Dr. Wendy Book  
APFED President

Alright, everyone. We're going to get started for the afternoon session, and then hopefully get everyone out of here in time to catch their flights. While everybody's getting settled, I want to again thank our speakers who are here today. We have the world's experts in eosinophilic diseases here today, and that is an amazing treat. I want to thank Knopp for making this opportunity possible, FDA for coming and listening to the patient voices, Mary Jo Strobel, who has already disappeared to do some more work for organizing this conference, and she's APFED executive director and is doing a fabulous job putting this all together, with the help of Jen Roeder who has been working behind the scenes.

So, we will go ahead and get started again. We're going to start with some polling questions, and then we're going to hear from our speaker. Our patient panel is, I'm sure, exhausted already, so this is it. You get to go sit and be an audience member after this and listen for the rest of the day. Alright, let's start with our first question. These questions all relate to the ideal treatment. So, your opinions on what the ideal treatment would look like. So, when considering a new treatment for hypereosinophilic syndrome, which of the following benefits would you consider to be the most important, and you can choose up to three; improved symptoms, decreased fatigue, lower counts, lower cortico steroid dosing, decrease in organ damage, have few or no side effects, and convenient to take.

Alright. I think we have most of them in, maybe a few more coming in.

Alright, looks like the priorities are improving symptoms and improving end organ damage along with everything else. So, everybody wants everything from this new treatment, no pressure, but all important things to those who responded. When considering a new treatment for hypereosinophilic syndrome, improvement in which activities of daily living would you consider to be the most meaningful, and again, you can choose up to three; greater participation in work or school, greater participation in social activities, improved concentration and focus, improved sleep, improved relationships, improved sexual intimacy, fewer emotional impacts, and less depression, anxiety or worry are the choices.
Alright, so the theme is less depression, anxiety or worry, improved concentration and focus, greater participation in social activities, work, better sleep, and a few others. Alright. Just two questions, yes? Okay. Alright. We're going to move into our last discussion segment, and then after that, we're going to hear from a couple more expert speakers on new treatments. So, with those things in mind, I'll start with our panel. Aside from a complete cure, what meaningful improvement would an ideal treatment provide?

**Shelly Parks**
*HES Patient*

Okay, so when I was first diagnosed with HES, my immediate thought was I was going to crush this disease and work to find a cure, and as time has gone on and my disease has progressed, I have come to realize that managing my symptoms is more realistic than finding a cure, not to say that I will ever give up hope, I hope that there will always be, in the long term, a cure for HES so that people don't have to go through what we have already faced. Yeah, symptom management would be just as important for me if not more important, that way I could get back to work, social activities, exercising, basically just having more of a life than I already do now.

**Tina Singer**
*HES Patient*

I would like to see infusions instead of having infusions, having pill form or the option to have pill form. I would like to see help with chronic fatigue, and then find a way to have the eosinophils in the blood match the eosinophils in the organs so doctors can better treat the patient's organ damage before it gets to the point of having to remove the organ. That was, in my case, more than once, where my eosinophils did not match my organs, and they were removed. So, I would really like to see that connection or test to help with that.

**Lorien Hall**
*HES Patient*

I guess I would just easier access to medication, just doctors being more educated in what's going on. I guess the most important thing to me is just quality of life and trying to be productive to society, feeling like you have a purpose. Any therapies that can allow any of us to do that, I think is good.

**Amber Felts**
*HES Patient*

I agree with what the other panelists said, and I'd also like to add an ideal treatment would be something that we also know how it may affect us in the future. Like I mentioned with Nucala, maybe someone here can tell me the answer, but I was told we're not really sure long term what this could do to your body. So, even though treatment might sound ideal, you might be sacrificing your health later for your health now, and then on top of that, affordable and less hoops to jump through to get to it.

**Dr. Wendy Book**
*APFED President*

Any comments from the participants here today? If you have any comments, raise your hand. Yeah, okay.

