



Delivering on the promise of treatments for rare disease

April 2025
From the Desk of CEO, Ashley Winslow, PhD**In times of uncertainty, the nonprofit model proves agile**

Odylia is no stranger to upheaval and uncertainty. Just two years after our founding, we—along with the rest of the world—were thrust into the COVID-19 pandemic. In 2020, our lead program was partnered with PTC Therapeutics. While PTC drove preclinical testing, we continued to advise the program, working to minimize the barriers that all rare disease treatments face, as well as the additional challenges created by COVID-19. Like many labs worldwide, we encountered disruptions due to social distancing, work-from-home mandates, supply chain breakdowns, shifting research priorities, and restricted sharing of tools and samples between researchers. At the same time, investor interest in rare disease and gene therapy became volatile. While startup investments initially surged during the early pandemic, funding quickly shifted away from rare disease drugs and gene therapies. As COVID persisted, many companies divested from these areas, leaving countless programs shelved or terminated. Our own lead gene therapy program, OT-004, for RPGRIP1 was dropped in this wave. The program returned to Odylia at the beginning of 2021, without a funding partner. By 2023, the biotech investment bubble had largely burst, leading to further uncertainty across the life sciences sector, widespread layoffs, and portfolio shifts. As a young organization dependent on fundraising and licensing, it was an uncertain time.

Industry volatility underscored a growing need for guidance among patient groups, academics, and industry partners. This shift provided Odylia with an opportunity to serve more people through our mission. In 2020, we not only continued to support our lead program but also expanded our gene therapy portfolio by adding the USH1C program. We also began offering our expertise as a fee-for-service, providing reduced pricing for nonprofits. By 2021, we formally launched the [Brydge Solutions](#) initiative, expanding our nonprofit biotech model to include fee-for-service. Importantly, Brydge Solutions has strengthened our ties within the rare disease community, diversified our revenue streams, and broadened the impact of our mission.

During the pandemic, we reduced our reliance on any single revenue source, making Odylia more resilient to market fluctuations. Our nonprofit model has evolved into a mixed-revenue structure, incorporating charitable donations, licensing revenue, fee-for-service, grants, equity compensation, and in-kind contributions. By blending traditional nonprofit and for-profit business strategies, we have expanded our partnerships and better served the rare disease community. This diversified approach has increased our financial stability during periods of instability.

Today, as political and economic uncertainties continue, we are seeing further challenges directly impact our partners and our work. Government agencies and universities have enacted hiring freezes, deprioritizing critical rare disease research. Restrictions on information sharing from federal agencies have resulted in wasted time and resources. Funding freezes on previously awarded grants have stalled research, while restrictions on scientific language have hindered effective communication and funding. These shifts threaten to undo decades of scientific progress, placing nonprofit organizations at risk and leaving the life sciences sector grappling with long-term consequences. These changes impact our partners, our work, and our ability to rapidly move programs forward.

Despite these challenges, Odylia remains steadfast in our mission. Our business model is built to be resilient, allowing us to adapt and continue supporting the community. Unlike many companies, we have not shelved programs or laid off staff. Instead, we have expanded our gene therapy portfolio to three programs, collaborated with more than 15 rare disease partners, been nominated for the Amgen Prize for an innovative tech solution, grown our team and Board of Directors, and initiated three groundbreaking partnerships set to be announced soon.

As a mission-driven nonprofit, we remain agile in the face of market instability. We continue to accelerate drug development for rare diseases while pioneering an innovative business model and tackling systemic challenges. We have proven that progress is possible—even against the odds—thanks to the unwavering support of our incredible community.

Odylia Program Updates

- Usher2020 and Odylia will begin preparation for regulatory interactions with the FDA to gain critical feedback on the USH1C Gene Therapy Program, with an expected meeting planned for early 2026.
- Data analysis and finalization is underway for a two-year study testing two USH1C gene therapy vector designs. A publication and presentation of these findings are in progress.
- A study evaluating the impact of delivering a gene therapy in different volumes to the retina in the USH1C genetic model is underway, with data analysis expected in the next 2-3 months.

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.



Odylia is excited about new partnerships in 2026 and those that have expanded this year, including:

- The Smith-Kingsmore Syndrome Foundation
- PURA Syndrome Foundation
- NPHP1 Family Foundation
- The Snow Foundation
- PSD Project

We are looking forward to working with these groups.

Odylia is thrilled to be attending the Ultragenyx Bootcamp this April in Boston. The bootcamp is an amazing opportunity for patient advocates to learn about the drug development process.

Through the Brydge Solutions initiative, Odylia partners with patient groups, academic institutions, and industry to accelerate therapeutic development for rare disease.

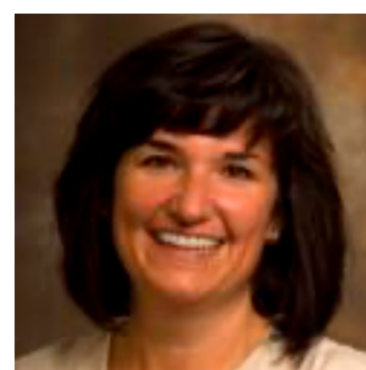
- Odylia received positive feedback from the FDA on the RPGRIP1 gene therapy program (OT-004) following a Pre-IND meeting in January of 2025.

- Manufacturing material from the scale-up production at Andelyn Biosciences, is being used to develop the potency assay at Pharmaron.

- A new manufacturing run is planned for this summer to produce a batch of OT-004 that will be used in toxicology testing

- Odylia recently hosted a Q&A for the RPGRIP1 community. Follow [this link](#) to view a recording.

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.

Meet the Odylia Team: Heather Greene
Director of Development

We asked Heather to share a little of her history.

Tell us about your path to Odylia:
"During my undergrad years, I knew I wanted a career in the nonprofit sector. I have been fortunate to realize my goal and have worked in the field for over thirty years, first with the March of Dimes and then with Special Olympics Georgia. I truly believe in the difference volunteers and nonprofits can make in every corner of the community to make life better. Odylia epitomizes Margaret Mead's famous quote, "Never doubt that a small group of thoughtful, committed citizens can change the world; indeed, it's the only thing that ever has." It is wonderful to be a part of the team and it has been great getting to meet the families, volunteers, and donors who make our work possible."

Why is Odylia's mission important to you?
"If not us, then who? The work Odylia is doing to advance rare disease treatments will make a drastic difference for rare disease patients. Moreover, it will change the way drugs are developed for rare diseases. The entire model will shift and, hopefully, will mean more and more patients can benefit from treatments that reach clinical trials. Odylia's mission will make a meaningful and lasting impact on thousands of patients and their families."

Heather Greene

- B.A., Sociology and Political Science, University of Michigan
- M.A., Nonprofit Organizations, University of Georgia

Heather resides outside of Atlanta, GA with her family. She volunteers with the Boy Scouts, the High School Band and Lacrosse Team, and her church.

MINTED Benefits for You & Odylia

MINTED FUNDRAISING PROGRAM

Save 20% on unique gifts for Moms, Dads & Grads

USE OUR CODE
FUNDRAISEODYLIA

Use our unique code to shop, and Minted will donate 10% back to us



Odylia staff members will be attending and presenting at a few conferences this summer. Let us know if you are nearby.

April 15-17 in Boston, MA
May 4-8 in Salt Lake City, UT
May 27-29 in Las Vegas, NV

June 10-12 in Bethesda, MD
June 16-18 in New York, NY
June 27-29 in Dallas, TX

Contact Us

As a nonprofit, Odylia is dependent on donations from individuals, companies, foundations, and people like you. Please consider making a contribution today!

[Donate Here](#)

