

Usher Syndrome Coalition | Advocating for Usher Syndrome Research Funding, Krista Vasi

Hi, everyone. I'm Krista Vasi, and I'm the executive director of the Usher Syndrome Coalition. I'm here to share how you can join the Coalition and become an advocate for Usher syndrome research funding.

I've worked for the Coalition for nearly a decade, but I first connected with the Usher syndrome community and this organization almost 14 years ago when I met an eight-year-old girl named Bella. Bella is the daughter of the Coalition's former chair and co-founder Mark Dunning, and she has Usher syndrome type 1B. Bella was the first person I met with Usher syndrome, and my first "USH" family member, as we like to call it.

I've been honored to feel like a part of the USH family, and to play a role in growing and connecting this vibrant global group. On the screen, I'm showing a grid of nine photos of different members of our USH family, ranging from a man tactile signing with a woman, to a woman with her guide dog, young children, and young women and men, all smiling, all living with Usher syndrome. The other two photos show Bella and me together:

One when she was about 12 standing next to me in Seattle, and the other-- the second showing us smiling at a concert just a few years ago.

Before we dive into advocacy, here's a brief overview of Usher syndrome and the role the Coalition serves. Usher syndrome is rare enough that most people have never heard of it, but in fact, it's the most common genetic cause of combined deafness and blindness. It's characterized by hearing loss or deafness, along with progressive vision loss caused by retinitis pigmentosa.

Some people with Usher syndrome also have balance issues or vestibular dysfunction. Between 13,000 and 56,000 people are affected in the US, and over 400,000 are living with the syndrome worldwide.

When the Coalition was founded in 2008 there was very little information out there. It was difficult to find support or near impossible to find anyone else living with Usher syndrome. There were a few researchers working on Usher syndrome, but they were isolated from each other.

It was clear that there was an urgent need to bring both families and individuals living with Usher syndrome together with researchers to find treatments and a cure. And so the Coalition was born. We bridge the gap between families and researchers while also providing information, resources, and support. We strive to be the most comprehensive resource for the global Usher syndrome community.

We do not fund research. Instead we are accelerating research by finding and supporting everyone living with Usher syndrome across all ranges, all types of Usher, and all modes of communication. There is no cure for Usher syndrome yet. The Coalition aims to change that. Building a community leads to treatments and a cure.

Now, turning our attention to advocacy, I'll share where we started and the milestones we've achieved, where we are today with our USH Champions, and where we need to go from here with your help. While the Coalition does not directly fund Usher syndrome research, we wholeheartedly believe in the power of this community. Engaged, informed, and vocal communities are behind some of the most meaningful shifts in research funding and attention paid to their disease communities.

Advocacy is particularly important if you're part of a rare disease community like Usher syndrome. Our USH family can and will succeed in advocating for increased federal funding and for Usher syndrome research, bringing us all closer to treatments.

We started by identifying the gap that we are working to fill. Put simply, more federal funding needs to be directed to Usher syndrome research in order to make meaningful progress toward treatment development. We need to do this in a way that doesn't impact the research currently being done for other rare diseases. The federal government has played a critical role in supporting the development of effective treatments and cures for many diseases.

ALS and Huntington's are just two examples of conditions that affect roughly the same number of people in the US as Usher syndrome, but Usher syndrome receives only a fraction of the funding that these and other deserving disease areas receive. The graphic on this slide shows a comparison between ALS, which affects 15,000 to 20,000 Americans and receives an average of \$79 and 1/2 million a year from the National Institutes of Health, or NIH. Huntington's disease affects about 30,000 Americans and receives an average of \$46 million a year.

Usher syndrome affects, as I mentioned, anywhere between 13,000 and 56,000 Americans, and receives an average of \$17 and 1/2 million a year, but we believe that number is actually closer to about \$7 million when you don't count projects where Usher syndrome is not a primary research priority.

I'd like to share a quick overview of our advocacy milestones. We started advocating for Usher syndrome in 2013. Much of our time was spent educating members of Congress and staffers about Usher syndrome since most have not heard of it. This laid the groundwork for future years and repeated meetings with members and staffers who now know Usher syndrome as the most common genetic cause of deaf blindness.

In 2014 we began advocating for the inclusion of specific report language and spending bills or appropriations that fund the National Institutes of Health, or NIH. This is essential to getting Usher syndrome research the funding that it needs from the federal government. Over the years, the Coalition has done a lot to make sure this happens.

Until 2015 we actually had no way of knowing how much funding the NIH was directing towards Usher syndrome research. So the Coalition worked with the president's Office of Management and Budget, OMB, to get Usher syndrome added as a new category in the NIH categorical spending list. And now, we can track funding year over year.

