



Delivering on the promise of treatments for rare disease

March 2026

From the Desk of CEO, Ashley Winslow, PhD

Strengthening Our Collective Impact

On February 25th in Washington DC, Odylia welcomed an incredible group of people for our annual Rare Disease Awareness Day Event, bringing together patient groups, researchers, drug developers, policy leaders, and regulatory scientists. I wanted to share my thoughts from the event and reflect on the strengths and challenges facing the rare disease community.

Recently, the rare disease field has faced significant headwinds. Funding constraints, increasing regulatory uncertainty, and shifts in the broader economic landscape have made paths to the clinic more difficult to navigate. Despite these challenges, we have begun to see the 'silver linings' appear, and we are hopeful for the meaningful change it foreshadows.

Some of those shifts are substantial, such as the reauthorization of the Priority Review Voucher and new FDA guidance on individualized therapies, and they create openings for programs that had previously stalled. These are not trivial wins and they didn't happen by accident. They are the result of years of persistence from this community, of individuals and organizations raising their voices, sharing their stories, and advocating for change. Together, that unified voice is impossible to ignore. And that voice will continue to be the catalyst for the changes that need to happen for a more equitable future for rare disease patients. Time and again it is those community stories that drive scientific inquiry, regulatory flexibility, and policy change.

Being a part of that spirit of collaboration is one of my favorite things about this community and what makes the rare disease community so extraordinary. There's a shared instinct to help one another and to pass along hard-earned lessons, to break down silos, and to ensure the next group can move faster and go further. That generosity fuels innovation and sets the rare disease field apart from traditional drug development.

At the same time, we know that momentum requires continued action. Policies evolve because people demand change. Progress depends on all of us continuing to show up, speak up, and share what we're learning. Knowing that our actions have elicited change strengthens our resolve to continue to push for better.

So, wherever you are, whether in the lab, the clinic, a patient organization, or a regulatory office—your voice matters. Share your challenges, your breakthroughs, and the insights that could help others. Celebrate the champions who have moved your work forward and the progress you've achieved together.

At Odylia, our goal is simple: to help forge new paths, strengthen this remarkable community, and amplify the collective voice that drives change.

Thank you for being part of it.

Odylia Program Updates



Brydge Solutions Update:

Odylia is honored to collaborate with so many incredible rare disease patient advocacy groups. So far this year we continue to work with the Smith-Kingsmore Syndrome Foundation, the DLG4 Shine Foundation, and Upstream Genes. We are also excited to newly partner with the MED13L Foundation and Save Sight Now. We're proud to support these communities as they advance efforts to better serve patients and families, engage with scientists and clinicians, and drive the development of new therapeutics.

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:

- Our research partner, Dr Friedhelm Hildebrandt at Boston Children's Hospital and his lab have finished collecting data on the natural history of vision loss in the NPHP1 mouse.
- We have confirmed that the vector drives dose-dependent expression of the NPHP1 transcript and protein through our partners at Powered Research. We will focus on confirming appropriate localization of the NPHP1 protein in the mouse model and assess early indicators of efficacy.
- We are piloting cell-based functional testing of the two vector designs through a partnership with the Zhen Liu lab in Hong Kong.
- Large scale clinical manufacturing activities are scheduled to begin in 2026.

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



USH1C Gene Therapy Update:

- Odylia's USH1C Gene Therapy Program was awarded a 2026 TRxA (Translational Therapeutics Accelerator) BRIDGe award from the Critical Path Institute to support early manufacturing and regulatory engagement.
- We will begin manufacturing activities in 2026 for the USH1C gene therapy program.
- A pre-IND meeting with the FDA is planned for 2026 to ensure a clear regulatory path for the USH1C gene therapy program.
- With a lack of an USH1C animal model with a visual phenotype, we are considering options for evaluating functional effects in patient-derived cell lines in collaboration with Kerstin Wolfrum (Univ. Mainz).

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



RPGRIP1 Gene Therapy Update:

- The final preclinical safety study for the RPGRIP1 program is underway at Virscio. Preliminary data will be available this summer on the evaluation of safety of OT-004 at three different doses and the biodistribution of the vector.
- Dose confirmation and device compatibility studies are planned for this year.
- Key clinical-stage activities, including early-phase trial preparations, are planned for this year.
- This program is actively seeking clinical stage partnerships, or late stage preclinical partnerships. Please [inquire here](#) if you are interested in learning more.

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.

Rare Disease Awareness Day



On February 25th, almost 60 people gathered in Washington, DC for Odylia's Rare Disease Awareness Day event. One of Odylia's goals is to elevate the collective voice of rare disease patients so in addition to bringing awareness to rare diseases in general, the evening included discussions around innovative approaches to overcoming obstacles in the drug development process. Meaningful group discussions were led by subject matter experts and rare disease patient advocates about the following topics:

- Patient Groups Driving Discovery and De-risking Therapeutics
- The Patient Voice in Drug Development
- Innovative Business Models and Partnerships

We are grateful to our Major Sponsors Andelyn and Upstream Genes as well as other sponsors Dash Bio and Virscio for helping to make the event a success.

Meet the Odylia Team: Wendy Erler, Board Member

Odylia welcomes Wendy Erler as the newest member of the Board of Directors. She brings more than two decades of leadership experience across patient advocacy, rare disease strategy, medical affairs, and commercialization in the biopharmaceutical industry.

Wendy currently serves as Senior Vice President of Patient Affairs at Sarepta, where she is a member of the executive leadership team. In this role, she bridges customer and company strategy to ensure patient-centricity across the organization, embedding the patient voice throughout the product lifecycle, from clinical trials to commercialization. She is widely recognized for her ability to build collaborative cultures and translate patient insight into meaningful action across complex organizations.

Odylia will benefit greatly from Wendy's authentic commitment to patients and her ability to connect communities with critical resources and services. Erler has built strong partnerships across advocacy organizations, healthcare providers, policymakers, and industry stakeholders.



Wendy Erler

- BA Miami University
- MBA from Saint Joseph's University

"I'm honored to join the Odylia Therapeutics Board of Directors," said Erler. "Odylia is tackling some of the most difficult challenges in rare disease by creating new business models to develop treatments for conditions that might otherwise be overlooked. I'm excited to support the organization's mission and help ensure that patient perspectives remain central to its work."

