just watching and listening to the comments that have been said today, I am so proud to be here and be able to listen to people that have actually made it so far. I thought I was not going to make it past 9 years old, and I'm 25 now. So it really gives me hope and I think it gives all of us hope to let us know that we can make it further than

what is expected definitely.

DR. EGGERS: Great. Thank you, Nikki. I think that is a perfect comment to end before lunch.

I know everyone didn't get a chance to speak. There is still another topic coming up in the afternoon.

We're going to take 45 minutes for lunch if that's okay. So please come back at 1:30, and we will have our Topic 2 discussion. And if you have any questions, come find us. Thank you.

(Lunch.)

Afternoon Welcome

DR. EGGERS: Okay. Can we start to make our way in and we'll get started? And as you make your way in, I'll refresh what we're going to be talking about in this topic. The format is going to be the very same. We're going to be focusing this afternoon on patients' perspectives on treatments for sickle cell disease. This includes what you're taking, what you're doing, how well those are working, what's not being addressed as well, what do you look for in an ideal treatment? And then we'll also have a

discussion on if you imagine you had the opportunity to participate in a clinical trial, and what would your thoughts be on that?

And as we get started, I am going to make an obvious observation that the lunch line was very long, and we apologize for that. We thank you so much for your patience. And if you need to finish your lunch and come on in or bring your lunch on in, please feel free to do so.

And so with that, we're waiting on one panelist, or a couple of panelists. If you're on the Topic 2 panel -- actually, if you are finishing your lunch and you're on the Topic 2 panel, go ahead and finish it and then come on in when you want. This is a very informal setting. We have some fantastic panel members here today to give us just a flavor of what it's like, about your experiences with treatments, and then we will broaden it to the facilitated discussion.

Are all the photo ops done? We've got all the pictures?

Panel Discussion on Topic 2: Patients' Perspectives on Treatments for Sickle Cell Disease

DR. EGGERS: So we have one caretaker, and I'm going to let him get started in the discussion, and that is John, and then we'll work our way down. Again, please try to keep it to 2 to 3 minutes and focus on the discussion questions that we have. Can we put the discussion questions up? You guys already know the discussion questions, but for everyone else in the room, these are the types of questions we are asking them to address.

All right, John. Oh, and I'm sorry, push your little red button on your mike. There you go.

MR. MOORE: Good afternoon, everyone. My name is John Moore. I have a 14-year-old son who has sickle cell and it's been a real challenge for 14 years trying to keep him healthy and happy. As a parent, you really feel an obligation to give your children the best, and when you feel that genetically you've given them something detrimental to their health, it really hurts, and watching your children suffer is something I wouldn't wish on anyone.

We are at this point considering a bone marrow transplant because we really haven't found any

of the treatments to be totally effective at diminishing or eradicating his pain. He's had a really bad year this year, hasn't been to school more than maybe 2 months out of the whole school year. So we are definitely on the track to try to get him cured, and as far as we know, the bone marrow transplant is the only cure.

At this point, he has gone through everything that we've been told is a way to treat sickle cell pain, and I don't really know that it treats his pain, but it does make it tolerable. He's taking morphine-based medications, supplying heat during all kinds of Jacuzzis, anything to massages, to try to ease it, but it's just so systemic that it's very hard to deal with.

At this point, we're just hopeful that this bone marrow transplant is going to be the cure, and now that they have a haplo method, his mother can be the donor, his mother or I can be the donor, so we don't have to have an exact match, and they don't have to totally stop the production of his bone marrow, it only has to be suppressed for a period of time. So

knowing that it's not as life risking as it had been, we're willing to take the chance, and he's all gung-ho for it. So we just at this point have decided that that's the only thing that we hold out hope for.

So I look at all you adults out here, and I know that your parents have gone through what I went through, and it's just a really hard thing to watch your children suffer. I don't know why the bone marrow transplant hasn't been more broadly advertised because as far as I know, speaking to all of the people that are adults who have had sickle cell, their childhood was very, very tough. Fortunately for us, our son was diagnosed as having it at birth, so he was treated from the very beginning. We have one relative who was never diagnosed until she was maybe 7. They even thought her parents had been abusing her because she was having these pains that they couldn't identify the source of, so in the State of Pennsylvania, fortunately they do test for it at birth.

At this point, I was wishing my son was here. We had a blood transfusion yesterday. He's feeling better, but he just wasn't up to coming. So

as his representative, I want to thank everybody here and anyone who has any power to do anything to make sickle cell a more publicly aware disease that should be treated, I would say, more aggressively, I thank you for your efforts.

DR. EGGERS: Thank you, John.

(Applause.)

DR. EGGERS: And next we have Tina Kay.

MS. HUGHES: And they told my mother, "You need to have a hysterectomy so you won't have any more children like this." She was pregnant with my brother, and the doctor was referring to me. My mother had choice words on the Army base in rural Georgia, and she never went back.

Today, treatment for sickle cell patients is go to the hospital, wait, wait, wait some more, and it doesn't matter that you're at a Level 9 or 10, it doesn't matter that you're in the midst of a stroke, it doesn't matter that you can't walk, it doesn't matter that the ambulance brought you in on a gurney and they sit you out in the hallway.

Treatment is push things like hydroxyurea on

us, which one clinical doctor told me was nothing but rat poison, and that's when I stopped taking it. I figure my people lived in this world way before pills, so I started to look for other things to do, like acupuncture, cupping, massage therapy, the TENS unit, and, of course, everybody's favorite, the heating pad.

(Laughter.)

MS. HUGHES: I think we're all like Lionel (sic) on *Charlie Brown*, if we don't have our heating pad, we're not going to make it. But I think that in today's time, research is moving a little faster, and believe it or not, there are researchers in Birmingham, Alabama, where I am from, who are looking at more than just a bone marrow transplant, they're looking at, how can we cure us, us brown people, with our own skin? It's not in the medical journals yet, but, believe me, in the next couple of years, you will be hearing about it.

My life as a child, was pretty -- I had a pretty good childhood. I stayed outside a lot. And believe it or not, Howard University has done research showing that Vitamin D from the sunshine is the best

thing for us. I worked a job where I worked outside about 80 to 90 percent of my job, and I traveled the country for close -- about 250 to 300 days out of the year. I never had a crisis, I never had a cold, because I was in the sunshine all the time. So maybe it's something that God is trying to give us, but we're staying in the house in the bed. Maybe we need to go pull a lounge chair out in our yard and get some sunshine.

(Laughter.)

MS. HUGHES: I've tried other things like compound pharmacy, eating natural organic ingredients in foods to help decrease the pain. I've tried Zija, Ambrotose, Body By Vi. You all know them all because they approach us saying, "Oh, you can be cured."

So I contracted hepatitis C unfortunately from a blood transfusion in the '80s. And sometimes the things that we think are helping us can sometimes help hurt us.

Another thing that we were talking about during lunch is constipation. Because we have so many toxic things in our bodies, it's so easy for us to

become constipated. So eat your green vegetables, the fruits. A lady shared with me, a doctor in the crowd, nana (ph), nana juice. I'm going to try it. Alkaline water, I drink that religiously to try to help get my pH balance back into balance because we are really acidic from the toxins that we have in our bodies.

So don't be afraid of other things. Try other things. Mix Eastern medicine with Western medicine. It's something for the Chinese to be able to live until they're more than 100 years, it's something to our ancestors who lived past the 100 years old by going back to the earth. Maybe that's what we need to start doing.

DR. EGGERS: Tina, do you have one more thing?

MS. HUGHES: Okay. I spend my time as a journalist. I write nationally for several publications. I'm a published author. I have two radio shows that can be heard across the world. I speak all over the country. And don't own this disease, don't say it's yours. It's something that you carry around with you, but don't own it.

DR. EGGERS: Thank you so much.

(Applause.)

DR. EGGERS: And you'll be followed by Lakiea.

DR. BAILEY: Thank you. I am --

DR. EGGERS: Oh, put your --

DR. BAILEY: Oh, sorry. Thank you. Better?

DR. EGGERS: Yes. Great.

DR. BAILEY: I am Dr. Lakiea Bailey. I am a research scientist and patient advocate. I have sickle cell disease hemoglobin SS. Well, actually I have hemoglobin S, hemoglobin Monroe. Monroe is another mutation for the hemoglobin, and it makes completely unviable hemoglobin. So essentially I just have one gene that works, and it produces sickle cell, so that's sort of how I present.

Our first question was about medical protocol, what we do. I am currently on folic acid and naproxen and hydrocodone as needed. I receive a monthly infusion of Desferal for the iron overload. Blood transfusions, but I typically insist that my hemoglobin fall below about a 6.2 before they

transfuse me, especially if my retic count suggests it's going to rebound on its own. My baseline is about 7.4, so 6.2 would be really too low for some people, but for me, it's doable.

Naproxen works really well for the bone pain. Supplemental oxygen but only during travel or when I'm going to conferences in high -- like I have a particular yearly conference I go to that's in the mountains of North Carolina, oxygen too low, so I take my oxygen then, but that's the only time I use the supplemental.

When hospitalized, I'm typically treated with morphine and Benadryl. It makes the pain somewhat more tolerable, but then I need the Benadryl for the itching. I have not come across anything that really does a great job on the pain.

My main objection -- it asked about that -- my main objection with my current medical treatment is that it only addresses pain. I was determined to be a non-responder for hydroxyurea, so a lot of people take the Hydrea, but I was a non-responder, and there is a small percentage of the population that just does not

respond. My fetal hemoglobin did not increase, but what did increase were the things that you don't want to increase, fingernails turned black, hair fell out. I would have dealt with all of that because, I mean, they make some awesome wigs, but I wasn't getting any support, it wasn't helping me, so I was a non-responder for the Hydrea, so that wasn't an option for me.

The oral medical for iron overload that most people take, put it in the orange juice, drink that chalky orange juice, one of the rare side effects is a decrease in hearing acuity, and they measured that my hearing decreased while on it, so I couldn't take that, so that's why I have to do the infusions for the Desferal to treat the iron overload.

Alternative treatments, so other than those things, I attempt to treat and prevent sickle complications with diet. I have noticed a very strong correlation with not only what I eat but when I eat it and how I feel the next day. So I try to use my diet a lot to monitor that.

This will sound weird, but I take a men's

vitamin. Okay, so iron overload, I have to avoid iron. When you grab the women-formulated and the men-formulated multivitamins, if you look on the back, they're generally the same except the men does not have iron in it. And so my multivitamin is a men-formulated multivitamin, which is the same, I haven't turned into a man, but it doesn't have iron.

(Laughter.)

DR. BAILEY: So I take that. I drink dandelion tea for the liver complications. It tastes the way I imagine freshly mowed grass probably would taste --

(Laughter.)

DR. BAILEY: -- but I have noticed that it helps with the liver pain, the inflammation upon palpation. It helps with the liver. So I don't remember who told me about dandelion tea, but I drink that. It's, like I say, gross, but it really does help.

And then one of my alternative treatments -- and I know a lot of people do this -- I try to detox with detoxing stress in my life. So I go home, I go

let my mom just mommy me, and I find that waking up at 3:00 a.m. to my 5-month-old nephew bouncing around and screaming actually does wonders for my health. So I do that.

(Laughter.)

DR. BAILEY: And my other alterative treatment, if you would consider alternative, would be faith and a whole lot of prayer.

Untreated complications was the next thing. My current treatment focuses entirely on pain and iron overload. I would love treatment that would address the underlying anemia and the vaso-occlusive events. The anemia, of course, is linked to the excessive fatigue, and the fatigue is a major, major issue for me, and then the blood transfusions, which causes the iron overload.

So if I could address that as well as those vaso-occlusive events, mostly my treatments, they're looking at downstream, they're looking at afterwards, but I would like to address upstream. Can we look at it before things get that bad?

