

The Research Process

Video description: Kevin (a male with short brown hair and dark grey shirt) sits in front of a dark blue background, looks into the camera and signs.

TRANSCRIPT: Hi! Today's topic is Bill Kimberling - a very important researcher on Usher syndrome – his seven steps to treatment on Usher syndrome. Step 1: Find the genes for each type of Usher syndrome. Step 2: In the previous video, we discussed genotypes and phenotypes. This step is to match genotypes, which are genetically confirmed, with phenotypes, which are based on observable symptoms but not always accurate. Step 3: Find animals such as fish, mice, rats, pigs, etc., that have or could have a type of Usher syndrome. Step 4: Understand the disease, and possible treatments. Step 5: Develop treatments that can be tested on animals. They might be successful but, if not, start over. Keep revising and testing until you find one that works. Step 6: Before you're allowed to try these treatments on humans, researchers must first get approval from the Food and Drug Administration also known as the "FDA." The FDA has strict regulations on human research. Once approved, begin trials on a very small number of human participants to minimize the risk to humans. The rules for using animals in studies and using humans in studies is different. If this first round of trials are consistently successful, they may increase the number of participants and continue testing. This process will be carefully monitored to make sure it's successful. The whole process could take several years to make sure there are no long term negative effects. If any are found, they would go back and adjust the procedure or protocol. You might wonder how researchers select people for clinical trials. Applicants must have had genetic testing. You should be registered so that researchers can find people with Usher syndrome. Each clinical trial has their own criteria as to who can apply such as only people with a specific subtype of Usher syndrome or people in a certain age group. Often, researchers only want people who have lost most or all of their vision because they don't want to take the risk that someone will permanently lose their vision if the treatment is not successful. If clinical trials are successful and peripheral vision is restored, or if their vision stabilizes, the next step might be to apply to include more people with different types of Usher syndrome. That's our hope. IF you're considering applying for clinical trials, the first thing you should do is get genetic testing. Second - register with the Usher Syndrome Coalition. Remember, the USH Trust registry is confidential. If researchers are looking for participants they will contact the USC. The USC registry director will contact individuals in the registry who match the researcher's request and provide them with details about the clinical trial. That's all.

The USC is not involved in research. They are the bridge between researchers and the Usher community, which is important. And again - confidentiality is a high priority in the process. It's important to register because many researchers are working on treatments but if they can't find enough participants time and money may run out on us and we'll have lost our opportunity. We don't want that! What if they are close to finding a treatment... but they need more people like YOU? This is important. C'mon - register so that if you're interested, you can participate in clinical trials. I also want to assure you that researchers must follow strict FDA guidelines to protect the safety and health of participants and to cause no harm. Thank you.