

USHER SYNDROME COALITION

CONNECTING THE GLOBAL USHER COMMUNITY

GROUNDING IN SCIENCE: March 2024

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

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March is a busy month here at the Usher Syndrome Coalition. A group of our U.S.-based USH Champions are preparing to meet with their Members of Congress to advocate for increased federal funding for Usher syndrome research. We will share how it went next month!

USH Connections Conference | July 19-20, Rochester, NY + Online

Hosted in partnership with the National Technical Institute for the Deaf

In other news, can you believe there are only 4 months until the 2024 USH Connections Conference? We look forward to meeting you there or online!

[Register here.](#)

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>

RESEARCH SPOTLIGHT

jCyte and Phase 3 U.S. Pivotal Trial

On February 21, 2024, jCyte released this exciting press release: "[jCyte Inc. Announces Positive Pre-Phase 3 FDA Type B Meeting and Outlines Plans to Start Pivotal Trial of jCell® for Retinitis Pigmentosa in The Second Half of 2024.](#)"

jCyte is a California based biotechnology company working to develop an innovative therapy to treat degenerative retinal disorders, including retinitis

pigmentosa (RP), and has received FDA Regenerative Medicine Advanced Therapy (RMAT) designation for its “first-in-class allogeneic cell therapy, jCell.” jCells are similar to stem cells that haven’t yet fully developed into mature photoreceptors and they [work to preserve vision](#) by releasing substances that reduce photoreceptor cell death and promote the function of surviving photoreceptors. Because cone survival is dependent on a substance released by rods, this cell therapy acts as a gene agnostic vision loss prevention treatment.

jCyte’s phase 2/3 clinical trial design was approved by the FDA and, jCyte states that, “Enrollment is expected to begin the second half of this year and more information will be shared when appropriate.”

What this means for Usher syndrome: jCell, this first-in-class allogeneic cell therapy, moving forward to Phase 3 brings us one step closer to a potential treatment for those with various forms of RP, including those living with Usher syndrome.

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

[View Current USH Research](#)

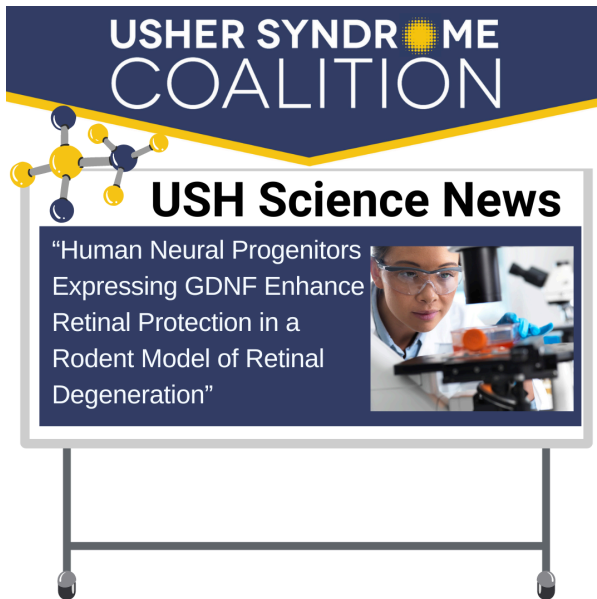
IN CASE YOU MISSED IT: SCIENCE NEWS FEATURE

Human Neural Progenitors Expressing GDNF Enhance Retinal Protection in a Rodent Model of Retinal Degeneration

October 3, 2023: Many modalities are being evaluated for the treatment of retinal degenerative diseases such as retinitis pigmentosa (RP), including stem cell therapy where dead or damaged cells are replaced by healthy ones. In a previous study, subretinal injections of human neural progenitor cells (hNPCs) and an engineered version that expresses a nerve growth factor (hNPCGDNF) successfully preserved visual function. However, these experiments were performed using mouse models showing early stages of retinal degeneration and did not translate to clinical patients in later stages of the disease.

In this study, two groups of Royal College of Surgeon (RCS) rats, which are a well-established model for RP, received subretinal injections of hNPC and/or hNPCGDNF in one eye at different time points (21-23 days and 60 days) to assess their effect on early and late stages of retinal degeneration. The remaining

eye was used as a control. At 90 days post-treatment, vision was measured and then all rats were sacrificed, and retinas were harvested and processed. Results show that subretinal injection of human neural progenitors expressing GDNF enables better visual and cellular preservation than just hNPC at both early and late stages of disease. Treatment with hNPCGDNF yielded multiple benefits, including activation of specific cell-survival pathways, reduction of oxidative stress, and promotion of cell regulation processes such as phagocytosis which improves cell health.