Another untreated issue is memory and the

retention of information, that's a constant. I am completely incapable of learning in class. I show up because it was required of me. I have to learn at home, just study on my own. I learn more 20 minutes on Google than I will 3 hours in class. And I was at a talk recently that actually showed a very strong correlation between vascular pain and memory loss, and I looked into it, and I didn't see any studies where they looked in it with sickle cell or even sickle mice, but it makes sense to me that if it's the case, the particular type of pain we experience, vascular pain, vaso-occlusive pain, might be related to some of the mental acuity deficits that we're experiencing. So that's untreated.

And I have had the -- and then this actually came up during the talk -- I have had the uterine crisis before. It never occurred to me that that could lead to early menopause and infertility until others started talking about it. So that was good information for the individuals that shared that, I really appreciate that, and that is another thing to put on the altar.

DR. EGGERS: Lakiea -- oh.

DR. BAILEY: My ideal treatment, you had ideal treatment. My ideal treatment would decrease duration of sickling events and increase my hemoglobin.

And you asked about experimental treatment participation. When considering whether or not to join experimental drug trials, I typically weigh the known and theorized risk of the drug against its potential benefit. I also consider the phase the drug trial is in, how long it's been tested, and the number of previous patients enrolled. So the severity of my disease is not yet to a point where I am bold enough and comfortable enough to be among the first few trials. I know that sounds counterintuitive and kind of hypocritical as a research scientist, but right now I feel like I want everybody else to go first.

(Laughter.)

DR. BAILEY: And so that addresses all of the different points that you asked.

DR. EGGERS: Thank you very much, Lakiea.

(Applause.)

DR. EGGERS: And then we have Olga.

MS. BARNWELL: Good afternoon, everyone. I'm glad to be here. I am 61 years old, living with sickle cell disease. I am the oldest of four siblings, three who do have sickle cell as well, two with the trait. My protocol is basically folic acid, elimination of stress. Say no to stress as fast as you can. Okay? I use a lot of therapeutic methods. I work with a team of naturopathic physicians, and a big part of my protocol is aromatherapy. I have included with that aromatherapy massage therapy. I do Yoga and Tai Chi. The Yoga helps with the breathing, okay, and when we get shortness of breath, it does help with breathing. Meditation is also wonderful for that. So I typically try to get my meditation in, in the mornings, and then there are evening meditations that are done as well.

During my daily routines, I have a pain medication, a pain oil, that I carry with me. So I don't have to take the pharmaceutical meds that they prescribe for us to carry, I just have my pain stick, rub and go, rub and go. So that's basically what I

do, I rub and go.

Side effects that I do have, in June I did have a TIA, so I am being treated with that, for that, and I'm undergoing quarterly blood exchanges for that. I'm doing that on an outpatient basis basically. And what I do is I prep with my oils when I go in to have the lines put in, and then after the line is taken out, I have certain oils that I do use as well that helps with the incision, healing of the incision, and help reduces some of the pain as well that I experience from that. When I'm having those exchanges, I do have lack of energy, and it takes me a couple of days to bounce back from that because of the exchange of the blood that occurs.

Basically this is a lifestyle choice for me. We do have choices. God did not put these plants and herbs here for us not to use. The side effects from the natural therapy, I have none, I have no side effects. We have to find what works best for us and stick with that protocol. When I do have to go into the hospital, I have my oils with me, I'm using them, I'm prepping myself before I go in, and the medication

I use is Dilaudid if I go, and the last time I went in, I had a bad reaction to that. So I'm like my body is just rejecting all of the pharmaceutical medicines because they're toxic. So my eating, I try to do as much organic eating as possible, do juicing, juicing your fruits and your vegetables as much as you can as well as --

DR. EGGERS: Okay, any final thoughts?

MS. BARNWELL: Pardon me?

DR. EGGERS: Any final thoughts?

MS. BARNWELL: Final thoughts is to continue to live every day out and make every day a victory, make every day a victory.

(Applause.)

DR. EGGERS: Thank you, Olga. Thank you.

And next we have Anthony.

MR. BRAXTON: Hello. My name is Anthony Braxton. I'm 31 years old. I have sickle cell SS. And I've been through many complications of sickle cell. When I was 14, I had my gallbladder removed. About 5 years ago, I had an ulcer on my ankle that I couldn't explain, had no idea where it came from.

Also, about 7 years ago, I had issues with priapism that it started as an adolescent, but about 7 years ago I had a priapism that lasted 4 days, and I went from ER to ER and no one really could help me. And I went to Johns Hopkins, I'm from the area, local area, and I went to Johns Hopkins in Baltimore, and they were able to help me. And they also have a sickle cell infusion center, it's the only one in the area, where we can go and get treatment and meds and not have to wait in the ER lines, and they have the doctors that specialize in sickle cell, and that's one thing that we actually have to do, is create more sickle cell infusion centers because, as all of the other panelists and patients and the audience knows, waiting in the ER for hours and hours and no one knows what to do is excruciating, there is no other way to put it.

At the current time, I'm on several different medications. Hydroxyurea, I was actually on 2,500 milligrams starting in 2004. Lately I had to reduce that because I started seeing the effects, the side effects, of it, and it became more of a

hindrance, and it also developed kidney issues with me. I have FSGS, a form of kidney disease, and that was kind of in correlation to the high dosage of hydroxyurea that I was on, so that's been reduced. Folic acid, OxyContin and oxycodone as needed. And lisinopril, I take that from the gout that I have developed from sickle cell as well. I have pain, swelling in the feet, leg area, very often.

The combination of these medicines and treatment has greatly reduced my hospital stays and the frequency of my severe crises. For the present time, this seems to be the best combination I've had, you know, in my 31 years, and I'm pretty satisfied with these treatments.

The treatments that I am on now, I do a monthly red blood cell exchange. I have an implant Vortex port, and that helps with the exchanges. Throughout my life, I've been hospitalized over 100 times, so most of my veins became scar tissue, so that was always an issue. Within the last year, I remember being hospitalized and having eight different people try to place an IV in me, and it was terrible. So the

Vortex port is something that was implanted, and it's been pretty good in my treatment.

Downside of it, when they access the port, it's painful, it's painful. It gets clogged, so they have to put declogging medicine in it, and it's a process. But I've learned that the things that may hurt me now may help me to have a better future, so I kind of deal with that.

Aside from the treatments and medicines that I'm on now, I have a regular exercise routine for the most part. As we get older with sickle cell, you kind of learn what you can do and what you can't, so when I can, I try and exercise, and I never try and do too much because it will end up hurting me in the long run, but I do try to incorporate exercises daily, even if it's only for 10 to 15 minutes, I try to get as much physical activity as I can.

Also, I have changed my diet. I eat a low sodium diet, also a lot of organics, natural foods. The things that are easily accessible for us to eat are the things that are so bad for us, you know. And what we need to do as a whole is just concentrate on

going back to nature, going back to these natural foods, natural supplements, herbs, all types of things, instead of all of these manmade products and all of these things that are loaded with so many bad things.

DR. EGGERS: Any final thoughts, Anthony?

MR. BRAXTON: Yes. My thoughts is there are treatments out right now for sickle cell and curing sickle cell, but what we need to do is, yes, we need more volunteers, and like she said, nobody wants to do it, but you have to think long term. If any of you guys have kids, you know, would you rather your kids go through the process of being in trials or would you rather do it for them?

DR. EGGERS: Thank you so much, Anthony.

(Applause.)

DR. EGGERS: And, finally, we have Adam.

MR. BUNDUKARMA: Is this thing on?

DR. EGGERS: Yes.

MR. BUNDUKARMA: Hi. I'm Adam, and I'm 48 years old, and like everybody else, I have sickle cell disease, and I hope to get to 68 and 71 like everybody

else.

I have a lot of issues that everybody else has, so I'm not going to bore you with the long list. I'm nervous enough as it is. But there is a lot of titanium and plastic and chemicals and tape and nails holding me together, so, you know.

(Laughter.)

MR. BUNDUKARMA: Yeah, okay, so the questions are right here. I guess I'll just stick to those.

Okay, yeah, so I'm taking a lot of medications, and they help, various treatments. Like my friend here, I have kidney issues which developed, and that was the craziest thing in my life because that added a lot more treatment to my plan, I guess I should say. Yeah, and I have like everything that everybody else has. I feel like really stupid sitting here listing off things because I actually feel pretty lucky.

I had medical parents, my parents are in the medical profession, so I got really good treatment when I was a kid, and my pain was managed really well.

I actually never saw a hematologist until I was, gosh, maybe 36 years old. And I actually see the wonderful hematologist at Johns Hopkins, and thanks to her and her staff, I'm still alive. She's the best hematologist in the world. Sorry.

(Laughter.)

DR. EGGERS: So, Adam, you had some really interesting thoughts on participating in a clinical trial, and I'm going to ask you to share those.

MR. BUNDUKARMA: All right, you wanted me to mention that stuff.

Okay, yeah. So like I was -- I guess when I was 18, 19 years old I participated in the trials for hydroxyurea because my parents made me, not because I wanted.

(Laughter.)

MR. BUNDUKARMA: And I'm really glad that it's helping people today, so, you know. I didn't want to do it, but now it's great. At this point, they, my hematologist and her team, they present me with any options for doing trials or tests or things like that, and I always, always say yes. There has

only been one that I haven't qualified for because of some issue, but, yeah, I do anything I can to help.

One of the things that I do believe in now -- I haven't always -- is being vocal about sickle cell just because I think that I'm probably in the place that I am because I've spent my life being quiet about it trying to fit in and be like everybody else.

So, yeah, one of the things that I have to say is a big struggle with managing sickle cell is stress, like everybody else says, but the stress for me comes with not wanting to actually do it, and also because I fought so hard to be a success in my life because I've had parents who were super successful, drove me crazy.

(Laughter.)

MR. BUNDUKARMA: So like I have like a really demanding job, and it's really, really hard to manage all the doctors appointments, medications, different treatment options, things like that, and work a full-time job and, you know, live. And so, yeah, that's one of the biggest, biggest problems.

I don't know, nobody mentioned today -- a

lot of people mentioned stress, but I don't think anybody really mentioned today that I believe with sickle cell patients that it's -- we don't ever relax, and I don't ever relax. I would love to see somebody make some famous horse pill or something --

(Laughter.)

MR. BUNDUKARMA: -- that would just, I don't know, calm me down or something. I never relax, it's so annoying.

I wasn't going to tell anybody this, but I had two joint replacements on my knee and my hip because of avascular necrosis and it advanced and I had so much pain I couldn't walk and everything, and they told me that the only thing they could do was do the joint replacements, so, yeah, that's where the titanium comes in. But, yeah, when I was recovering from those, it was really hard just because sickle cell patients, our muscles are really, really tight, and so it's hard to do things where it requires flexion

I don't know. What else do I want to add?

DR. EGGERS: Any final thoughts?

MR. BUNDUKARMA: Yeah, yeah. I'm going to shut up now.

(Laughter.)

DR. EGGERS: These were fantastic.

(Applause.)

Large-Group Facilitated Discussion: Topic 2

DR. EGGERS: Is it on? Yeah.

I want to extend another sincere thank-you to the panelists. It really does take a lot of courage to come up here in front of a large crowd and share such personal stories, and you each gave such a nice range of treatments that you're taking, and I think it really gave us a good picture of sort of your whole treatment. Were there any clarifying questions from any of the panel, any of the FDA panel, for any of the panel members?

DR. FARRELL: I think we would like to know something about the use of iron chelators, what percentage.

DR. EGGERS: Did any of the panel, did any of you use iron? Okay. We'll get into that in a second.