In 2015 the Coalition also launched the first global Usher Syndrome Awareness Day, which now falls on the third Saturday in September each year. This was also entered into the Congressional record by Senator Ron Wyden of Oregon. Since that time, members of your USH community have received proclamations from their governors recognizing Usher Syndrome Awareness Day in 20 states.

The Coalition continued to work to get report language included in specific appropriations bills from fiscal year 2015 through fiscal year 2018. In 2020 we reactivated our outreach efforts to members of Congress, virtually, of course. And we asked for their help in advancing our goals in Congress and with government decision makers. The response was fantastic.

We also launched USH Champions. Our team of advocates ready to make their voice heard and help ensure Usher syndrome research gets the federal funding it needs. Last year, we were able to make some important contacts and develop relationships with some key staff and members in Congress. Representative Jim McGovern in Massachusetts and Senator Ron Wyden in Oregon also placed statements in the Congressional record acknowledging Usher Syndrome Awareness Day.

This year, I was also able to have an introductory meeting with my own congressional representative here in Massachusetts, Lori Trahan. In short, for a grassroots team with just a few volunteers, volunteer lobbyists, and myself, we've accomplished a lot. Imagine what we can do if everyone who has an interest in advocating for Usher syndrome gets involved.

Now, if we've gotten this far and you are interested in becoming an advocate, this last bit of my talk will give a little bit more context about one of our strategies. Getting report language and spending bills and why this matters. So why do we care about appropriations bills?

Appropriations are annual decisions made by Congress about how much money the federal government agencies like the National Institutes of Health receive, as well as identifying funding priorities. Report language are essentially additional directions that accompany the funding amounts that's often included in appropriations bills. While report language is not legally binding, it is generally followed by agencies like NIH.

So at the beginning of the calendar year, the president submits his or her proposed budget to Congress. The House and Senate Appropriations committees are responsible for allocating funds among 12 appropriations subcommittees. The one we care about, the one we focus on right now is the Subcommittee on Labor, Health, and Human Services, Education, and Related Agencies.

Now, that's a mouthful, so we often refer to it as "LHHS" for short. This is the subcommittee that funds NIH, so this has been our primary focus.

Now, NIH is the nation's medical research agency. It's made up of 27 institutes and centers, each focusing on particular diseases or body systems. So the key institutes that we focus on for Usher syndrome are the National Eye Institute, NEI, and the National Institute on Deafness and Communication Disorders, NIDCD.

We work to get report language from the LHHS subcommittees, directed to NEI and NIDCD specifically, urging these NIH institutes focused on the eye and the ear to prioritize Usher syndrome research.

Here I've listed the current members of the Senate Appropriations LHHS Subcommittee. These slides and lists will be made available to download and view at your convenience. I also have the House Appropriations Subcommittee on LHHS, and if you have a member on either the Senate or House LHHS subcommittees, please reach out to us. These are key for us.

Now that we've caught up on what we've done so far in our advocacy, here's a snapshot of where we are today. This map of the United States shows where we have 28 USH Champions. These volunteer advocates are in 20 states representing 28 congressional districts. Our goal is to have at least one USH Champion in each state, and perhaps even one champion per district so every single member of Congress will have at least one Usher syndrome advocate directly communicating with them.

So where do we go from here? Our annual awareness day is quickly approaching, falling on Saturday, September 17 this year. The House and Senate are in session the week leading up to our USH day, so this is a great opportunity to make your voice heard. My first ask is, can you come to Washington, D.C. for a day on Capitol Hill?

It's recommended to try to meet your members mid-week, so that would be Tuesday, Wednesday, or Thursday, September 13, 14, or 15. But if you can't come to DC, don't worry. You can also visit your member's district office near your home that same week. You'll meet with your members staff, but that's great because staffers get the work done.

Alternatively, go virtual. You can send a letter, email, or tweet your members the week leading up to Usher Syndrome Awareness Day. We'll share suggested messaging and tweets as we get closer to the date. In the meantime, don't forget to ask your governor to proclaim September 17 as Usher Syndrome Awareness Day this year. We'll share a link that explains how to do this.

So, now here are some next steps for you. You can sign up to become an USH Champion. We have the website link provided. And email me, Krista, if you can go to Washington DC September 13, 14, or 15,

if you know anyone in government or have valuable contacts, if you have experience or expertise in advocacy, or you're just generally interested. Also, be sure to ask your governor to proclaim Usher Syndrome Awareness Day.

So, thank you so much for sticking with me through this, and I hope to hear from some new USH Champions soon. Take care.