Next question for the panel is what factors or information on the potential benefits of treatments do you take into account when you decide on a course of treatments, and when you decide you're going to start a new therapy, what are the types of things you take into account?
Amber Felts  
*HES Patient*

Well, since I just did this recently, some of the stuff I took into account was what my current treatment, I was on prednisone, what the long term effects of that were going to be, what they were, how they were affecting me in the moment, and compared to other options in terms of how I would be able to function better on a daily basis. So, I guess it comes down to the side effects would be my first considerations. Yeah, I'm going to go with that.

Lorien Hall  
*HES Patient*

I've always done just the benefit versus risk factor. For me, steroids are a huge risk because of the bone loss. So, even though cyclosporine is not that great of a drug, my kidney is in better shape than my bones, but it's always been a benefit versus risk factor for me.

Tina Singer  
*HES Patient*

I was originally on high doses or prednisone for five years, and that was the only option I was given from my doctor, and I just trusted him. When things started getting worse, that's when I looked into getting into the test study, and that was kind of like my last hope at the time. When I asked my doctor about newer treatments for HES, he says that he doesn't think that they will work as well for my HES and would have more side effects. I would definitely consider a different form of treatment if it helped me with the chronic fatigue.

Shelly Parks  
*HES Patient*

I pretty much agree with the rest of the ladies up here. I do a benefits and risk analysis to see if the benefits for this drug will outweigh even some of the side effects that I may have from it, and if long term, if this is a drug that will keep me healthy longer than what I'm on now, it's definitely a risk that I would take.

Dr. Wendy Book  
*APFED President*

Any comments? Got a question back there.

Audience member  
*HES Patient*

Another thing I look at with new drugs is effectiveness because I would like to know is it an 80 percent chance that I'll see results, I'll see improvement, or is it a 30 percent chance because even if it has less side effects, I don't know if I'd be willing to jump on this new bandwagon and try this medication if it's not more likely to work.

Dr. Wendy Book  
*APFED President*

Alright. Any other comments? I'm going to go off script for my own curiosity here. Those of you that are in a clinical trial or on mepolizumab, Nucala, was it your doctor who suggested that, or did you bring it to your doctor and ask for it?

Tina Singer  
*HES Patient*

Okay. I actually found Dr. Schwartz by reading about the trial online, and I went to him, and he went through all the procedure and everything. I sought him out.

Lorien Hall  
*HES Patient*

I was presented with the mepo from an old immunologist I had right before I was going in for the bone marrow transplant, so as a last option.
Amber Felts  
*HES Patient*  
I learned about Nucala from the online community through APFED and brought it to my doctor and asked her to get me approved.

Dr. Wendy Book  
*APFED President*  
Alright. My next question for the panel, and Mary Jo will be able to take some online calls during this time as well, when you're weighing the potential benefits of a treatment versus common side effects, how do you weigh the potential benefit versus common side effects such as headache or nausea? How do you balance that risk benefit as you're thinking about a new therapy?

Amber Felts  
*HES Patient*  
For me, I think it just comes down to what the risk is and if I can manage it. For example, when I was on Prednisone, that was helping my symptoms, but I knew it was doing other damage. So, I supplemented with things that I knew would protect my body from the bad side effects. So, if it's something I can somewhat control and help and I think it's worth it, again, as she mentioned, just outweighing the benefits versus the risk and how those balance out.

Lorien Hall  
*HES Patient*  
I know that when you're sick and you think of death often, I mean, I've pretty much said I'll do anything and everything just for a chance to survive. So, once you've been sick and suffered, you're not really like oh, I'm going to get a headache, oh, I'm going to throw up once. None of that's really ever crossed my mind. It's always like can you help the disease or illness?

