

(Christina Fasser) Thank you very much Mark. I didn't know that I was a hero, so I learned something new today. Ladies and gentlemen, dear friends. I feel very honored and privileged to give one of the introductory talks at this usher syndrome conference.

I thank you very much the doctors Kerstin and Uwe Wolfrum for having invited me and the organizing committee to speak to you on behalf of Retina International. Retina International is an umbrella organization of 43 organizations, patient-led organizations that promote research into retinal degenerative diseases such as retinitis pigmentosa, Usher syndrome, macular degeneration and similar conditions, as well. I think that's one of the most important things, to foster mutual support of each other affected by one of these diseases.

As Mark said: First we have to live it today. We have a wonderful future to come, but today is the day we have to enjoy. And having retinitis pigmentosa myself - I'm blind in the meantime of it - but one of the biggest supports in my life were my role models with retinitis pigmentosa and all my international friends within Retina International community.

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That's one of the biggest pleasures for working for Retina International that you meet friends that you would never have met otherwise. So I met a very long time ago already Sebastian Klaes, an usher person here very important for the organization. Of course also Dr. Reinold Funkischitzki who was one of my peers in the very beginning up to the late age of today. And I think just learning from each other, it's the best thing that life gives you with Usher syndrome or retinitis pigmentosa. Now just coming back to what we are doing at present - what are we concerned about? Retina International's objective is to make sure that every patient with a retinal degenerative disease gets the latest information about ongoing research and information that is validated. To make sure that patients know what is important and what is quack.

When we are affected by a degenerative disease for that at present no cure is available, then a lot of quacks show up, and we see that for instance at present in the stem cell area, where FDA just has issued a very clear warning letter. Stem cell research is an interesting research. It's already done in some human clinical trials which show first very positive results. However, these are very first results. Every stem cell treatment offered economically, you have to pay for it, is trash and might be even very dangerous for you. And I think these are the kind of messages we have to give.

On the other hand Retina International and its member organizations represent more than 1.6 million people affected by retinal degenerative diseases. And that's a huge

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amount. Compared once again to the overall estimation of people with retinal degenerative diseases it's about 10%. But 10% is a big number which can change the world. And how can we do that?

The first gene therapy has been approved last December in the United States by FDA, and very luckily it has also been very quickly reimbursed by the health insurance. We do hope that in Europe EMA will issue a positive opinion within the next weeks or days - we don't know really - and hopefully will register it by the end of the year. And then we will meet the cultural differences. Europe is divided into 26 countries. And within the EU the authority to decide what is reimbursed or not reimbursed stays with the member countries. That means discussions have to be done in 26 countries to get it reimbursed.

To do this, we will need a lot of lobby. And we will need the patients and also you with Usher syndrome to go out, because the Usher syndrome at the moment we have three clinical trials ongoing, among them also gene therapy trial. If they should be successful, then it's also your concern whether it's reimbursed or not. Therefore it's so important that we all tell the insurers why it is so important that the treatment or a gene therapy is paid for for the patients and that we do have access. Access to therapy is a human right.

All European countries and a lot of other countries have signed the United Nations' declaration for the right for people with disabilities. In article 5 of this declaration it

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is stated that each person with a disability has the right to access treatments that lower the burden of our disease. And that means we don't need cure, hundred percent vision.

I think it would be wonderful if we got treatment that is slowing down the progression of the disease or preservation of the disease. And for this Retina International has also taken up the cultural differences between Europe and other outside US countries and FDA. In our countries the health payers are asking what the extra value for the patient is. What is really important for us. And there we need the help of all of you. I am a person that has lost vision completely. And I do know how helpful it was when I only had night perception and could direct me myself toward a window or follow something, or even when I had a very small visual field that allowed me to read print.

For health payers sometimes this is no difference. So we patients, we are the experts, and you with Usher syndrome, you are the experts, too, to tell the payers what is important to you, how good it is to see a little bit better at nighttime. How good it is to have even a small visual field preserved, and how it helps you for social integration and to stay in the labor market. Economists always want figures. And of course staying in the working place means we are there, integrated in our social lives. Please stand up. We as patients, we are taxpayers, we have the right to complete inclusion into society, and we just have to call for it. We don't beg for it. And on the other hand, take your most important strengths. You as a patient, you as a

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parent, you as a sister or a brother or an uncle or nephew of a person affected by Usher syndrome, you know what it means to live with Usher syndrome.

Go out and tell the people that it is not a lost case, that we have lives that are worth to live, to live from day to day and that we all are part of the society. We are ready to give our part, and we wish and we ask for our integration. And just start to be the expert today. Learn from each other, and also share just laughter and fun, because that's also important in life. I wish you a wonderful conference, a lot of new persons to meet and perhaps some new friendships for a lifetime. Good luck to all of you.

(applause)