Odylia THERAPEUTICS

Delivering on the promise of treatments for rare disease

**November 2025**
**From the Desk of CEO, Ashley Winslow, PhD**

## A Letter of Thanks

"It takes a village" is a familiar phrase in the rare disease community. When the stories of success are retold it is usually the story of big personalities and big wins. It is often hard to see the hundreds of people behind each incremental victory, the inevitable setbacks, and the perseverance to keep moving programs forward. Behind every "big win" are hundreds of people whose steady efforts, years of learned expertise, creative problem-solving, and resilience make progress possible. In this season of thanks, on behalf of everyone at Odylia, I want to say a heartfelt "Thank you" to our village.

At Odylia, we're advancing three gene therapy programs for rare vision loss disorders and working with nearly 20 patient groups through Brydge Solutions. Even just one program—like Odylia'sgene therapy for RPGRIP1-associated retinal dystrophy (OT-004)—brings together the dedication of more than 100 people.

From the start, Odylia has advanced the RPGRIP1 program building on foundational research from academic labs, partnering with industry, and continuing the work even when many shifted away from rare disease gene therapy. With the help of generous donors, volunteers, and partners, we've navigated early manufacturing and key regulatory milestones. The RPGRIP1 community has been a critical source of support, raising more than $500,000 to keep the program moving. That grassroots commitment was instrumental in helping us secure an additional $1.5 million award from the Foundation Fighting Blindness in 2024 to fund vector manufacturing and toxicology studies, which are on track for early 2026 completion.

Andelyn Biosciences has been a critical partner in this journey. Together with their passionate team, we've developed a manufacturing process for the novel AncBD capsid and built a lasting partnership that will speed future programs, saving both time and resources on the way to the clinic.

At the same time, we deepened engagement with the FDA, guided by two volunteer boards—the RPGRIP1 Patient Advisory Board and Clinical Advisory Board. These 15 dedicated individuals helped shape a clinical trial plan that reflected both scientific understanding and the lived experiences of patients and caregivers. When we brought it to the FDA, the design was approved without pushback—a testament to the thoroughness and collaboration of these boards, and enabling us to keep the patient and caregiver voice at the center of our work.

We've also formed strong partnerships with Virscio, who is conducting our toxicology studies, and Pharmaron, who supports potency (activity testing). Both have been strong strategic partners with deep expertise, both helping us overcome challenges unique to nonprofit drug development. And we are actively adding to this list of partners during this final 6-9 month preclinical push for the RPGRIP1 program. We look forward to growing our village as we advance to clinical trials.

Every program at Odylia is a true team effort. Our staff, Board, Patient Group and Communities, and Advisors work hand-in-hand with external collaborators—each bringing an average of 5-15 new people and their own expertise and passion to the program. In the past three years alone, at least 75 people have directly contributed to the RPGRIP1 program, and more than 100 have played a role since its beginning.

We are deeply grateful for everyone's time, expertise, and perseverance. Thank you to our community, to our past, current, and future partners. Together, our village is building a new model for advancing treatments for rare diseases—you are a part of that story, bringing hope to the patients and families who are waiting.

Thank you!

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Planning to order holiday cards or personalized gifts this season. Visit **MINTED** for a great selection and an awesome discount. Plus a donation will be made to Odylia. Use the code: FUNDRAISEODYLIA

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## Odylia Program Updates

Odylia | NPHP1 Family Foundation

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.

- The natural history study in the NPHP1 mouse model is progressing in the laboratory of Dr. Friedhelm Hildebrandt at Boston Children's Hospital, with data anticipated by the end of 2025.
- Odylia and the NPHP1 Family Foundation have engaged Powered Research to conduct an IND-enabling pharmacology study.
- Dosing for the four-week pharmacology study was initiated at Powered Research at the end of October.

Current program funding is provided through our partnership with the NPHP1 Family Foundation.

USH1C Gene Therapy Update:

- Odylia Therapeutics will begin manufacturing activities in 2026 for the USH1C gene therapy program.
- In collaboration with University of Tübingen, Johannes Gutenberg Universität, Institute of Animal Physiology and Genetics, and Universität München our manuscript titled *"Subretinal injection volume correlates to persistent outer retinal thinning in the pig eye"* has been accepted for publication in *Investigative Ophthalmology & Visual Science*.
- Usher Syndrome Odylia are working toward a pre-IND meeting with the FDA to ensure a clear regulatory path for the USH1C gene therapy program. The meeting is planned for the first half of 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:

- We have had a very successful manufacturing run of OT-004 (the RPGRIP1 gene therapy) at Andelyn Biosciences which will be used in the upcoming safety study.
- The safety study protocol for the RPGRIP1 program is in the final stages of development and review, in collaboration with Virscio.
- Odylia plans to initiate the final preclinical safety study in early 2026 with our toxicology partner Virscio.
- The next RPGRIP1 Q&A is scheduled for December 10, 2025 at 1:00pm ET. Please register at the link below.
- In September, we launched the latest round of the RPGRIP1 Fundraising Campaign. So far, we have raised $80,000 of our $140,000 goal. Please consider making a donation **HERE.**

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.

BRYDGE SOLUTIONS
Odylia

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, the DLG4 Shine Foundation, Upstream Genes, and PBD Project. We have immensely enjoyed getting to be a part of their village as each group makes progress towards better supporting their communities, engaging with scientists and clinicians, and developing therapeutics. A few highlights of the progress of these groups over 2025 include: the generation of a new animal model, development of a behavioral experiment for a drug repurposing screen, initiating a new patient registry, collection of patient samples for biomarker analysis, multiple family conferences, outreach to dozens of interested new research collaborators, and the running of multiple successful medical and scientific advisory board meetings.

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**.

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Join us to hear the latest news about the RPGRIP1 Gene Therapy Program including an update on manufacturing, and have your questions answered by our scientists.
**Please Register for the Community Q&A HERE**

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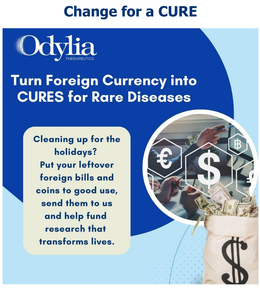
## Change for a CURE




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 bills 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.

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