Okay. Let's put up the polling question. You still have polling questions in the afternoon. The afternoon isn't free of polling questions. What we would like -- and this is for everyone, this is the pediatric and the adults, to give us a sense of how much -- what the range of treatments you might be taking. In the past year, have you or your loved one used prescription medicines or medical treatments to treat sickle cell disease? So this is in the past year, and you can check all that apply. Folic acid, prescription pain meds, hydroxyurea, blood transfusions, oxygen therapy, antibiotics, transplants, other prescription medicines or medical treatments, no prescription medicines or medical treatments, or you're not sure.

(Answering question.)

DR. EGGERS: And on the web, you may have to scroll down to see all those answer choices if you're on the web.

(Answering question.)

DR. EGGERS: Okay, has everyone had a chance to answer? Good. Okay, we'll go on.

Okay, there are a lot of treatments being used. Folic acid and the prescription pain meds as well as the antibiotics. Let's see, I'm looking, G. So 14 percent of you have had transplants, bone marrow transplants. We'll follow up on that in a second to get your thoughts on that. Very few of you are taking nothing, 4 percent.

On the web, are they similar numbers? Pardon me?

MS. VAIDYA: It's similar.

DR. EGGERS: Oh, similar. Okay, we're getting the A-okay that we're similar on the web. Okay.

So the few treatments that we want to follow up on in particular, and the first one, the one that Ann said.

Ann, can you repeat which one you want to know about?

DR. FARRELL: I'm just wondering about iron chelator use.

DR. EGGERS: Okay. Does someone in the audience want to talk about that first? I know we

have Lakiea. Okay. The microphone is coming.

MS. RENE: You can hear me.

UNIDENTIFIED FEMALE SPEAKER: For the web.

DR. EGGERS: Actually for the webcast, we would have --

And your name is Nancy.

MS. RENE: It's Nancy. And my grandson takes Exjade, and he's been on it since he was probably 2 years old when he started his blood transfusions, and we're very thankful because otherwise he would have to be in the hospital hooked up to an IV in order to chelate. This he just does at home, and he hasn't had any ill effects.

DR. EGGERS: Back there?

MR. VALENTINE: I take Exjade as well, but mine was put on hold because I've been getting like a lot of burning every time I drink it as well as I had real bad auditory and visual hallucinations, and I started losing my hearing. So I did have it before when I was younger, like you have, with chelation. I had the chelation before and didn't have any of the side effects like I do now when I take the Exjade, so

-- does it work for you, the chelation, does it work better than the --

DR. BAILEY: The intravenous?

MR. VALENTINE: Yeah.

DR. BAILEY: It works well for me, yes.

MR. VALENTINE: Did you take both? Were you on the --

DR. BAILEY: I did. I tried the oral. I had the decreased hearing, so I had to come off of it, and it was working and I would have stayed on it except for the hearing, so I had to go intravenous.

MR. VALENTINE: And, now, do you guys use the cups? I met an Exjade rep and she gave me a -- like I guess there is supposed to be cups given to us where it actually has like whatever dose you're on, and then you mix it, and it gives you a certain time? So it's battery-powered and it has a timer which you're supposed to go by to mix the medication instead of have to constantly redoing it and --

DR. FARRELL: Thank you very much.

DR. EGGERS: Did that answer your question?

DR. FARRELL: Yes. I was interested in

seeing how well these medicines work, and clearly there is a need perhaps for the development of more effective iron chelators, especially with the side effects.

DR. EGGERS: So maybe I could ask a question with a show of hands. How many feel that those treatments are working for them, those of you who are taking it? Show your hands?

(Show of hands.)

DR. EGGERS: A couple? And how many of those of you who are taking those find them not as effective as you would like?

(Show of hands.)

DR. EGGERS: Okay. All right.

As far as other treatments, are there other treatments that you would like to know about? Hydroxyurea? We heard a variety. We heard that Lakiea was a non-responder.

Anthony, you felt like it was working well for you.

Does anyone else feel like it's -- want to share their story of how it's working well for them?

(Show of hands.)

DR. EGGERS: Okay, in the back there with the tan shirt.

MS. McCLINTON: I wouldn't say working well. I was on hydroxyurea when I was younger, as a child. At the time I needed it, I was averaging about three hospital stays a month, and I also lived in Boston at the time, so cold weather was horrible for me.

I took myself off of it when I was an adult because at the time the doctors couldn't tell me the long-term effects of taking the drug. I don't want to substitute sickle cell for another disease that I know nothing about. I've been living with sickle cell for a long time, I kind of know what to expect. I don't want to trade it for something else. So I'm one of those who get pushed hydroxyurea all the time, but I refuse it. I don't want to take it.

DR. EGGERS: Okay. Thank you. Over here? I think in the pink? Right here first with Judy.

MS. GRAY JOHNSON: Yes. I initially was introduced to hydroxyurea many years ago when I was in the hospital and I had been hospitalized several times

close together, and this doctor, hematologist, just suddenly appeared and did not examine me or anything and just wrote out a prescription for me to take hydroxyurea. And so I was released home, and I did as expected, filled the prescription, and eventually I became paralyzed and I remember calling the doctor saying, "Something is wrong," but they did not respond quick enough, so I took myself off of it.

So for many years after that I would not take it, but now after that, a few years ago, maybe about 4 or 5 years ago, I was going to the hospital many, many times, and I was really getting scared, I said maybe I better take another look at this, and I had been discussing this with my new hematologist, and he put me on 500 milligrams of hydroxyurea. So that, coupled with Procrit, has kept me out of the hospital for the last 3-1/2 to 4 years. So it worked in that regard.

DR. EGGERS: Thank you, Judy.

MS. GRAY JOHNSON: One thing I wanted to say about the Exjade, I've been on that, too, and I took it for about a month and a half, that was because I

had a high iron, and that medicine is very, very expensive. You're talking about 5,000-plus dollars for a 1 month's supply. So when people are asking for it, just know that we need help with that. It just so happened that my insurance took care of most of it, but my copay, in addition to all of my other medication, I mean, you know, that made it even higher for me, but I paid $50 a month.

So while, yes, it can work and it can work and all, but it's very expensive and sickle cell patients need help with that.

DR. EGGERS: Thank you, Judy.

MS. GRAY JOHNSON: $5,000 for 1 month, 5,000-plus for 1 month, that's saying a lot.

DR. EGGERS: Thank you, Judy.

Yes. Yes. Go ahead, Jonca.

DR. BULL: I'm just wondering, given the large role that transfusions play, if there have been any challenges with transfusion reactions.

DR. EGGERS: Okay, we have some hands.

Go ahead, Andrea, you go.

NICOLE: Hello. My name is Nicole. I have

sickle beta thal. I would say in regards to transfusions, because I've had a number of them over time, I've developed a number of antibodies that has caused me a couple different things. One thing would be future blood accessibility to that blood. When you do develop so many antibodies over time, it takes you longer to get blood that you may need for a transfusion. And then also I would say for the transfusion part was -- ooh, that must be the memory thing we were talking about earlier --

(Laughter.)

NICOLE: -- being able to have transfusions, I actually got an antibody that caused me to have pulmonary embolism. So there are a lot of different side effects to having a number of transfusions over time.

DR. EGGERS: Thank you.

One more on this and then we'll go on.

YOMI (ph): My name is Yomi. I have sickle cell SS. I was actually on hydroxyurea for about 11 years, and it was working good for me, but I wanted to have kids. So in talking with my hematologist, they

recommended transfusions. And we started out with whole body transfusion, the full exchange. They would draw out almost everything and give you equal volume. That was okay for a while, but then I developed antibodies and I actually had a miscarriage that we were wondering whether it's because of the antibodies and everything. So now she has changed it to simple exchange where they will take out 1 pint of blood and give me 2, and so far it's been good. It seems like some of the antibody thing has been -- I don't know if it's red to white, but the results look better, but we're still experimenting because that's just like 3 or 4 months.

DR. EGGERS: Okay. Thank you.

Yes, Tina.

MS. HUGHES: I failed to talk about pregnancy, and when I was pregnant, I had to get blood exchanges, and they also thought that my baby, while inside of me, would have to get transfusions as well. So weekly I would have to go to the high-risk doctor to make sure she was getting enough blood supplied to her brain. But I've had really bad reactions to

transfusion therapy, well, exchange now. I blew up like a Michelin Man. It's real hard for me to find blood, for them to find blood for me, now because I have lots of antibodies. Sometimes I have to wait 3 to 4 days for them to find blood somewhere in the country.

DR. EGGERS: Thank you.

Okay. Any more questions on -- okay. I was going to ask about transplants. Do you want to -- was that your -- okay. Let me ask about transplants, and then I'll come to you.

So there were some in the room who indicated that you've had a transplant. Would anyone share their story about how that's their experience with that?

(Off mike comment.)

DR. EGGERS: I'm going to -- is it -- no, I don't think it has to be specifically.

(Laughter.)

DR. EGGERS: Okay. We'll come to you, Margaret.

MS. MORGAN: Yes, my name is Gwen Morgan.

I'm from Atlanta, Georgia, and I have a son with SS disease. He received a transplant last April. Because he's the only child, we could not find a match through the bone marrow registry, so we did a half match with his dad, and the closer we got to the 90 days, he lost the graft, so his bone marrow was unsuccessful. But I've heard stories of -- success stories, and I'm just hoping and praying that we'll find a cure soon.

DR. EGGERS: We had one more over here, and then we'll go over there to Margaret.

MS. WOOLFORD: Hi. My name is Teonna. So I actually had a bone marrow transplant 2 years ago. Just like what she was saying, I could not find a match, so I did a clinical trial at Hopkins, and my mother was my donor. About 90 days into it -- I mean, they were doing testing like weekly, and my numbers weren't looking good. I definitely had more of my cells than my mom's cells, but, you know, we were still praying, we were still faithful, we were hopeful, and then I just got really sick, I started having really high fevers, and basically what

happened, my bone marrow, instead of kicking and revving back up like it was supposed, it shut down for 140 days. So I wasn't making platelets, I wasn't making any kind of cells. I was rejecting what they were putting into me, and I had to get bone marrow aspirations like every 3 days, and the slides, it was like nothing to look at.

But at the same time, I do know of people who have had the transplant and they are doing really, really well. So I think it's just very personal for everybody. And, I mean, my story, everybody's story, is really unique, but I am also very hopeful that within a year or 5 years -- that's what I've been told -- that bone marrow transplants will be a little bit more successful, so that's what I'm hoping for.

DR. EGGERS: Thank you very much.

We had one over here? Margaret?

MS. HADNOTT: I was listening to Adam, it is? Adam, you said you had a transplant? A kidney transplant.

MR. BUNDUKARMA: No, no, I didn't have a transplant. I had joint replacements.

MS. HADNOTT: Oh, okay. I had a kidney transplant because over the years my doctors told me that my kidneys were wearing down, and between that and high blood pressure, between sickle cell and my high blood pressure, my kidneys went down. So even though I'm going to be 70 years old this year, I have quite a few replacement parts.

(Laughter.)

MS. HADNOTT: I have a new knee, I have a new hip, and my daughter gave me a kidney. So I'm still here.

(Applause.)

MS. HADNOTT: And I want you to know the only medicine I take every day is folic acid. But over the years I've had my share of crises. I don't anymore. But one of the things I really would like to stress is to get rid of stress. "No" is a complete answer.

DR. EGGERS: Thank you very much.

MS. HADNOTT: And the other thing is your nutrition, and that will help save your kidneys because over the years it does happen to sickle cell

patients.

Thank you.

DR. EGGERS: Thank you very much.

Ann, you had a question?

MS. PARISER: Yes. I just had a question about clinical trials in general. Can people share an experience perhaps they've had participating in clinical trials or if you decided against it or if you would like more information on research, what are some good ways to communicate these across the community?