Tina Singer  
*HES Patient*  
I look at the quality of life. Basically, if I found a treatment that would give me more energy and allow me to do more things, but I would get a case of nausea and maybe in the morning and the evening, I feel like I can handle it, I would definitely take the benefit of having that energy and a better quality of life, and able to do more things because at this point, I don't do a lot of things, a lot of anything, except for teach. I would definitely look towards the benefit of having more energy and sacrificing maybe feeling sick once in a while just so I could have a little more energy during the day.

Shelly Parks  
*HES Patient*  
I pretty much agree with everything else everyone has said. For me, I suffer from severe GI tract issues. If a drug is going to cause more nausea, vomiting and things like that, for me, that's something I have to consider because at the place where I'm at now, anything I eat makes me sick to my stomach. So, if the drug were to be able to allow me to eat again and put weight back on and be healthier, and there were still those small side effects, headaches, nausea, vomiting, 110 percent I would do it if I could just, like they said, get the quality of my life back up to a point where I could leave my house, and I could do all the things that I love to do, even go to the grocery store without being in pain. I would definitely take that on.

Dr. Wendy Book  
*APFED President*  
Any comments from participants in the audience? No? Alright. So, let's take that same concept a step further. How would you weight the potential benefits of a treatment against less common side effects that are serious, such as liver/kidney damage? At what point does that risk benefit become worth it?

Tina Singer  
*HES Patient*  
I would not try a treatment that had a chance of a serious risk factor. With immune system being weaker, it's just ... and I don't have any organs left to take out. It's kind of like, don't have to worry about the liver or anything, but if the risk factor is something major, then it's not worth trying when I already have the odds stacked against me with HES.
Lorien Hall  
*HES Patient*  
I guess I ... a lot of my medicine has potential to affect the internal organs, like the kidney and liver, but we do a CT scan every year, and a heart scan, and if everything's looking good, then we say stay on the same medication. Like when my kidney failed, we obviously got off the cyclosporine, and then it came back, and then we reintroduced it.

Amber Felts  
*HES Patient*  
I think it depends on where you are with the condition. If I were in a position where I had tried everything, and I was suffering and not able to function, then I would consider something that was risky if I felt like I didn't have any other options left, and especially with all, like she mentioned, we get blood tests constantly, we have our hearts checked, we have our organs checked. So, if I knew I was going to be monitored enough to catch something like that, then I would be willing to risk it a little bit if I was in that position.

Shelly Parks  
*HES Patient*  
I agree with them. I think I'm kind of at the point with my disease that I would be willing to try anything. At this point, I failed several, several rounds of different medications, and even with being on the high dose methotrexate, my immune system is very suppressed, and I've already run into several infections and extended hospital stays. I think at this point, I would really do just about anything to get better. Maybe I'll regret that later.

Dr. Wendy  
*Book*  
*APFED President*  
Maybe not. Any comments from any of the patients or caregivers in the audience about weighing risk benefit of medications? We've got two fathers in the room. No comment about how you'd weigh that risk benefit? You've got to say it into the microphone.

Audience member  
*Parent of HES Patient*  
I think they've said it all. I think they said it very, very well, and I would agree with everything they said. It depends on the position you're in and what the risk/reward would be.

Audience member  
*Parent of HES Patient*  
So, since you asked, as a father, one challenge we had, a frustration we had as my daughter was diagnosed, she was younger than 12, and she wasn't eligible for some of the medications. Mepo was out, but I think it was compassionate use at the time, and there were protocols, so she couldn't get it. At that point, risk/reward, yeah, give it to her. Anything. Her quality of life was so poor that, absolutely, we would do it. So, yeah. There you go.

Dr. Wendy  
*Book*  
*APFED President*  
So, you think that the same statements hold, even for the kids who are young?

Audience member  
*Parent of HES Patient*  
Absolutely. It all holds. Yeah. That would be the one comment for me. I guess the FDA had the age limit, and I understand. There's reasons. I get it. There's reasons why, but in our case, we wanted the opportunity for her to have something. Her only choice at that point was the prednisone or the methotrexate.