Odylia THERAPEUTICS

Delivering on the promise of treatments for rare disease

October 2024
From the Desk of CEO, Ashley Winslow, PhD

Accelerating Progress: How Odylia is Expanding Access to Rare Disease Therapies

At Odylia, we believe that accelerating therapeutic development for rare diseases isn't just about scientific breakthroughs—it's about expanding access to the resources and information needed to make those breakthroughs happen. The drug development process, particularly for rare diseases, has long been a challenging and often inaccessible journey. Odylia's mission is to change that, and we're making strides toward transforming the landscape for rare diseases.

Founded with deep expertise in drug development, Odylia was built on a foundation of strategic insight and a passion for helping those with rare diseases. In just two years from the founding of Odylia, we had two gene therapies in development for vision loss disorders. But we didn't stop there, as we began fielding countless questions from patient groups looking to develop their own therapies—often without the backing of biotech or pharma—we saw an opportunity to broaden our impact.

In 2020, we launched Brydge Solutions, an initiative designed to help patient groups navigate the complex and evolving drug development process. Leveraging our experience from our internal pipeline programs, we made the process accessible to the broader rare disease community, providing the tools and guidance needed to help accelerate the development of life-changing therapies. What started with two programs quickly grew, and we have now worked with more than ten different patient groups and launched two additional programs aimed at improving the overall drug development process for patient groups.

We're always looking for ways to eliminate barriers to drug development, and one of the ways we've done that is through the **Odylia Library**. This resource hub offers free access to critical information for patient advocacy groups and others working in rare disease therapeutics. Through the Odylia Library, we share scientific Landscape Analysis Reports and a growing collection of videos that explain key aspects of the drug development process, empowering groups to make informed decisions and move forward with confidence. And we're always expanding. If you have a topic you'd like to see covered, feel free to reach out to us at info@odylia.org.

This year, we took another major step forward with the announcement of the **Odylia Collective** in April. This exciting new initiative centralizes the vendor marketplace for drug development, making it easier for patient groups to find critical services through one streamlined, searchable platform. Both The Odylia Library and The Odylia Collective are open-access tools, ensuring that the largest possible audience can benefit from the knowledge and resources we've built. These initiatives directly feed into our mission of accelerating therapeutic development and expanding access for the rare disease community.

In just five years, Odylia has grown beyond the term "nonprofit biotech." In addition to developing new rare disease treatments, our work now encompasses consulting, portfolio management, education, resource building, and technology solutions—all with the goal of breaking down the biggest barriers standing in the way of transformative medicines. We're not just advancing treatments for a handful of diseases; we're forging a new path and business model that empowers the entire rare disease community. We are broadening access to drug development and we hope others can share in our learnings.

At Odylia, we're tackling the most critical roadblocks in the path to therapies for patients with rare diseases. Together, we're making the impossible, possible—one step at a time.

Stay tuned for what's next as we continue to accelerate progress and expand access for those who need it most!

Odylia Welcomes Elizabeth Attias as the newest member of the Board of Directors

Click here to learn more

Odylia Program Updates

- Odylia expects feedback from the FDA on the RPGRIP1 gene therapy program (OT-004) through a Pre-IND meeting in the first quarter of 2025.
- Our manufacturing partner, Andelyn Biosciences, has completed a scale-up run of OT-004 to test how larger batch manufacturing of our gene therapy performs.
- Analytical testing is underway of this scale-up batch to determine if it will be used for our final toxicology study.
- The Catalyst Campaign will raise much needed funds for the RPGRIP1 Gene Therapy Program for further analysis of the current vector being produced. To ensure the program can reach clinical trials as soon as possible, we must continue to raise funds for each phase. For information and to contribute to the campaign, **visit our site**.

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.

- Usher2020 and Odylia are exploring regulatory engagement in 2025 to gain critical feedback on the USH1C Gene Therapy program to-date.
- Data processing and finalization is underway for a two-year study testing two USH1C gene therapy vector designs. We are discussing with our collaborators a potential publication and presentation of these findings.
- We are currently evaluating the impact of delivering a gene therapy in different volumes to the retina in the USH1C genetic model system. The interim time point for this study will take place in November.
- We are seeking a new dynamic QA specialist to join our team. Please forward any inquiries to **jklein@odylia.org**.

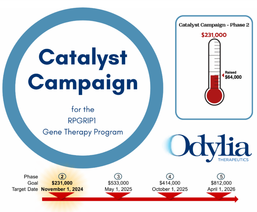
USH1C program funding is provided by The Usher2020 Foundation and the FAUN Foundation and we have partnered with University of Tübingen, Institute of Animal Physiology and Genetics, Johannes Gutenberg University.

BRYDGE SOLUTIONS
Odylia

Odylia is currently working with three Brydge Solutions partners, JEM Therapeutics' Jordan Avi Ogman Foundation, the Smith-Kingsmore Syndrome Foundation and the NPHP1 Family Foundation. We are also excited to announce a new partnership beginning this month with the PURA Syndrome Foundation (PSF). We will be working with the PSF to advance their strategic planning for therapeutic development.

Please **check out our video series** that aims to address many of the common questions and roadblocks that rare disease patient advocacy groups face.

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



Exciting New Ways to Fundraise for a Cure

MAMA B's Pecans

Odylia is piloting a new fundraiser with Mama B's. You can sell bags of flavored pecans to friends and relatives (they make great gifts for teachers and neighbors too.) **Contact us** to get an order form and more details.

Change for A Cure

Turn loose change into treatments for rare diseases. Millions of dollars of foreign currency is stored in homes across the US and the World - currency with no value where it is.

Our program **Change for A Cure** turns foreign coins and bills into donations for Odylia. If you have loose change or cash lying around from a trip abroad, consider sending it to Odylia, to be turned into a donation to further our mission.
Click here to learn more.

Meet the Odylia Team: Jenny Klein

Manager of Operations & Program Management

We asked Jenny to share a little of her history.

Tell us about your path to Odylia:
"I received my masters degree in physiology from North Carolina State University, and during that time I interned for a local biotech company discovering and developing therapies to treat rare diseases. Upon graduation I accepted a full time position with that company where I used AI to discover novel and repurposed compounds that could be used to treat rare disease via chaperone therapy. I already had an extensive background in rare disease research, particularly lysosomal diseases because I had previously worked for a nonprofit, the National MPS Society, where I'm also a member. Once in biotech my interests quickly turned to learning how to navigate the drug development landscape and develop gene therapies after founding the Mucolipidosis Collaborative Research Network and joining the board of ISMRD, the International society for Mannosidosis and Related Diseases. In both these instances bringing treatments to patients is of the utmost importance. My nonprofit background and biotech experience converged when the opportunity arose to work at Odylia Therapeutics where I get to bring my learnings and skill set to others through our internal pipeline and Brydge Solutions program."

Why is Odylia's mission important to you?
"In 2001, I myself was diagnosed with a rare lysosomal disease, Mucolipidosis type III. I grew up attending scientific and family conferences and gaining extensive knowledge in the field of lysosomal disease and broader drug development landscape. I want to share the learnings and expertise I've gained over the years with the broader rare disease community and make this knowledge accessible to families and patients who need it most."

Jenny Klein

- B.A., Psychology, North Carolina State University,
- B.S., Molecular Biology, North Carolina State University,
- MS., Physiology, North Carolina State University

Jenny is originally from Michigan and now resides in Raleigh-Durham North Carolina. She's a certified Yoga teacher in Vinyasa and loves spending quality time with her three pups and fiance.