DR. EGGERS: I'm actually going to hold on Ann's question because we're going to be coming up with that question in a few minutes, so when we're done, if we haven't answered all your questions, then we'll come back to it.

I want to see on the web if there are any web comments on the treatments that we've talked about.

MR. VALENTINE: All right. So we've had a lot of comments coming in through the web. I'll try to summarize these as best I can.

We have had some people say they've had

success with a bone marrow transplant. Another specific experience that was shared were that a patient with SCD said that pain meds were less effective for them. Some of the broad alternative treatments included broadly alternative medicine, faith and prayer, and nutrition, and more specifically mentioned were sunlight through optic nerves has increased endorphins to fight pain and fatigue, Vitamin D supplementation, using prenatal vitamins to avoid that issue of iron, B-12 for energy, pears and apples for energy as part of nutrition. For supplementation, dandelion, L-arginine, and L-methionine for slowing the sickling cycle.

There were a couple of comments about being mistreated using detox or other -- being treated as having drug-seeking behavior when coming to the hospital, so there are some concerns about that.

And as for things that people would like to see, they said treatments that would create new gene activity to produce blood that does not sickle. Also a unique comment was they would like to see a home blood test like for diabetes.

DR. EGGERS: Okay. All right. Thank you.

I'm going to go to our next polling question because it gets at the other types of therapies that people have been talking about. We've heard a lot about that today and we heard that on the web. So let's just get a poll to see a sense in the room.

In the past year, have your or your loved ones done anything besides those prescription medicines to treat your sickle cell disease? And you can check all that apply. Over-the-counter pain meds; massage or acupuncture; vitamins or dietary supplements; D, take extra fluids; E, followed a special diet such as avoiding certain foods; F, attended some pain program or support group; G, use some other therapy; H, if you don't use any of these therapies; or, I, if you're not sure.

This may take a while for some people, I think, who have maybe several of these.

(Answering question.)

DR. EGGERS: Okay. If everyone is about done, we'll go on to the results. Very high numbers here. So it looks like 83 percent take over-the-

counter pain meds. Now, can I get a show of hands how many -- let's just see a show of hands, how many take a prescription pain medicine every day or almost every day regularly?

(Show of hands.)

DR. EGGERS: Okay. And how many take an over-the-counter pain medication daily or almost every day?

(Show of hands.)

DR. EGGERS: Okay. Thanks.

And then, let's see, what's D? Extra fluids. Okay.

Vitamins, dietary supplements, and herbal remedies. I think we heard a lot from the panelists and on the web about that.

Gosh, I have a hard time reading these letters. E, what's E? I've got to get right up close.

Okay. So are there any of these other therapies? I know we've heard a lot from the panel members. My colleagues?

(No audible response.)

DR. EGGERS: Okay. All right.

I have a question for a show of hands in that how many of you would say that overall these non-drug therapies or these non-prescription therapies are as important to your overall treatment regimen as any prescription medicines or therapies that you do?

(Show of hands.)

DR. EGGERS: Okay. Thank you.

Yeah, sure. If you think about your non-drug therapies, such as the ones listed, would you say that those are as important to you in your overall treatment as the prescription medicines that you take or the therapies?

Tina, yes.

MS. HUGHES: One other therapy that hasn't been talked about is art therapy. And a lot of organizations get a lot of money for art therapy because it releases endorphins that makes you feel good. It's a feel-good chemical that helps you forget about the pain for just a little while. And art therapy can consist of dance, poetry, music, anything that comes under the auspices of the arts.

DR. EGGERS: Thank you, Tina.

Okay. We'll take two more comments on these non-prescription therapies, and then we'll move over, I have another general question.

Right there?

MS. VALENTINE: Another form of therapy we discovered wasn't offered to the sickle cell population was hippotherapy. That's the use of equines to warm your muscles prior to kinesio or physical therapy and also equine-assisted or forms of animal therapy. It's routinely offered to patients of cerebral palsy, stroke. We use comfort dogs for veterans returning from war for PTSD. That has not been offered to the sickle cell community, and it is a form of therapy you can seek out in your neighborhoods you need to find. If you even Google it, "animal-assisted therapy" or "equine therapy," it will also help with mobility and it also helps with low impact stretching and motion especially if you have acetabulum AVN.

DR. EGGERS: Okay. Thank you.

Okay, one more? And I'm going to let

Soujanya pick the person.

DR. HSU: I'm Dr. Hsu from the University of Illinois. I'm not a patient, but you have to mention heat and warmth. Everybody is using that, and that's not included. And sometimes there are hospital restrictions about what kind of temperature you have, and it puts a crimp on what kinds of non-drug therapies you can have.

DR. EGGERS: Thank you.

Okay. So I'm going to -- we have time -- by the way, would anyone mind, if the meeting, since we started a little bit late after lunch, if we go till about 4:15 to end? Of course, you can leave early when you want, but we're going to plan to go until about 4:15 if that's okay.

I have a general question for a show of hands, and that is, when you think about all of your therapies together, how many of you would say that you are able to keep your sickle cell disease and its effects in decent control? How many of you feel like your sickle cell disease is being pretty well managed right now?

(Show of hands.)

DR. EGGERS: Okay. And how many of you would say that it is very much not being well managed?

(Show of hands.)

DR. EGGERS: Okay. Let's take for those who are not being well managed, those that just raised your hand, if you can give me in a few words what your ideal treatment would address better, what would it be?

UNIDENTIFIED FEMALE SPEAKER: Hi. My ideal treatment is that sometimes I don't need to go to the emergency room. And I notice that for a lot of other diseases, there are some form of home therapy. I think it's imperative that people with sickle cell anemia, especially people with ports for IV saline solution, IV saline therapy, that we could do that at home, and oxygen therapy, things like that can be done at home, and when I asked my doctor about these types of treatments, I was always met with opposition because the fear from the medical community was that having something where you can have a port and have saline therapy that abuse, that people would put

things into their ports at home that they were not supposed to have, but I think that with the proper education and the proper training of patients so we could handle a lot of things at home and kind of eliminate some of those ER visits, that's what I would like to see.

DR. EGGERS: Thank you. Thank you.

Someone else.

MS. OLA: I just wanted to say for me the therapy that's not being addressed is the tissue damage. I understand the pain, I can deal with the pain on a variety of levels, but what I feel is being most effective to my future and I'm most concerned about is the fact that my organs are dying, my tissues are dying, every time I'm having a sickle cell episode, whether I'm in the hospital or I'm at home. And when you go to the hospital, the only thing that they're giving you generally is pain medicine, but nothing addresses the tissue damage that's occurring with every single crisis.

And for people that die from sickle cell, it's never on the death certificate that they died

from sickle cell pain, it's always they died from complications of sickle cell, and these complications are coming because of the tissue damage that's occurring, and really that's what I want treatments to address.

DR. EGGERS: Great. Thank you.

(Applause.)

DR. EGGERS: Two more, if there are two more.

MR. MOORE: Well, for my son, the reason we've decided to do the bone marrow transplant is because he's pretty healthy right now. He hasn't had to have any of his organs removed or replaced or treated. So we kind of want to take this opportunity while he's pretty healthy. He had a good response to hydroxyurea for a while, but after a while it didn't do anything.

So we don't feel -- it's like being caught between a rock and a hard place, you really don't have options. You are kind of damned if you do, damned if you don't. So we're hoping that we can preserve what little general good health he has now and benefit from

it. We don't want to wait until he's had a stroke or he's had to have his spleen removed. So we're trying to hold onto the good health while he's still young and make that move and just pray that it works.

DR. EGGERS: Thank you, John.

I'm going to tee up on the phone. We can take one or two callers on the phone. I would ask you just to keep to the specific topic of, what would you look for in an ideal treatment? What would it address in your health? And we'll do that in a few minutes.

One other comment, one other person who wants to talk about the ideal health -- ideal treatment, I'm sorry.

MS. BROWN-WATTS: I would like to see an ideal treatment that addresses the whole, the whole person. We have a lot that is compartmentalized, and we miss a lot of other psychosocial, mental health, things of that nature, that directly affects the body. Your mind directly affects your body. And I would like to see some systematic comprehensive strategies to address the whole person.

DR. EGGERS: Thank you very much.

(Applause.)

DR. EGGERS: On the web, do we have any other new comments in on ideal treatments?

(No audible response.)

DR. EGGERS: Okay. Then I think I -- oh, let me just go to the phone first. So I think I've learned my lesson.

Operator, are there any calls?

(Laughter.)

OPERATOR: Yes. We have a couple.

Cassandra, your line is open.

CASSANDRA: Hello?

DR. EGGERS: Hi, Cassandra.

CASSANDRA: Hi. Can you hear me? Sorry.

DR. EGGERS: We can. We can. What are you looking for in an ideal treatment?

CASSANDRA: I would like to see more going towards like holistic treatment. You know, a lot of foods can cure a lot of ailments, celery helps with inflammation. You know, just something that isn't really going to poison our bodies and have so many side effects that we're suffering from sickle cell, we

don't want to suffer from like any other side effects other than what we've been dealt with.

So I just would really like to see like maybe a turn towards more natural holistic, like Yoga, not necessarily just foods, just taking care of your body in a natural way.

DR. EGGERS: Thank you very much, Cassandra.

One more on the phone, Operator, please.

OPERATOR: Jonathan, your line is open.

JONATHAN: Hi. I have a very interesting question. It's not one that gets brought up a lot. As I was growing up, my parents did their best to teach me the basics, what I need to know to take care of myself, but I lost them early on in my teen life and grew up pretty much by myself, and there were no groups, there were nothing in the hospital to help me out. And I just wanted to see, what can you do to -- if they're ever in that situation when they're alone and they don't have anywhere to turn to? And myself, I've ran into a lot of uncaring arms and a lot of opposition because I looked like I was normal most of the time, and people were just never around when I was

ill.

DR. EGGERS: That is a great question. I'm not sure if we're going to be able to -- I think that question would take hours to answer. So here is what I'm going to suggest -- oh, we have an answer. Sorry.

MS. LaMAR: Hi. I'm Mary Bentley LaMar with the Sickle Cell Association of New Jersey. We're part of the Sickle Cell Disease Association of America national network of community-based organizations. I would encourage that individual and anyone dealing with sickle cell disease to connect with an organization like ourselves or like or similar organization in their area so that they can connect with other individuals who are dealing with it and help raise awareness so that more people know about it so it's not a foreign concept when they encounter someone who is dealing with sickle cell disease.

DR. EGGERS: That is a great point.

Yeah, go ahead.

MS. MURPHY: I'm from New York, and I was diagnosed in 1956. What I am surprised about, in the Bronx, the biggest hospital is Montefiore; in

Manhattan, it's Columbia Presbyterian; in Brooklyn, it's Interfaith. Each has a sickle cell department. I have spoken to each of these doctors that works with sickle cell. They don't know each other. That's a major problem. How can you be getting funds or lack of funds and not know if I say "David Diuguid," not know his name, or whosever at Montefiore or whosever at Interfaith? That is a serious problem. And why aren't they having groups themselves? As other, as I wait, because I have to go for Aranis (ph) every 3 weeks, the oncologists, those people, they have organizations in the same hospital, and they'll speak to them, and I say, "Well, how come they ain't coming over to me?"

DR. EGGERS: So this is a very --

MS. GRAY JOHNSON: I would just like to add to what they have said. Listen, every hospital should have a patient representative, and if they don't, then you can inquire why or whatever, but that, number one. And if so, make sure that you maintain a relationship with that person and report everything to that patient representative. Now, if for some reason or another

that is not -- that they don't have it or you're not satisfied or whatever, get familiar with the Joint Commission, it's out of Chicago, Illinois, and they will listen to whatever problems that you're having. We're hearing too much of this, but there is -- but we all have to be vigilant, and we just can't sit back and complain, we have to do something.

