



Delivering on the promise of treatments for rare disease

July 2025
From the Desk of CEO, Ashley Winslow, PhD

Stabilize the System

Scientific progress is often imagined as singular groundbreaking discoveries—but the reality is more complex. Biology and drug development are rarely linear endeavors or large leaps, each step forward is the result of years of inquiry, consensus building, and perseverance. Though it feels slow, decades of foundational research and incremental discovery build breakthrough therapies. Importantly, this steady climb depends on sustained financial support.

For most of the last century, the US federal government has been the backbone of scientific discovery. Since NIH funding peaked in 2003 (inflation adjusted to 2023 dollars: \$48.5 billion), the lowest funding point was in 2015 which saw a 22% reduction (inflation adjusted) from 2003. This budget reduction took place over a 12 year period, with a single year reduction *never exceeding more than a 5% budget cut*. But the Trump administration's proposed 40% cut—from **\$48 billion to \$27.5 billion**—would be the largest ever, returning funding to 1997 levels within a single year.

Federally funded research has underpinned major medical innovations including gene editing, CAR-T therapies, AAV technology, and the mRNA technology that enabled COVID-19 vaccines. While industry leads late-stage development and clinical trials, it relies on the academic research funded by government dollars. Without federally funded research, there is no pipeline for new therapies.

This ecosystem—federal funding, academic innovation, and industry translation—has worked well for many areas of medicine. But for rare diseases, cracks in the system forced patients and families to take an active role. As industry lost interest in rare diseases due to small market sizes, patient communities became funders, collaborators, and drug developers.

These groups partner with academic researchers, fund early-stage projects, and even launch companies to advance promising treatments. The work is driven by urgency—and hope—but also necessity. Rare disease patients don't have the luxury of waiting for the system to change.

Now, with declining federal funding, cooling investor interest, and a biotech industry shifting away from high-risk areas like gene therapy, over-stretched patient groups are shouldering more of the burden. This is not sustainable. If the system continues to weaken, rare disease patients will suffer first.

Odylia has lived this firsthand. There isn't a week that goes by without a conversation with our patient group partners about funding cuts and the impact on our partner labs. And as significant leadership changes were made at the FDA earlier this year, the industry partners Odylia was in discussion with lost their investor base, directly impacting program timelines and milestones for our lead gene therapy. We are at a critical juncture. Patient groups and nonprofits will continue to step-up out of necessity, but the life sciences sector is at risk.

- We need to focus attention on areas of need and highest impact:
- Advocate for stable federal research funding and educate communities on the impact of current budget cuts. Medical advances are built on a system of incremental discoveries; stable funding is imperative to prevent the system's failure.
 - Create opportunities to fund the most critical science. Researchers are eager to collaborate with patient groups to continue progress.
 - Invest in tools that will have the most impact on current and future work, setting rare disease communities up for future success.

It is time to be bold, and find innovative and empathetic partners who will challenge the system. We need to align on what matters most. At Odylia, we remain committed to advancing science—for every disease and every patient.

Odylia Program Updates



Brydge Solutions Update:

Odylia is honored to work with so many amazing rare disease patient advocacy groups. This year we have partnered with the PURA Syndrome Foundation, the Smith-Kingsmore Syndrome Foundation, the Snow Foundation, and RBD Project. This summer we also began working with DLG4 Shine Foundation and Upstream Genes. All of these groups are doing incredible things for the patients they represent including: launching a patient registry, initiating biobanking efforts, drug repurposing screening, gene therapy development, funding research, and so much more.

Through the Brydge Solutions initiative Odylia partners with patient groups representing a wide range of diseases at various points in their therapeutic development journey. If you know of additional groups who could benefit from scientific and strategic partnership, please have them [email us](#).



NPHP1 Gene Therapy Program:

Together with the NPHP1 Family Foundation, Odylia is developing a gene replacement therapy for vision loss caused by mutations in the NPHP1 gene.

- Odylia has designed an AAV gene therapy and partnered with the lab of Dr. Friedhelm Hildebrandt at Boston Children's Hospital to test it on two different genetic models.
- This work follows a successful gene replacement therapy proof-of-concept conducted in the lab of Dr. Lingzhong Sun, at Southern Medical University in Guangzhou China.
- Dr. Hildebrandt's team is currently conducting the natural history, pharmacology, and efficacy studies, with a goal of having initial data by the end of 2025.
- Following the preclinical studies and manufacturing, Odylia will prepare for IND submission in 2027.

