

(Mark Dunning) Hi everybody. Wow, look at this crowd, this is awesome! My name is Mark Dunning, I'm the chairman of the Usher Syndrome Coalition, and I'm also the father of a 19-year-old daughter who has Usher syndrome type 1B.

So this is, I am not just one of the speakers, I am also one of you guys, one of the families here. And my job today is to kind of introduce what the purpose is of the meeting here and what we hope to accomplish. I also want to let you know that you will see on your chairs, there are flyers about taking pictures for the Usher Syndrome Society. We would love to get people's photos. You have probably seen the pictures out in the lobby area. We have a fairly well-known photographer here taking pictures, portraits of people with Usher syndrome. And you can do that upstairs in room E6. But if you need guidance getting there, we have four people here who will guide you upstairs where you can have your photo taken.

I hope you use that opportunity, you use the breaks or any other time you have to get up there and get your photos taken. So welcome. This is the 10th time, the 10th annual family conference that the Usher Syndrome Coa-

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lition has helped to organize. And this is the first time we've done it in Germany. And I can tell you based on this crowd, we'll be back, because this is pretty amazing to see all these people here. I want to thank Irmgard, who is up here handling the accessibility, Sebastian, who has done a ton of work with organizing the family side of things, and of course Uwe and Kerstin, who organized the whole area here and did a ton of work on this and are running around quite a bit. So I want to thank those guys for everything that they have done. (applause)

I suggest giving each of them a kiss when you see them. So the purpose here today really is to try and build an usher syndrome community. And there is a lot of reasons for needing to do that. We are all here, we are all interested in treatments, I know you are all here to learn about the science. But the truth is that none of us are really concerned about going deaf, excuse me, being deaf and going blind.

What we are really concerned about is being socially isolated. And that's the real problem with the disease is that it separates us from the things we want to do and the people we want to be with. And that's what we are trying to overcome with these treatments. So the medical side of it is really important, because it gives us access to this other thing that we really want. So the way for us to get to these treatments is through you guys. You are the source of everything that happens in research. You are the source of the genotypic information that we found.

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So we know about the genes, because we know people with Usher syndrome. We know about the phenotype, what happens with the disease, because we know people with Usher syndrome. You guys are the sources of funding for all the research that has happened, whether you have written a check, or you have run a fundraiser, or you have just simply lobbied your government or lobbied a funding organization. That's where the money comes from. It comes from you guys, whether directly or indirectly. Money is a critical component defining this research, finding treatments.

You guys are also very important though for the next step in moving these treatments forward. You will hear in the science talks today that we are getting pretty close to bringing stuff out of the lab and into the clinic. But that next step is the big step. It's the most expensive step, and it also really requires you guys, because to be able to get something from the lab to the clinic requires us to do clinical trials, that means trying this in humans for the first time. And clinical trials are not a guaranteed thing, in fact, only one in 12 clinical trials results in a treatment in clinic. That doesn't mean that they all fail, it means that a lot of it is trial and error.

So you have to go through a lot of clinical trials to be able to get to the point where you have treatments for the entire community. And to be able to do all those clinical trials you need a lot of people. And that means we need a big community, we need the community to participate to be able to move these things forward. But we have a pro-

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blem with that. The problem is that we are not in touch with enough people with Usher syndrome. There are hundreds of thousands of people in the world with Usher syndrome, and we are only in touch with a small fraction, with one or two percent of those people. So we are going to need just about everybody to be able to move these things from the clinic into treatments. So events like this, where we get to meet people and connect with people and hold onto people are incredibly important for us to be able to make it to clinic and get things to the clinic.

You guys are also an important factor in motivating pharmaceutical companies to invest in usher syndrome research. Because pharmaceutical companies want a market. They want a place where they can sell their treatments. And so the more people that we can say we are in touch with, the larger a market we can present to them, the better the chances are that they will continue to invest in the research for Usher syndrome. So the community is important on those levels just to be able to deliver any sort of the treatments we are going to talk about today.

But remember, ultimately what we are trying to cure here is not deafness or blindness, it's social isolation. Well, look around you. This is why we need this community. Okay, you guys literally are the cure. There is no social isolation in this room today. There is 250 people here. And this is really what we want out of the community. In this room you are not alone. In this room are people who understand you. There are people going through the

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same thing that you are going through. There are people in this room who share the same fears, who share a lot of the same goals. This is what we want. This is the challen-



ge to social isolation is building this community. So there are treatments coming, and they will be here soon. And in the interim we want the community so that we have each other, so that we have this, and we have opportunities to be with people who love us, to be with people who understand us.

There are people in this room who understand me better than my own family, because my aunts and uncles, they love me, but they don't know what it's like to have a child with Usher syndrome. But people in this room do. I have connections to people in this room who I have never met that are deeper with them than with my own family, and

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so that's what we want to really build out of this. That's the goal of this community is to fight that social isolation. And the best way to do that is to be together and to hang together on this stuff. So we have the means to find treatments. And you will see that here over the course of the day today, there is a lot of wonderful stuff coming. But in the meantime, we need each other.

And so we don't want this to be just a one-time thing where you guys just come out today and then we lose touch with you. We want to maintain contact, we want you to join the different parts of the organization that help to maintain accessibility between each other, and we don't want to lose touch with you. We don't want to meet you for one time and then not see you again. And I know that that's not the case.

I'm looking over here at my friends from Australia whom I see on a regular basis. I have friends from Austria whom I see on a regular basis now. And so there is lots of people that I've become very close to simply because of going to these conferences. We want to maintain that connection, we want to maintain that community. Okay? So that was all I wanted to say. I also have the great pleasure of introducing one of my heroes, Christina Fasser from Retina International, who is going to come up and talk to you a little bit more. I want to adjust the microphone before you... It's right in front of you.