DR. EGGERS: Thank you, Judy.

MS. GRAY JOHNSON: Thank you.

DR. EGGERS: This is a very important topic, and we're going to have to move on from the caller's important question. But I do encourage you, we have a docket, and so use that if you have a point that you would like to make on this topic. Please send it to our docket.

Okay. I forget your name. Fran. You've raised your hand a few times, so we'll go to you, and then we're going to have to move on, we want to get to an important discussion on clinical trials.

MS. VALENTINE: There is something called Home Telehealth. It is more and more we're using technology to our advantage. We currently monitor

heart failure, COPD, hypertension, diabetes. Registered nurses where they're case managers, they monitor their vital signs and answer health questions every day. They red alert, I call them. I watch their weight. They gain 1 to 3 pounds? We're contacting the doctors. I think sickle cell patients could benefit from that. They can also do home IV therapy we do. Our hematologist, he hooks to a CAD pump and goes wherever he has to go if he can't hydrate by mouth adequately. So I think it's a physician preference.

But speaking to the barrier, of breaking down barriers, everyone with your young children, start your to-do list. Make them articulate, outspoken. People that meet you will not know anything. I will not even be able to tell you over the years, the 35 years, being a nurse how many times I've given an on-spot teachable moment for sickle cell. Do that. Start them young because we have to advocate also not be fragmented, yes. But Telehealth, you can Google it, but it may be something that could be beneficial.

DR. EGGERS: Thank you very much.

Patient Perspectives on Participating in a Clinical Trial to Study Experimental Treatments

DR. EGGERS: Okay. We're going to move on to a discussion on clinical trials because this is an important topic that my colleagues would really like to know a little bit more about.

We're going to read a scenario of a clinical trial, but before doing that, we have one polling question to ask, which is: Have you or your loved one ever participated in a clinical trial studying experimental treatments for sickle cell disease? Yes, A; B, no; or, C, I'm not sure.

(Answering question.)

DR. EGGERS: Okay, and we'll go to the results. Oh, half and half. Okay. Is that -- okay.

All right. So we have lots of experience in the room as we go on to the next few slides.

So for a few minutes I would like you to imagine that you have been invited to participate in a clinical trial to study an experimental treatment for sickle cell disease. The early research in animals

and people shows that this treatment may decrease the number of pain crises or hospitalizations in some people with sickle cell disease. Now, the purpose of the study is to better understand how well this treatment works and its safety. The study will enroll 1,000 participants with sickle cell disease. Also imagine that the clinical trial lasts 1 year and involves four clinic visits each occurring every 3 months.

More common side effects of this therapy may include nausea, diarrhea, fatigue, headache, and rash, and rare but more serious side effects may include infection, bleeding, and life-threatening allergic reaction.

So the question I have is, what comes to mind as you hear this scenario?

Raise your hand if you would like to speak and we'll try to take -- someone who hasn't spoken much. Okay, in the back there?

MS. ROBINSON: Hi. My name is Mattie. I'm with the William E. Proudford Sickle Cell Fund, a community organization, but I have a very intimate

relationship with sickle cell disease. I was born with sickle cell SS and I also have persistent fetal hemoglobin, and I chose a career until recently in biomedical research specifically doing research on treatments for sickle cell.

So the thing that jumped out at me in that hypothetical scenario is the phrase, "This drug is expected to help some people." If I were vetting this therapy and trying to decide whether or not I wanted to subject myself to this experimental medication, I would want to know exactly how some people were determined, what exactly you used to determine who this helps, because I think in the end you need clear guidelines of, who are the people that are going to be helped by the therapy? And we've seen here that there is a lot of diversity in the symptoms that people have and their experience with sickle cell disease. So if it's a drug that helps people who have ulcers, I might not be the best person for that study. So I think very detailed and clear information is necessary.

DR. EGGERS: Okay. Thank you. Anyone else?

DR. BAILEY: Sara, may I?

DR. EGGERS: Yes.

DR. BAILEY: That is exactly the type of questions that I would want to know. How is this going to help sickle cell? What's its method of action? Is it something that I would benefit from? Is it something that I need that outweighs the risks of it? For the one or two times that I have been involved in drug trials, these are the questions that are the most important to me. I need to know not just this is going to help sickle cell but this is going to help treat the persistent acute chest syndrome that you're dealing with by approaching this particular target area.

Those are the kind of questions because when you're talking about research studies that don't involve inhalation or injection or infusion of a medicine, then I'll participate in that as much as I can, but when it comes to actually taking a drug into myself, I would need to know more about how that drug is going to work and why you think that's something that would work for me.

DR. EGGERS: I heard a lot of positive

reactions to that.

Anyone else? Over here?

MR. SWEET: Hi. Oh, excuse me. Hi. My name is Jay. And this exact scenario reminds me of when I went to Duke University with their sickle cell clinic. I absolutely hated it. I've learned, thanks to them, just because it says it's a clinical study and it's supposed to help sickle cell does not mean it's going to help sickle cell, it does not mean it's going to help you. It's going to help them first and then it's going to help you, if it helps you, because when I went to Duke University -- and I moved to North Carolina because I thought -- I heard so much great things about Duke helping sickle cell and having studies and some of the best doctors from all over the world were going there. I never met more disrespectful doctors in my life and --

DR. EGGERS: I think we don't want to focus on any particular people or organizations in here, so --

JAY: I get that. Long story short, because of the experience that I've had, I would be more

questioning about what is the final result you're looking for, and is it going to help me out in the long run? Like she said, the drugs going to your system, I don't want to be a lab rat to know, I just want, is it going to help me more versus kill me?

DR. EGGERS: Okay. Thank you. Thank you.

We have a few polling questions that are going to tease out a little bit of your thoughts, and we can build upon that for a bit to give you kind of all a chance to weigh in here.

So there are many, many factors that go into your decision about whether to participate in a clinical trial, and we have pulled out just a few of those factors that are sort of the most relevant to FDA and what we think about as we think about clinical trials.

So of the following factors, which two would you rank as most important to your decision about whether to participate in a clinical trial to study an experimental treatment? Is it the common side effects? Is it the rare but serious side effects? Is it considerations about how the treatment might

improve your particular health? How the trial might affect your current treatment plan, what you're currently doing to treat your sickle cell? Any requirements of the trial, such as whether you have to go for regular blood tests or go to the hospital? Or length of the trial? So of those factors, if we said please pick two that would be most important to you, which two would those be?

(Answering question.)

DR. EGGERS: Okay. So the most common on the in-person, in here, the most common response was the common side effects -- no, I'm sorry, the rare but serious side effects. I've got to read the bigger words here. The rare but serious side effects, followed by how the treatment might improve my health, and then kind of equally split between common side effects and the treatment plan followed lastly by the requirements of the trial.

And on the web, do we have something similar?

MS. VAIDYA: So on the web, we have 90 percent who say rare but serious side effects, and

then the next is 51 percent, how the treatment might improve my health, and then goes on to how the trial might affect my current treatment plan, at 32 percent.

DR. EGGERS: And I think we've heard from the panelists and from the comments, we've heard about each of these factors. I'm going to turn to my colleagues and see any factors that you want to follow up on why someone picked one of these things.

Anne, did you have any other questions about the clinical trial participation?

DR. PARISER: Yes. I would like to follow up on there is a lot of research going on. What are some of the best ways to communicate information? How do you get your information? What are some of the best sources? Where would you like to hear this from? And where would you like to get more information?

MS. OLA: Hi, again. I'm Tosin Ola from Sickle Cell Warriors, and this was one of the questions that we asked our community, and most of the people that participate in clinical trials or would be interested in participating in clinical trials, the main gripe that we get is that they don't hear about,

and one of the ways that most people stated in that question -- we got 93 responses -- 30 percent said that they wanted to see commercials on television, PSAs, just like we have for other diseases and other conditions, on the radio and where they would get their information anyway, have news reports on the news, things like that.

The other group said that they would like more online information accessible to them. And a couple of patients mentioned that they had gone to clinicaltrials.gov and it was really hard to navigate the site and really hard to figure out what trials they were eligible for and what hospitals were doing the trials and things like that.

And then the third resource was just community-based organizations that they were affiliated with and that if their physician or their hematologist told them about it, they would like to hear mostly from there. And most of the things that we hear is that their doctors are their trusted source, but some reason their doctors don't even know about the clinical trials, so when they hear about it

and they ask their doctors about it, the physicians don't know about it, and this is just their hematologist and their regular doctors.

DR. EGGERS: Okay. Does that resonate with folks here, that that's how you would like to know about these things?

DR. BULL: Sara, I have a quick question. I was just wondering of I guess the previous poll that found that 49 percent had participated in a trial, how did that come on your radar if it's not being communicated by the health care providers? How did you find out? How did participation come about?

MS. NELSON: The same way I found out about this meeting, a friend happened to tell me about it, I happened to hear about a clinical trial. My physicians don't even know about this meeting, and they're at the University of Michigan.

MS. MURPHY: I heard it on a radio station that's tended to gear itself to the black community, but I only heard it that one time.

DR. EGGERS: Back there. Right behind, one row behind.

MS. ROCHESTER: Thank you. My name is Gloria Rochester. I'm from New York. I hold two hats. I'm a parent of a 40-year-old daughter who was diagnosed with sickle cell. I'm also a part of the national SCDAA organization. We are a community-based organization. And rearing my daughter, I did not hear a lot of the information that I get in the hospital; I get it on the outside. My daughter was born in the early '70s, and I had to go outside and learn how to cope with it and take care of my daughter.

Most of the things that are going on, I think the community base serve a very vital role in the community. Of the people right in our backyard, we do the assessment and the survey. So things like this coming through, we have the people right in front of us. It's not only by one hospital, but we will deal with hospital right around the different boroughs. So I think things like this would be really important to go through the community board organization.

Thank you again.

DR. BULL: One follow-up question. One of

the things that we've witnessed in recent years has been galvanizing, gathering of, patients around social media, sharing stories on Facebook sites, developing their own independent lists. So I'm just wondering, does that exist in the advocacy community for sickle cell?

(Chorus of yeses.)

DR. BAILEY: Yes. That is actually one of the best ways to access the patient population. And just in my personal experience when I was recruiting for studies, I just needed a tube of blood, doing it through the clinics, you get a few patients, but patient-to-patient networking through the community-based organization, for our community specifically, it's unlike any other disease I've ever worked with, most people, they go to clinicaltrials.gov, they go to the doctors, but in this disease specifically, we go to each other, and it's -- Tina Kay's radio show she mentioned, it's Sickle Cell Warriors, Sickle Strong, supporters, and it's these groups that the information gets out. Even for this meeting, a majority of us are here because we found out about it through pushing it

through social media. That is one of the -- in my opinion, the most effective way to reach this particular patient population, is through these organizations.

DR. PESANTE: I want to echo what Dr. Bailey said first, that's absolutely correct. I'm also a family physician, and I didn't hear about this through being a family physician. It wasn't in the *American Academy of Family Physician* magazine, it wasn't in the *New England Journal of Medicine*, there was nothing coming to me as a physician to tell me about this. I found out through *What'z Da Count Radio*, *LA Talk Live*, through Dr. Bailey actually.

I think one of the issues that we're not really addressing in this whole, "How do you get the word out and how do we communicate?" the young man over there began to try to articulate an issue that's kind of an elephant in the room that we've touched on, which is the trust and the communication between our community and the medical community.

(Applause.)

DR. PESANTE: If we can change those

dynamics such that our community receives the same respect as the mainstream community in how we're dealt with by the medical community, that would greatly improve another way of getting things out. So, yes, we could use social media, but we would also trust the physicians to guide us on which trials were good and which ones weren't and it would be a completely different dynamic.