The NPHP1 Family Foundation has also assembled an eight-member scientific advisory board, with representation from Stanford University, Johns Hopkins University, the Retina Foundation, private industry and more. [Learn more here](#).



USH1C Gene Therapy Update:

- The vector design for the USH1C gene therapy program has been selected.
- Critical studies for the USH1C gene therapy program have finished and study reports and publications are in progress.
- Usher2020 and Odylia are preparing for a pre-IND meeting with the FDA to gain critical feedback on the USH1C Gene Therapy Program. A meeting is planned for early 2026.

USH1C program funding is provided by The Usher2020 Foundation and the FAUN Foundation and we have partnered with University of Tübingen, Institute of Animal Physiology and Genetics, Johannes Gutenberg University.



RPGRIP1 Gene Therapy Update:

- A new manufacturing production of OT-004 is planned for September with our partner Andelyn Biosciences, and this material will be to be used in our final preclinical safety testing.
- Work on the first of six assays has finished. We are working to secure funding to complete the remaining assays.
- Odylia plans to initiate the final preclinical safety study in late 2025 / early 2026 with our toxicology partner Virscio.

Current program funding is provided through generous donations from the RPGRIP1 community and Odylia supporters, as well as through a grant from the Foundation Fighting Blindness. This program is actively seeking clinical stage partnerships, or late stage preclinical partnerships. Please [inquire here](#) if you are interested in learning more.

Change for a CURE



Turn loose change into treatments for rare diseases. Millions of dollars of foreign currency sits in jars (and couches!) across the US and the World – currency with no value where it is. Odylia's Change for a Cure turns foreign coins and cash into donations for our mission.

- How can YOU help?
- If you have loose change lying around from a trip abroad, send it to Odylia, to be turned into a donation. [Find shipping instructions here](#).
 - Share our Change for a CURE posts on your social media so others can send in coins.
 - Contact a local coin shop or other business and ask if they will put up a collection box and/or pass out flyers to people who bring in foreign currency. [Use this flyer](#).
 - See if your place of work will let you do a coin drive to collect foreign currency.

Contact us to get flyers or collection boxes.

Meet the Odylia Team: Sharmila Vijay Title: Gene Therapy Consultant

We asked Sharmila to share a little of her history.

Tell us about your path to Odylia:
"Two years into my consulting phase as a drug developer, I was looking for nonprofits that could use my skill sets gained over 30 years of my full-time biotech career. I reached out to a past colleague of mine from the early 2000s who connected me with an Odylia Board Member. It's a small world, and I am grateful for my network, albeit small, that shares a passion for science. I deeply admire people with a similar mindset and a sense of community to make a difference for patients who would immensely benefit from potential gene therapies in development that are ably supported by Odylia."

Why is Odylia's mission important to you?
"I believe in Odylia's open model of being enablers and sharing know-how to overcome common barriers in drug development. I am truly inspired by the passion and fierce dedication of the small Odylia team of volunteers and staff. Having been part of the RPGRIP1 pipeline program for the past three years, I have had a front seat to observe their commitment. It is a simple goal of tackling the steps to make a safe and efficacious drug reach the intended target of rare disease patients in time to reverse or check their debilitating symptoms impacting vision. The mission is all the more critical to navigate with efficiency given the challenging funding environment. I continue to be hopeful as Odylia is continually connecting multiple patient advocacy groups and for-profit vendors to coalesce towards meaningful solutions for patients."



- Sharmila Vijay
- MA Molecular Biology, Cal State Univ., Fresno, CA
 - MSc Zoology, Univ. of Madras, Madras, India
 - BSc Zoology, Univ. of Madras, Madras, India

Sharmila holds two patents and has authored many published papers.

To ensure we send emails of most interest to you, please indicate your preferences

- The RPGRIP1 Gene Therapy Program
- The USH1C Gene Therapy Program
- The NPHP1 Gene Therapy Program
- Brydge Solutions
- Fundraising

Join Global Genes for the 2025 RARE Drug Development Symposium. This hands-on, expert-led event is designed to help rare disease advocates build expertise, connect with leading therapy developers and researchers, and confidently navigate the drug development process. Whether you're initiating research efforts or looking to refine your strategy, this symposium offers practical insights to accelerate progress in research strategies and activities.

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