The image shows the USHER SYNDROME COALITION logo at the top, featuring a stylized globe icon. Below it is a banner for "USH Science News" with a molecular structure graphic. The banner contains the text: "Human Neural Progenitors Expressing GDNF Enhance Retinal Protection in a Rodent Model of Retinal Degeneration" and a small photograph of a scientist in a lab coat and safety glasses working in a laboratory.

What this means for Usher

syndrome: Previous preclinical studies demonstrated the potential of hNPC in treating RP, which resulted in an ongoing Phase 1/2a clinical trial. This new study revealed the addition of GDNF to hNPC outperformed hNPC alone for the treatment of early and late-stage RP and may become a potential therapeutic option one day.

[READ ARTICLE](#)

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

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.

ON WELL-BEING: Humor

Humor has been proven to be a valuable resource in stress management. If this is something you struggle with, don't worry, you're not alone! Fortunately, humor is a skill that can be learned, with practice.

"**Humor** can be described as a cheerful-relaxed attitude towards life or fundamental basic temperament, which becomes evident in how often a person laughs at different life situations, smiles and is happy." ([Nadja 2016](#)).

Some of the stress and anxiety that comes with a rare disease is due to the way it affects you in a social capacity. Having any degree of dual sensory loss may impede your ability to communicate, connect with others, or navigate your surroundings. Perhaps, there's also a level of social difficulty in participating in humor among peers when you have Usher syndrome (USH). For example, in a classroom environment, it may be challenging to pick up on a joke due to difficulty accessing residual hearing, background noise, speech cadence, and timing.

Parents of children with USH may also find themselves experiencing higher levels of stress, as raising a child with a disability can be emotionally, physically, and financially challenging ([Heidi, 2020](#)). People with Usher syndrome and their loved ones could benefit from intentionally including humor and laughter in their lives to help cope, and learn to embrace a more positive mindset.

It can be easy to become overwhelmed in navigating the complexities of this rare disease, and the relentless work advocating for needs to be met. Try using humor as a coping strategy for informing others about USH. It may be hard- AND- it's WORTH the work to foster a sense of humor and laugh to reduce stress. Laughter is beneficial for everyone.

Benefits of laughter

A good sense of humor won't cure Usher syndrome but it can help you manage it. On a physical level, laughter can result in relieving muscle tension, feeling lighter, and can boost your immune system.

Emotionally, humor can help you get out of your head, connect with others and manage anxiety. Using humor as a coping mechanism and emotional regulation tool may help foster a sense of control and resilience.

On the whole, incorporating a sense of humor can create a more positive self-concept, greater self-awareness, and a more positive attitude toward life.

How to foster your sense of humor:

- [Try Laughter yoga](#) - Look it up. You won't be disappointed.
- Engage and seek out humor in life through funny stories, movies, jokes, and funny people.
- Practice laughing. Reflect on how you feel after.
- Make a list of movies, shows, comedians, and comics that make you laugh. Save the list someplace it is easily accessible to you.

- Laugh about USH mishaps like stumbling over something or mishearing something.

Humor encourages a positive attitude towards life and the world around you. Learning to embrace a sillier side of life can help you find courage, peace, and joy. Life is full of potential, and you deserve to show up as the best version of yourself.

References

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- <https://www.mayoclinic.org/healthy-lifestyle/stress-management/in-depth/stress-relief/art-20044456>
- <https://www.wikihow.com/Do-Laughter-Yoga>
- <https://www.healthline.com/nutrition/laughing-yoga>

We share the research and peer-reviewed literature that offers insight into well-being: the science behind staying grounded. Fill out this [poll](#) to request a topic.

[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.

USH Life Hack of the Day

Send your USH life hacks to info@usher-syndrome.org

The [Be My Eyes](#) app can also be used for generating Image Descriptions of a photo. If a friend texts you a photo and you aren't quite sure what you're looking at, click on share photo then scroll down to "Describe with Be My Eyes" for a detailed Image Description! -USH3A iPhone user.



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