There has to be more respect and more trust between our community and the medical community. I think a lot of that comes from the medical community, and it starts in medical school, the way the doctors are trained. I saw so many episodes of differential treatment with the exact same symptom profile that was different based on the color of skin, and so if we change how we train our medical practitioners and include more cultural sensitivity at that level and more education about sickle cell -- everything I learned about sickle cell I had to study on my own, which is crazy, because I went to medical school. So I think that that's another aspect that we need to address to fix this problem.

DR. EGGERS: Thank you very much.

(Applause.)

MS. HUGHES: Also, when we do hear about the clinical trials, we aren't given a whole lot of information. It's just a clinical trial, it's a flyer, but there is not a lot of information. So why would I want to participate in something where you're not giving me enough information to make an educated --

UNIDENTIFIED FEMALE SPEAKER: Decision.

MS. HUGHES: -- decision.

DR. EGGERS: Okay. Nicole has a question.

DR. VERDUN: So those that have participated in clinical trials, was that through a large academic center or did you -- after you found out about the trial, enrolled in the trial, was that through a large academic center or through some other avenue? And if you could speak to that, that would be helpful.

DR. EGGERS: Maybe a show of hands. So if you've participated in a large academic center, raise your hand.

DR. VERDUN: Specifically adults, sorry,

because I think the childhood community is a little bit different.

(Show of hands.)

DR. EGGERS: And then some other way.

(Show of hands.)

DR. EGGERS: Okay. So do you want -- yeah. Someone in the --

JONATHAN: Hello?

DR. EGGERS: Yes. Hi, Jonathan.

JONATHAN: Hi. I have to speak for my wife. She's not feeling well. But we've been going to a sickle cell comprehensive center for 8 years now, and we've participated in a few studies over those years, but only one of those times did her hematologist actually tell her about the study, and it was only in passing, if we happened to see the director, who we became familiar with by going to the center so often, that we found out about these studies. So they were never brought to our attention. And we've discovered after they're done, there was a study that we would have been interested in participating in that we never heard about. And I just think there is an extreme

lack of knowledge about what's even out there going on, and I think you could get a lot more participation if that was communicated properly.

DR. EGGERS: Okay. One other comment? Someone raised their hand? Someone who hasn't -- in the back I think. Sorry, we're trying to make sure that we get everyone's voice.

MS. ROBINSON: Hi. I know I spoke earlier in this same segment, but I just wanted to say that we live in these bodies. We have the information that is useful to learn more about sickle cell disease and to help gauge whether a treatment is effective or safe. So the most important resource here is not tapped effectively, and I think by changing that, we will progress in leaps and bounds.

DR. EGGERS: Yeah.

MS. BROWN-WATTS: I know you may think it's different for children. No. We're not getting the information either. And I live in Oklahoma and I ask the doctors about trials. They have no idea what trials are happening, what trials are going on, they can't tell me anything. So it's not different for

children.

DR. EGGERS: One more comment on this?

UNIDENTIFIED FEMALE SPEAKER: I participated in a couple of the trials, but I first learned about it through an organization in New York called SCAC, and my doctor -- because I didn't know much about sickle cell when I was diagnosed, I asked a lot of questions, and I am one who will not do something until I know the benefits or the pros and the cons of it, and she was gracious enough to explain it to me. She explained it in detail. She never flinched at any questions I asked, and it made me feel comfortable. And that's where the level of trust comes in because if you can't communicate with your doctor and ask questions, why are you going to participate in anything? So because of that trust factor that I developed in my doctor, I was willing to participate in that study.

DR. EGGERS: Thank you so much.

On the web, do we have any comments? I mean, we have a lot of comments, but maybe just a select few of those.

MR. VALENTINE: Right. So we did have some trends in what some of the clinical trial considerations for being involved were. Many echoed what were said in the room, but many people talked about how the short-term side effects would be manageable for a trial but there would be much more concern about the long-term side effects or potential side effects. Someone mentioned that they actually have trial burnout from being in many trials as a child and they don't know if they have the emotional strength to go back through and deal with unknown or unanticipated side effects as an adult.

As was talked about in the room, people want more information about the motive of those sponsoring trials. Someone mentioned actually they would want transparency in information on the actual animal trials, the pre-human trials. And then someone also mentioned it would depend on the distance of trial sites and if they would be able to keep their same community doctors while they were participating in a trial.

And then there was an overwhelming response

of people on the web saying that, yes, they find out about trials through either socially geared websites or social media.

DR. EGGERS: Okay. Thank you.

We have one more polling question I think to wrap up the discussion on the clinical trials, and that is, just after hearing our discussion and thinking about that scenario, we are definitely not holding you to this answer, it's just to get a sense of your thoughts, if your loved one had the chance, had the opportunity, to participate in a clinical trial to study an experimental treatment, one that looks kind of like that scenario that we presented, which of the following would best describe your thoughts? Yes, I would want to know more, but I'm generally willing to consider participating; no, I would probably not consider participating; or maybe, I'm just not sure whether I would be willing to consider participating or not. This is your last polling question and then you can put the clickers away. Oh, please don't take the clickers out of the room, though.

(Laughter.)

DR. EGGERS: They have to stay here.

(Answering question.)

DR. EGGERS: Okay. Can we see the results? Okay, so it looks like there is a general willingness to at least learn more and consider with the majority, with the highest response, at 46, saying yes, and very closely followed by maybe. And it sounds like you need a lot more information on the trial, you need a lot more communication to you, and I think you've made that point very clear.

On the web, do we have similar results?

MS. VAIDYA: Hello. We have very similar results on the web saying 48 percent say yes, about 38 percent say maybe for participating.

DR. EGGERS: Okay. Thank you.

I'm going to turn to my colleagues, and you have one last chance if you had any other final questions to ask.

(No audible response.)

DR. EGGERS: Okay. We have been at a discussion for several hours, and that is a lot of

work on your part, it's a lot of work on our panelists' part. I want to thank the panelists again for having that courage and taking the time to share their thoughts. And I want to thank everyone in the facilitated discussion. This is a hard thing to do. Thank you for your patience. Thank you for your dialogue and your building upon one another as you're speaking. I think we've really gotten what we need.

Kathy is going to close and provide some final comments after -- not yet, Kathy. We have a public comment period that Pujita is going to manage. And this gives people a chance, if you signed up for it, to talk about something else besides the topics.

So with that, my job here is done and I'm going to turn off the mike and just say thank you.

(Applause.)

Open Public Comment

MS. VAIDYA: Hello, everyone. I would like to thank you all for coming here today once again. And we are now going to move on to the Open Public Comment session. And for those of you who are not aware, the purpose of this session is to allow an

opportunity for those who have not had a chance to speak on issues that are not related to our two main discussion topics. This is also an opportunity for folks who are not patients or patient representatives to comment. Please keep in mind that we will not be responding to your comments, but they will be transcribed and will be part of the public record.

Since we would like this to be a transparent process, we encourage you to note any financial interests that you have that are related to your comment. If you do not have such interests, you may state that for the record, and if you prefer not to provide this information, you can still provide your comments today.

So we have collected sign-ups before the meeting and during the break. We have 12 people signed up and about 30 minutes for this session, so please be respectful for your other colleagues here and other patients and please try to stick to the about 2-1/2-minute limit. We won't have a timer, but I will have my phone here keeping track of time. So if you approach the 2-1/2 minutes, I will let you know

to start wrapping up.

So I will briefly run through the order of speakers, and I apologize if I mispronounce your name. We have Lakiea Bailey, George Carter, Johnnie Tidwell, Marybeth McAfee, Tosin Ola, Jay Sweet, Wani (ph) Thompson, Lewis Hsu, Kim Smith-Whitley, Ibrahim Kargbo, Tina Kay Hughes, and Dawn Nelson.

So could we have Lakiea Bailey first?

DR. BAILEY: Thank you. I actually just signed that and then you suggested -- well, first just to say that it isn't very often that there are so many of us patients from all over in one place, and so with that in mind, we have reserved the restaurant Friendly's for 6:00 p.m. tonight just for all the patients to come out, if you are interested, have dinner, we can all have dinner together, and just fellowship with each other. And Friendly's Restaurant is at 12046 Cherry Hill Road. It's about 4 miles from here, and that is at 6:00 tonight, just if you are at all interested. We'll be there, we'll have a section set up just for us just to spend time together. Thank you.

MS. VAIDYA: Thank you, Lakiea.

Next we have George Carter.

DR. EGGERS: While George is coming up, can I just ask Lakiea to say the address again?

DR. BAILEY: Of course. That's Friendly's Restaurant. It's 12046 Cherry Hill Road, Silver Spring, Maryland. It's about 4 miles from here. And actually you can get on friendlys.com to look at their prices and menu and things like that.

MS. VAIDYA: Thank you, George.

MR. CARTER: Thank you for this opportunity. A family of someone with sickle cell disease faces numerous circumstances that challenge their lives. Sometimes these challenges overwhelm them and put a strain on relationships. Psychosocial problems have overcome many clients and their families because of the physical and mental suffering that they have gone through.

In Dallas, September 27, 2006, the PR newswire said sickle cell disease is one of the most prevalent and costly genetic disorders in the United States today. One in every 4,000 Americans is born

with a form of SCD and many experience chronic anemia, stroke, spleen and kidney dysfunction, pain crisis, and susceptibility to bacterial infections. Moreover, the National Institute of Health, NIH, estimates that almost one-third of adults with SCD develop pulmonary hypertension, a life-threatening condition resulting in a tenfold greater risk of death.

Due to this high disease burden, the Sickle Cell Disease Association of America reports that sickle cell disease results in an estimated 750,000 hospitalizations a year. The cost of these hospitalizations was estimated then at $475 million. According to the National Institute of Health report in 2007, nationally the number of hospitalizations among adults with sickle cell disease was 83,149. In the *American Journal of Hematology* in June of 2009, a study reported that the annual cost of medical care in the United States for people who suffer from sickle cell disease exceeded $1.1 billion. The high proportion of sickle cell disease costs associated with inpatient hospitalizations suggest that the interventions that reduce complications such as pain

crisis could be cost effective and cost saving. Allow me to repeat two sentences for the research and medical community and drug community. The American Journal of Hematology, a study says those who suffer from sickle cell disease, the cost is $1.1 billion and that if there were more interventions, then this would be cost effective and cost saving.

So this is a good measure for you all to take to heart so that you can find what we need to put this disease behind us. Thank you.

MS. VAIDYA: Thank you, George.

(Applause.)

MS. VAIDYA: Next we have Johnnie Tidwell. So if you could just raise your hand.

MR. TIDWELL: Good evening. I just want to say -- oh, speak up.

(Laughter.)

MR. TIDWELL: I just want to say we, as people, those that have sickle cell, my mom always said a squeaky wheel gets the grease, and we are starting to squeak.

I would like to say thank you to the FDA for

this opportunity, but just keep on keeping on. You're going to leave here, you're going to die with something, if it's nothing but old age, something is going to take you out of here. So just keep on keeping on. Do what you have to do. Empower others. Help others. That's what we need to do, and I'm sorry I can't make this meeting at Friendly's because I'm on my way back to Newport News, but we need to stay in contact through whatever means and keep on keeping on. Keep on putting pressure on our congressmen, our senators, until we get the results that we need.

Sickle cell is here, we're alive, we're living, and we're living longer. I am 60 years old. They told me that I would never reach to see 15. Then they told me I would never reach to see 20. Then they told me I would never reach to see 35. I'm still here. By the help of the good Lord, I'm still here and I'm going to hold on and I'm going to hang on until He calls me home.

(Applause.)

MS. VAIDYA: Thank you, Johnnie.

Next we have Marybeth McAfee.

