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USHER SYNDROME COALITION

CONNECTING THE GLOBAL USHER COMMUNITY

GROUNDED IN SCIENCE: September 2025

A balance of research news and well-being for the Usher syndrome community

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Last week, our executive director, Krista Vasi, spoke on a panel at the RARE Drug Development Symposium, hosted by Global Genes. The discussion, *Developing Data Assets to Support Therapeutic Development*, highlighted the Usher Syndrome Coalition's work to bridge the gap between families living with USH and researchers working toward treatments. With more than 200 attendees - three-quarters of them advocates for their rare disease communities - the event was a powerful reminder of the impact our community can have when we come together.

And speaking of coming together, Usher Syndrome Awareness Day is right around the corner on **September 20th**! We hope you will join us in raising awareness and recognizing the strength of our USH community. We'll soon share a list of events happening in honor of USH Day. If you're planning to celebrate in any special way, please email Clare Weigel at communications@usher-syndrome.org to have your event included!

Join the USH Trust to stay updated on clinical trials and new participant criteria. Only ten questions need to be answered to register.

Join the USH Trust

RESEARCH SPOTLIGHT:
jCyte announces publication of phase I/IIa safety study of retinal progenitor cells in retinitis pigmentosa

In late August 2025, jCyte announced encouraging news for people living with retinitis pigmentosa (RP), including those in the Usher syndrome community. The company published results from its first major clinical study, which tested its cell-based therapy, called jCell. This therapy uses special “retinal progenitor cells” that are placed into the eye with a small injection. The hope is that these cells can support and protect the light-sensing cells in the retina, slowing down or even improving vision loss.

In the early study, 28 adults with RP each received a single dose of jCell. The main goal was to see if the treatment was safe, and the results were positive. No one experienced serious safety problems, and the cells did not cause rejection. Most side effects were mild, such as eye irritation, and went away on their own. Even though the focus was on safety, doctors also measured vision. People who received the highest dose—three million cells—saw an average improvement of about nine extra letters on an eye chart after one year.
Although not everyone improved, this early signal gave hope that jCell could have real benefits for vision.

Because of these results, jCyte has moved forward into a Phase 2 clinical trial, called JC02-88. This new study is a big step forward. It is testing a larger dose—8.8 million cells, which is about 50% more than the highest dose given before. Up to 60 adults with RP, ages 18 to 60, will take part. Some participants will receive jCell, while others will get a sham procedure (a fake treatment), so researchers can carefully compare results. After six months, those in the sham group will have a chance to receive the actual treatment as well.

What this means for the Usher syndrome community: Unlike some therapies that target just one gene, jCell is designed to work no matter what genetic change caused RP. That means it has the potential to help people with many different types of RP, including USH-related RP.

Check out our Current USH Research page specific to USH subtype as well as other gene-independent therapeutic approaches.

View Current USH Research

IN CASE YOU MISSED IT: Science News Feature

NIH Researchers Develop Eye Drops that Slow Vision Loss in Animals

March 21, 2025: Scientists at the National Institutes of Health (NIH) have developed new eye drops that may slow vision loss in diseases like retinitis pigmentosa (RP). The drops use small pieces of a natural protein called PEDF, which protects the retina—the part of the eye that helps us see. In mice with RP-like disease, the drops saved up to 75% of light-sensing cells, kept vision stronger for longer, and caused no harmful side effects. The treatment worked fast, reaching the retina in less than an hour.

The drops also protected human retina cells grown in the lab. Because of these results, researchers plan to start testing the drops in people soon. In mice, combining the drops with gene therapy kept vision stable for six months, suggesting the drops could work well alongside other treatments.

What this means for the Usher syndrome community: For people with Usher syndrome, these drops could be life-changing. They might slow vision loss, giving families more years of usable sight and independence. Since there is currently no cure for USH, an easy-to-use drop could provide hope and buy time until gene therapies or other advanced treatments are available.

Read Article

DISCLAIMER: The Usher Syndrome Coalition does not provide medical advice nor promote treatment methods. USH Science News is intended to help summarize more complex literature for the community to use at their own discretion.

For more science news, check out our Science News page, organized by treatment approach and type of Usher syndrome.

ON WELL-BEING: USHchats

Connect with other families living with Usher syndrome

USHchats virtual sessions provide a monthly opportunity for parents and carers of children and young people living with Usher syndrome (aged 0-25yrs) around the world to connect with each other. These informal Zoom calls take place on the 3rd week of

the month (see flyer for times), and are designed for parents and carers to connect with others whose children are at different ages and stages, to share experiences, ask questions, learn from and support each other. They are hosted in partnership with Usher syndrome organisations from around the world, with sessions at two different times each month to capture different time zones, and they offer English and Spanish language groups. Parents and carers have commented on how valuable these sessions are to them:

> "I don't know of any other family in my country. And then I join these calls, and here you all are. It is a lifeline for me."
>
> "The best advice I've had since our child was diagnosed has come from other parents who are on the same journey. Our friends and family want to support us, but they don't know how. The professionals we work with don't have experience of what it is like to live with Usher syndrome as a family, day to day. USHchats is where I get to meet others who understand and can share what has worked for them."

The next USHchats session takes place on **Thursday, 18th September** at 3pm and 8pm EST (*Fri 19th Sept in Australia) - see the image for details. You can sign up to receive invites for all USHchats sessions here: http://tinyurl.com/USHchats

USHER SYNDROME DATA COLLECTION PROGRAM

As the world continues to get to know the individuals living with Usher syndrome, it's a great time to join the Usher Syndrome Data Collection Program - the USH DCP - so researchers can better understand this diagnosis. If you'd like additional support enrolling, please reach out to Yael Saperstein, our Community Enrollment Coordinator for the USH DCP. Yael is an expert on the enrollment process, accessibility, and guiding new participants every step of the way. Contact Yael here: y.saperstein@usher-syndrome.org.

USH Tip

With fewer daylight hours in the Northern Hemisphere, you can make small adjustments in your daily routines to prepare for the fall / winter seasons such as setting up timers for lights or bringing an additional flashlight out with you rather than a phone light.

Send your USH Tips to info@usher-syndrome.org

Have you joined the Usher Syndrome Coalition Discord Community Server?
It's a safe place for the community to connect with each other. Join here: https://discord.gg/czwHGaDu7W

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