

USHER SYNDROME COALITION

CONNECTING THE GLOBAL USHER COMMUNITY

GROUNDING IN SCIENCE: January 2025

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

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Our thoughts are with our USH Community in Southern California as we witness the devastation from the wildfires. In light of recent events, it is even more imperative that disaster relief and emergency response factor in people with disabilities in communications and planning. For some resources, check out the [Disability Rights California Emergency Preparedness page](#).

RESEARCH SPOTLIGHT: Nanoscope Therapeutics

Nanoscope Therapeutics is a clinical-stage biotechnology company developing gene therapies for retinal degenerative diseases. In March 2023, Nanoscope announced positive topline results from its Phase 2b RESTORE clinical trial for MCO-010, a gene therapy for retinitis pigmentosa.

Late last year, in October 2024, Nanoscope presented conclusive trial data that demonstrated high and low doses of MCO-010 led to a statistically significant improvement in visual acuity over patients who received a placebo. The degree and duration of visual improvement achieved in the trial were unprecedented. Following these promising results, Nanoscope plans to file a Biologics License Application (BLA) in early 2025 to seek approval for marketing and commercializing this groundbreaking treatment. Details about the BLA process will be featured in the January 24th edition of our Science Education Clinical Trials series, available on our Facebook and Instagram channels.

[Nanoscope Therapeutics Press Release](#)

Check out our Current USH Research page specific to [USH subtype](#) as well as [gene-independent therapeutic approaches](#).

View Current USH Research

IN CASE YOU MISSED IT: Science News Feature

[Researchers closing in on genetic treatments for hereditary lung disease, vision loss](#)

March 4, 2024: For potential gene therapies for retinal degenerative diseases to be effective, there needs to be a good way to deliver the therapy to the photoreceptors in the retina. One delivery strategy is to use lipid nanoparticles (also called LNPs), which are a protective shell that gets wrapped around the gene therapy cargo and helps the cargo enter its target cell.

One common challenge with using LNPs is that they tend to accumulate in certain cells in the body (like the liver) rather than in the retina. To overcome this challenge, Drs. Gaurav Sahay and Yulia Eygeris tested dozens of modifications in the lipids that make up the protective shell of the LNP. They tested how these modifications improved the ability of the LNPs to deliver cargo to the photoreceptors and identified several candidates that could effectively deliver their cargo to rods and cones.

What does this mean for the USH Community? Gene therapy is a potential treatment for USH. However, one challenge for effective gene therapy is delivering the cargo (a healthy copy of a gene) to the right cells, like photoreceptors. It's also important to avoid delivering it to cells that don't need it. LNPs are a great candidate for gene therapy delivery because they can potentially carry very large cargo (like some of the USH genes). So, improvements in LNP technology that lead to effective delivery of large cargo to the photoreceptors can then be applied to USH subtypes caused by mutations in large genes.

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. As always, consult with your trusted healthcare provider if you have questions or concerns about your situation.

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

ON WELL-BEING: Setting Intentions for the New Year

As we welcome the new year, many people turn to resolutions. Let's consider an alternative: setting intentions. Unlike resolutions, which focus on specific outcomes, intentions are about aligning your actions with your values and the direction you want to take in life. Intentions can be empowering because they emphasize growth and flexibility, which is especially valuable for those navigating unique challenges in the Usher syndrome community.

Tips for Setting Meaningful Intentions

Reflect on the Past Year

Before setting intentions, take time to reflect on the previous year. What were your biggest achievements? What challenges did you overcome? Acknowledge both your strengths and the areas where you'd like to grow. Accessible tools such as journaling, voice recording, or tactile graphic tools such as raised-line paper or tactile drawing boards can help you capture these reflections.

Focus on Values

Intentions are most meaningful when rooted in personal values. Take a moment to identify what matters most to you. Is it community, health, creativity, or something else? For example, if community is a core value, your intention might be to build stronger connections by joining a [support group](#) or participating in local events.

Break It Down

Once you've set an intention, think about small, actionable steps you can take to support it. For instance, if your intention is to prioritize self-care, you might commit to setting aside daily quiet time or reaching out to a peer support network. Breaking intentions into manageable steps makes them easier to incorporate into your daily life.

Stay Flexible

Life is unpredictable, and it's okay if your intentions evolve over time. What's important is that they continue to align with your values and current circumstances. Give yourself permission to adjust as needed.

Tools to Support Intention-Setting

There are many tools available to help you track and maintain your intentions. Consider using large-print or braille planners, digital apps compatible with screen readers (such as Todoist or Google Keep), or tactile markers to create a visual or physical reminder of your goals. These tools can make the process more accessible and engaging.

Highlighting the Importance of Self-Compassion

As you work toward your intentions, remember to practice self-compassion. Challenges are a natural part of any journey, and it's important to treat yourself with kindness. Celebrate small victories, and don't be discouraged by setbacks. Growth takes time and patience.

Some Resources to Explore

Mind Body Skills Group (8-week program)

Join the next no-cost, virtual Mind Body Skills Group, hosted by Deb Marinos, starting at the end of January. This interactive group is designed for fluent ASL communicators to connect and share emotional experiences with others living with Usher syndrome or RP.

First session: January 30th, 2025

Time: 4:30-6:30 pm Pacific Time / 7:30-9:30 pm Eastern Time

Duration: Weekly for 8 weeks (January 30 - March 19, 2025)

Group size: Limited to 8 participants

Participant criteria:

- Aged 16 years or older
- Living with both RP and deafness
- Committed to attending all 8 weekly sessions
- Open to sharing emotions and participating in exercises
- Agreement to maintain confidentiality of group discussions and stories
- Willing to complete pre and post-learning goals, and provide feedback

Registration deadline: January 15, 2025. Don't wait - spots are limited!

Register here: <https://adaptabilityforlife.coursetorm.com/course/8-week-mind-body-skills-group-vision-loss>

Hadley Helps

If you're looking for additional support, consider the resources offered by Hadley. Hadley provides free workshops and tools for personal growth, including courses

on emotional wellness. Their services are designed to be accessible for the blind and visually impaired community. Visit hadleyhelps.org to learn more.

Let's start the year with purpose and positivity. What intentions will you set for 2025? We'd love to hear from you!

Check out our Mental Health Resources webpage

DISCLAIMER: The information and resources on this website are provided for educational and informational purposes only and do not provide medical or treatment advice. Check out our mental health resources page on our [website](#). As always, consult with your trusted healthcare provider if you have questions or concerns about your situation.

USH Tip

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

Flashlights are an incredible, affordable tool to keep at all times.

Ambutech makes a flashlight that clips on white canes and has adjustable-size clips for various canes - shop the [Pharos Cane Light here](#). For those who don't use white canes, a flashlight with a curved 180 degree span and different light settings is an excellent option, like [this one from Amazon](#), which is also water-resistant.



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