MS. McAFEE: That's a little bit hard to follow, but my name is Marybeth and I am the Associate Director of Health Information at Genetic Alliance. We are a nonprofit umbrella organization, and we're made up of about 10,000 health-related organizations including 1,200 support and advocacy groups.

We applaud the FDA and its Patient-Focused Drug Development initiative. We have followed the FDA's engagement thus far and are eager to offer the public tools to make it easier to share one's experience. Through an unrestricted educational grant from pharma, Genetic Alliance is using a new online tool to gather perspectives of individuals affected by sickle cell disease and some of the other conditions on the FDA list. Our goal is to give people who cannot come to the FDA or easily access the docket a way to be heard. We hope to collect a robust dataset of opinions and to offer a diverse range of perspectives to the FDA.

Several sickle cell organizations, including Citizens for Quality Sickle Cell Care, located in northern Connecticut, North Alabama Sickle Cell

Foundation, Sickle Cell Disease Association of America in Southern Connecticut, and the William E. Proudford Sickle Cell Fund in Baltimore and D.C. area have customized our new tool for collecting the experiences and opinions of people affected by sickle cell disease as well as their families and caregivers. The portal is called Platform for Engaging Everybody Responsibly, or PEER, and each organization has a survey portal on their website. Each individual taking the survey chooses meaningful sharing of privacy and data access preferences that reflect his or her needs and interests. Simply put, you control who sees the answers to your survey.

Over the next 2 months, these organizations, as well as we've recently been joined by Sickle Cell Warriors, and our Genetic Alliance portal will be collecting information. We will put all of the survey answers that we have permission to share in the FDA docket.

It is a privilege to work with the various sickle cell organizations involved in this initiative. We have heard the voices of you who have shared your

stories here today, and we will work as hard as we can to use our social network platform to help you and your loved ones be heard. It is our greatest desire to let all of your voices be heard loud and clear. We stand with you.

MS. VAIDYA: Thank you, Marybeth.

(Applause.)

MS. VAIDYA: Next we have Tosin Ola, if you could raise your hand.

MS. OLA: Hi, everyone. I just want to really thank everyone that came out today, especially I refer to sickle cell patients as sickle cell warriors because every day that you are able to get out of bed, you are winning the battle that day. And I know that many of you can completely resonate with a lot of the experiences that have been shared today in one way or another.

And I just want to encourage everyone to stay active in advocacy. More people need to speak out and more people need to talk about how sickle cell affects them. I believe that part of the reason why sickle cell has not received the treatment modalities

that it can compared to other conditions is because this is a disease that many people were taught to be ashamed of, and this is a unique opportunity for us to lift that veil. You no longer have to be ashamed of having sickle cell disease. It's okay to tell people that you have sickle cell disease.

Sickle Cell Warriors is dedicated to promoting advocacy within the sickle cell community, and we are offering several advocacy classes throughout the year for people who want to sign up for advocacy. In addition, the community did resonate that they were very interested in clinical research, and they expressed that they wanted more opportunities made aware to them. So we would like to serve as a liaison. If there is any clinical research and you guys are looking for participants or you want people, you can come to us, and we can disseminate the information to the community. We're definitely opening ourselves up to that.

The third thing that I wanted to mention is that there is a lot that goes on to sickle cell that is not just about the drugs, and I know that FDA is

focused about the drugs and on clinical research, but there are so many psychosocial issues that we deal with on a regular and daily basis. You're talking about work, you're talking about school, you're talking about the issues with your family, and maintaining social relationships. All these issues need to be addressed obviously in a different forum, but, please, if you do have the ability and the opportunity to do so, please consider sickle cell because it is a disease that affects more than 100,000 people in the United States, and it's definitely -- that number is astronomically growing because there is not enough education on sickle cell trait awareness, which needs to be a major medical standpoint that has not really been addressed.

So thank you very much to the FDA for inviting us and allowing us to participate, and we really do appreciate being given the opportunity to speak out about sickle cell disease. And once again, I'm just reiterating, keep talking about sickle cell because things are happening and it's because of every single one of you in this room, and we are grateful.

Sickle cell disease affects single tribe, every single nation, every single person under the sun, and it's not just a black disease, this is a human disease, and we are all affected in one way or another. So we all need your help to eradicate sickle cell disease forever. Thank you.

MS. VAIDYA: Thank you, Tosin.

(Applause.)

MS. VAIDYA: Next we have Jay Sweet.

MR. SWEET: I just have something that I wanted to say about how I've been treated and how I've seen sickle cell over my short stay on this planet.

Can you hear me now?

(Chorus of yeses.)

MR. SWEET: Good. Sorry. Meds, it messed up my voice.

I've done the McDonald's March of Dimes, I have participated in school fundraisers for cancer awareness, I've given change to the AIDS stands in front of Walmart or the Salvation Army. I even see billboards about donating cars to the blind. I'm done. I'm tired of seeing every other chronic

illness, STD, or whatever get all kinds of funds and publicity while sickle cell shares none of the limelight. I'm now waiting, wanting, and working towards a sickle cell, maybe a walk, a drive, march, fundraiser, something, something more than a website and a meeting that we have once in a blue moon.

A couple years ago I was going to the store and I saw one of those cancer stands out in front, and the guy asked me to donate, and I said, "No." The person behind me had the audacity to tell me I'm heartless and cheap. "You can't donate to cancer? That's just wrong. You have no heart," and went on about how I'm the problem. And I looked at him and I just said, "Do you know what sickle cell is?" and he was like, "No." That's the problem.

There is not enough support for the sickle cell community. Sure, there are websites, there are Facebook pages, there are support groups, the SCANJ, the SCDAA, that's all well and good, but that's not enough. We need to have a march of sickle cell anemia. We need to stand outside of Walmart with buckets and collect change. We need to have

billboards, awareness in schools, not just for the students but for the teachers as well. We need to do this, not just some rich person who has a girlfriend or boyfriend who has sickle cell, not the doctors who are looking for another trial study, we, my sickle cell brothers and sisters, we need to do this.

MS. VAIDYA: Excuse me, Jay. I would like you to wrap up and possibly just mention your final words.

MR. SWEET: The treatment for sickle cell patients in hospitals is downright disrespectful. I'm not a drug addict, I'm not faking it, I'm not looking for my next fix. I'm looking for help so I can find -- so I can get in and out. Just because I know what exactly I need in the ER does not mean I'm an addict, it means I'm trying to get in and out.

Doctors, you need to stop asking me in the ER what normally happens when I get into a crisis. If you haven't read my medical records and you have no intentions of giving me what I normally get, then don't ask me.

MS. VAIDYA: Thank you, Jay.

MR. SWEET: Just because -- and last thing. Just because I'm calm and collected does not mean that I'm faking it, it means I've had great practice. I'm in pain a lot. Just because I don't show it to you and crying does not mean I'm not in pain. And we need the doctors, the nurses, to understand that we're not faking it. The ones who are faking it, kick those to the curb, but don't penalize the majority of us because of what the few do.

(Applause.)

MS. VAIDYA: Thank you, Jay. Thank you for your comments. And you are welcome to submit to the docket as well.

Next we have Wani Thompson.

MR. DOZIER: Donnie (ph) has given me her microphone. She says everything has been covered.

My name is Farron Dozier. I'm a retired Sergeant First Class, United States Army, and I have the sickle cell trait. I have an organization called *What'z Da Count* on sickle cell trait prevention and I host the radio show WDC Radio that I've created, that God blessed me with, to advocate for sickle cell trait

and disease. You can't talk about one without the other.

Just a few key points that I want to mention. Language is so important, the way we describe it, the way we say it, the way it feels to us. Everybody's individual expression and language is different. Sickle cell disease is an individual disease. Everybody is affected differently, different pain issues, different crises, different triggers. It's individual. I don't know how you're going to get a medication to take care of an individual person's disease when it's -- I don't understand that.

You talk about prevention. I'm a prevention advocate. You can prevent sickle cell disease. 100,000 are born with sickle cell, we know it's probably more than that, but 3 million or more have the sickle cell trait. So why are you not talking to us with the trait who can have a choice when it's time to have a child versus not knowing after the fact?

(Applause.)

MR. DOZIER: We all know in other diseases that diet is important. Sickle cell disease, lupus,

cancer, those are all triggers from our diets. If you look at your ancestry in sickle cell disease, where it derived from, we probably should be eating to our ancestors, where we came from. You know, some of the things, Mexican food, we probably don't need to be eating.

(Laughter.)

MR. DOZIER: Natural foods, electrolytes, alkaline. There are some natural studies out there, oxyhemo plus ImmunoBoost and sea moss. I have an advocate who is actually on this study right now, that just started it.

Language again for me is important. You say sickle cell is a disease, but it fights malaria, so how can a disease fight something like a virus? I don't understand that one.

Donation. We have to talk about blood drives and bone marrow drives. That's so important, people. Our nationality, we do not donate blood. We need our ancestry bloodlines to donate to each other.

Preventive measures for sickle cell disease --

MS. VAIDYA: Excuse me. Sorry.

MR. DOZIER: Is that it? Okay.

MS. VAIDYA: I need to ask you to please wrap.

MR. DOZIER: Okay. And the last thing I just want to say is just again knowing that sickle cell trait is in your genes, you want to talk to those people who carry the trait, thalassemia trait, hemoglobin C, hemoglobin D, all those when come in contact with sickle cell disease -- sickle cell trait is a form of sickle cell disease.

MS. VAIDYA: Thank you.

(Applause.)

MS. VAIDYA: Next we have Lewis Hsu.

DR. HSU: My name is Lewis Hsu. I am here on behalf of the Sickle Cell Disease Association of Illinois and the support group of the University of Illinois Sickle Cell Center. And my financial disclosure is that my whole job is about sickle cell, and so if we get rid of sickle cell, I'll be out of a job, I'll be very happy.

(Laughter.)

DR. HSU: Just a few sound bites. One is from Ms. Beverly Chickwidozi (ph) in Chicago, is that what sickle cell has meant for her a lot is absences, absences from work, absences from school. She had pain episodes the day that she was taking her GRE exam. She had pain on her wedding night. And how much this was impacting her was that even though she is trying to be a dependable worker, she finds it hard to do that because of her sickle cell disease, and this is impacting her quality of life as well as her work, but she soldiers on nevertheless.

From Ms. Gloria Gilliam (ph), who is in her mid-seventies in the University of Illinois in Chicago, sickle cell has impacted her mobility. So this is a very lively lady who was a beauty queen, but is now in a wheelchair. She has AVN of the hips, of the shoulders. She needs to have a personal health aide to help her with even such things as grooming herself and reaching up for things, but she still has such joy in life that she wants to sign up for wheelchair dance classes so that she can get out to meet more people.

She also passed on the message that she is somebody who participates in clinical trials and is such a sophisticated clinical trial participant that she would list out seven or eight questions that she would ask people about, "What is this trial about? What is going to be involved? What is going to be asked of me? How many days, weeks, or months will I be committing this? And, lastly, how will I learn the results of the experiment?" So she was very interested in that. And she will be in the support group often and say, "I helped make hydroxyurea possible because I was in those clinical trials, and if there is another clinical trial, let me know about it, I can sign up."

I have other patients who say that, "I'm on disability, I can't work, and therefore participating in clinical trials is my way of giving to society and also channeling my frustration with my bad medical care or the lack of treatments."

And then, finally, the two elements of the quality of life measurements that have been mentioned, one is that fatigue does affect many, many people, and

sometimes it's fatigue when you are having exertion, sometimes it's not able to walk 5 blocks or to climb the stairs or gym.

MS. VAIDYA: Excuse me.

DR. HSU: Yes. Wrapping up.

MS. VAIDYA: Could you please wrap up? Thank you.

DR. HSU: But sometimes it could also be fatigue just at rest, and there is a difference there for heart failure. And bone marrow transplant is one of the things that can really help quality of life. One of my patients said that before going to bone marrow transplant he couldn't go work out because he would get pain episodes after just a gym workout, but now after transplant, he's working out, his biceps are twice the size they were before. So he's very happy about it.

MS. VAIDYA: Thank you, Lewis.

DR. HSU: Thank you.

(Applause.)

MS. VAIDYA: Next we have Kim Smith-Whitley. And I would like to remind everyone to please try to

stick to the 2-1/2-minute limit just to stay on time so that we don't get out of here too late.

DR. SMITH-WHITLEY: Hello. My name is Kim Smith-Whitley. I am the Clinical Director of the Children's Hospital of Philadelphia Sickle Cell Program and also the Chief Medical Officer for the Sickle Cell Disease Association of America.

So I only speak, as far as nomenclature is concerned, as regards to children and adults living with sickle cell disease as patients today because that is the common theme that providers, the FDA, and us in the community are really addressing this very important issue collaboratively all in the room together to work for a final endpoint.

So I just wanted to point out that we have heard many themes today. We've heard about pain, we've heard about lessening the intensity, the duration of the pain. We've actually heard about different types of pain and how to address those issues. And we've also heard about the silent impact of sickle cell disease, the chronic organ damage, the kidney disease. We've heard about the need to address

the whole patient and to focus on survival, to focus on survival.

So when we really summarize what's happened today and we take these messages back to our communities, I really would like everybody to empower themselves, to spread the message about the importance of clinical trial participation. And to change the framework in which we're doing it, we really need to be responsive to this public docket that is going to be open until April of this to go on and write your comments and maybe ask yourself, "As I take my folic acid, as I take my penicillin, as I participate in the care that exists now, that has let me live this long, somebody participated possibly in clinical research to get me here. What can I say in this open docket so that we can do things differently that encourages people in this room today to participate in clinical trials so that 10 years, 20, 30, 40 years from now there are fewer people sitting in this room trying to address the same issue?"

Thank you.

(Applause.)

MS. VAIDYA: Thank you, Kim.

Next we have Ibrahim Kargbo.

MR. KARGBO: First I would like to say thank you to the FDA for providing an opportunity for us to come express our thoughts and our experiences.

We all know that developing trust, whether it's with your hematologist or with a government organization, requires us sharing an issue and us seeing action being taken. So I'm addressing this specifically so everybody sitting down with the FDA, you have learned a lot about us, we have opened up to you in ways that many of us do not open up to our hematologists, we've opened up to you in ways that many of our families have never seen us before. So now we are asking you, what should we expect in terms of action? We know how slow government moves --

(Laughter.)

MR. KARGBO: -- but when it comes to dealing with the lives of people every day, we need quicker action.

Thank you.

(Applause.)

MS. VAIDYA: Thank you, Ibrahim.

DR. FARRELL: I want to really thank all the participants. As I said earlier in my opening remarks, we're all part of this community trying to solve this problem, and I think as Dr. Whitley was very eloquent, we all are part of that solution, and we had a wonderful participation from all the communities that are involved -- the pharmaceutical community, academia, patient groups, advocacy groups, the press, and interested observers -- and so I think working together is how we're going to solve the problem of this chronic and debilitating disease.

Thank you.

MS. VAIDYA: Thank you, Ann.

Next we have Tina Kay Hughes. If you could raise your hands.

MS. HUGHES: I host two radio shows that come on the internet and that are heard around the world. I'm always looking for sickle cell patients and parents, caregivers, to tell their stories because our stories need to be told. So if you want to tell your story, please see me afterwards.

Also, I have a book and a CD for sale and I do have them with me. And my website is www.tinakay -- T-I-N-A-K-A-Y -- .net. And I'm just going to share a small piece from my CD.

I feel like there is an urgency on my life because they say the life expectancy of a sickle cell patient is 45, maybe 50 years old. God has given me this duty, this calling, to be his mouthpiece and not just a pretty face on a sickle cell poster board. Even days that my body aches to no end, I remember the stories from other people who have sickle cell disease just like me going into bankruptcy, debtors court, losing their jobs, using up their 401(k)'s they once had when they could work, pawning belongings to make ends meet, driving themselves to the emergency room because this disease is just too much for their own families to be there for and with them.

I hear the voices and I hear the stories, so, yes, I take up the torch of light like the torch from the Olympics being passed on so this disease will not remain some dark curse that many sickle cell patients think it is. Yes, I take up the torch for

those who need to be educated. I take up the torch to let whoever is clueless about what they have deemed an orphan disease, that they say plagues 100,000 brown people but a census has never been taken in this country. I will even take up the torch to reveal the imbalance between different diseases when it pertains to different races receiving far more than what we get. They get funding and support and all kind of education. So, yes, I stand here with this torch in my hand and I will carry it, I will light up the way even when the road is narrow --

MS. VAIDYA: Tina --

MS. HUGHES: -- even when my body seems to be failing me, even when there is only God with me, until I can carry this torch no more."

(Applause.)

MS. VAIDYA: Thank you, Tina.

And last we have Dawn Nelson.

MS. NELSON: I would like to thank the FDA for having this meeting. And it was worth missing work to be here and fly here. But one of the things -- I've lost passion for my work because my daughter

has sickle cell. And I don't know if other people feel that way. Nothing else seems really important when you're fighting this battle all the time. But I've gotten a lot of information, a lot of passion, a lot of -- I'm so happy to see that people are 71 years old and 68. And she texted me, and she's in her hospital room.

But one thing I've heard that I could probably get some passion from -- I'm an audiologist -- is that there is lots of hearing loss, and we haven't discussed that today because our agenda is a little bit different. And I know that a gentleman here has done some studies in hearing loss, and that's my area, balance and hearing, and I just wanted -- but I knew that this is a -- if I wanted some patients and if I wanted to get some subjects for a research study, I've got a whole room full of people, but I have no way to get in touch with you. And so I asked if there could be a sign-up sheet. There is probably a HIPAA violation, there is probably some other name for it in this setting, for the FDA to give me the names of the people that are here.

But I would first of all like to get an idea of how many people are affected by hearing loss. The room is kind of empty now, and I would just like to get a feel for what that is about and if that's something worth looking at. And what about balance issues? So, you know -- thank you.

Andrea? Where is Andrea? Did you set up -- there's a sheet outside? There is a sheet outside on the table, and if you would be willing to put your name on it and your e-mail address, then I can go back and talk to Fred Bess at Vanderbilt, and we're interested in doing some sickle cell research, that's something I could develop some passion about. And I would happy to look into that. And I can see a career, another -- I mean, I have a career, but I would love to -- a career path that I think that I could make a difference.

But I would love to be able to learn more from you and to get more from you, do surveys, some of this information that we need, but we need to be able to get in contact with you. So if you would sign that sheet, give your e-mail address, I would be happy, and

I can work with Andrea to get your information.

MS. VAIDYA: Thank you so much, Dawn.

Thank you, everyone, for your comments.

So before we get started with our last agenda items, I would like to ask you to please pass your clickers to the far ends so that our FDA staff will be able to pick it up.

And now I would like to call Dr. Kathy Robie Suh --

DR. EGGERS: Pujita, could I interrupt? This is Sara.

MS. VAIDYA: Ooh, right over here.

DR. EGGERS: Yeah. I just want to interrupt for one minute.

MS. VAIDYA: Okay.

DR. EGGERS: And that is, we've had some questions on the web about how people can find out more information about future patient meetings. And so FDA has a Patient Network website. If you go to fda.gov, you will -- oh, if you go to -- he gave me the website. If you go to www.patientnetwork.fda.gov, you will find information for patients and patient

advocates, and it's a place that patients can learn more about participating in clinical trials and other FDA topics as well as sign up for the Patient Network Newsletter, which highlights meetings like this one that happened today.

MS. VAIDYA: There's a sign back there.

DR. EGGERS: Oh, there's a big sign back -- oh, look.

(Laughter.)

DR. EGGERS: It comes. There's a sign.

MS. VAIDYA: Thank you, Sara.

And now I would like to call Dr. Kathy Robie Suh to the stand.

Closing Remarks

DR. ROBIE SUH: Well, we have come to the end of what has been a stimulating and productive day. Your discussion today underscores the need for treatments for sickle cell disease as an unmet medical need. The information, comments, and perspectives you have provided today will help us at the FDA as we work to advance development of treatments for sickle cell disease that address patient needs.

Before I thank you all for coming here, I want to just take a moment to recap just a few of the points of the discussion today.

You discussed the health effects of sickle cell disease that are most significant to you as patients and caregivers. You discussed the specific impacts that the serious health effects have on your daily lives, on school attendance and concentration, on work, on caring for your children and families.

We heard that especially for young patients in school, concentration and ability to focus mentally, the challenge of being mentally present, the challenge of maintaining social relationships with friends and peers are critical to you.

We heard that sleep disturbance is very common.

We heard that you worry about dying early, about a cure not coming in time.

We heard that you worry about being able to work to your potential and about being able to have children and a family.

We heard that physical activity is often

severely limited.

We heard that pain is a constant and chronic problem that is managed with varying degrees of success, but that pain medications have effects of their own that can make your daily life more difficult.

We heard about the long pauses sickle cell disease causes in your lives.

You provided insights into the range of approaches and things that you are doing to manage your disease, including hydroxyurea and transfusions, iron chelation, but more broadly, non-prescription, diet, lifestyle changes, and alternative therapies.

You discussed the challenges to making the best use of what's available and you commented on the limitations and shortcomings in your interactions with the health care system.

You discussed the physical toll sickle cell disease has taken on your lives: splenectomy, cholecystectomy, compression fractures of the spine, chronic bone pain, avascular necrosis, joint replacements, leg ulcers, stroke, loss of hearing,

vision problems, priapism.

You talked about things that you would want in an ideal treatment including a direct effect on the disease and something to stop the tissue damage.

You shared your perspectives on current and emerging treatments and on clinical trials or more experimental treatments.

We heard that sound rationale for expecting a benefit and a complete explanation, full explanation, of a clinical study, and being treated with respect are important to you in considering whether to participate in a clinical trial.

We heard that you are concerned about rare but serious side effects of medications.

As Dr. Farrell described to you in her comments at the beginning of today, the development of new treatments is a long and arduous process. Your contributions today will help us at FDA work with the manufacturers, academic community, health care community, advocacy groups, and patient community to work toward designing trials that will focus on developing the new treatments for sickle cell disease.

On behalf of the Division of Hematology Products and all of us here at FDA, thank you all for participating in today's discussion. We thank both those who are here present in the room and those who are participating on the web. In particular, we especially appreciate and thank the patients' parents and caregivers who have taken the time and effort to come, speak, and write or voice their experiences, concerns, and perspectives on living with sickle cell disease. Expressing this requires a certain amount of bravery, and we appreciate that. We especially thank all the panelists for their articulate and strong testimonies of their experience with sickle cell disease and its treatment and what sickle cell disease means in your daily lives.

And, finally, we also thank the health care providers, representatives of the pharmaceutical industry and academia, other government agencies, and FDA colleagues for taking the time to listen as well.

Again, thank you for participating and a safe journey back for all of you who are traveling.

(Applause.)

DR. EGGERS: With that, I will officially close this meeting. And I will echo Kathy's remarks, I hope that you have safe travels, whether you're just getting around the Beltway or whether you're flying to Chicago or California. Thank you very much.

UNIDENTIFIED FEMALE SPEAKER: Evaluations in the back.

DR. EGGERS: Oh, yes. Thank you for doing my job for me. We'll take evaluations in the back.

(Whereupon, at 4:06 p.m., the Sickle Cell Disease Public Meeting on Patient-Focused Drug Development was adjourned.)

H

I