



IMPACT REPORT

Transforming Drug Development
for Rare Diseases

odylia.org

WE ARE A FORCE FOR CHANGE

Odylia is dedicated to accelerating treatments for rare diseases. Through a nonprofit biotech business model, Odylia develops transformative therapeutics while working hand-in-hand with patient groups to advance their drug development efforts. We are intent on making the drug development process accessible to rare disease groups. While it may seem straightforward, the reality of it is not.

The limitations of existing business models hinder for-profit biotech and pharmaceutical companies from pursuing viable treatments for rare diseases. While these companies are eager to make a positive impact, they must generate a significant financial return on investment (ROI). Odylia is not constrained to a model that generates ROI, enabling us to commit to advance therapeutics that are safe and effective, rather than commercially viable.

By transforming the existing model, we can convert rare diseases from life-limiting conditions into treatable or even curable ones, ultimately redefining what is deemed possible.



A DIFFERENT KIND OF NONPROFIT

Odylia Therapeutics is delivering life-changing therapies to patients often overlooked by traditional drug development.

We drive therapeutic advancements through two core initiatives: The Odylia Pipeline and Brydge Solutions. The Odylia Pipeline includes three gene therapy programs currently in development. Through Brydge Solutions, we offer our drug development expertise to the broader rare disease community, enabling progress through flexible and collaborative partnerships.

At Odylia, we measure our success through shortened timelines, lower costs, and fewer stalled or terminated programs. Rare disease programs need more than just scientific expertise, they need dedicated champions who will propel them to clinical trials.

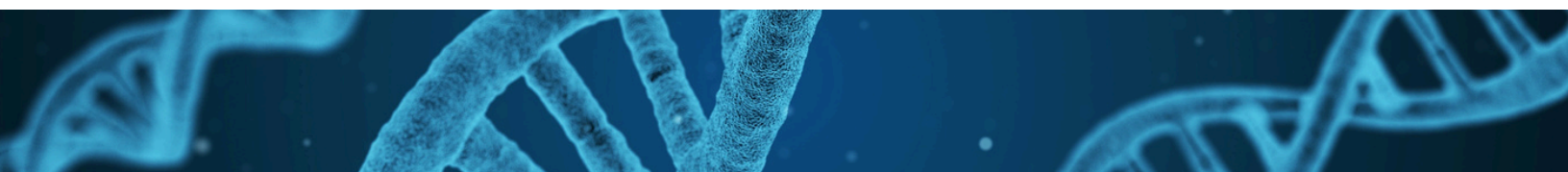
MISSION

Odylia accelerates therapeutic development for rare diseases.

VISION

We are prioritizing a future in which drugs are developed for every rare disease based on safety, efficacy, and the available technology, regardless of commercial viability.

... A DIFFERENT KIND OF BIOTECH



RECOGNITION & MILESTONES

Impact for rare disease patients in just 7 years

- RPGRIP1 pipeline program (OT-004) receives *Rare Pediatric Disease & Orphan Drug Designation* from the FDA (2021)
- Official launch of Brydge Solutions Initiative (2021)
- Launch of the Odylia Library (2023)
- Top-Five Finalist for the *2024 Amgen Prize*, Concordia Summit Presentation (2024)
- *The Brint Family Translational Research Program* recipient from the Foundation Fighting Blindness (\$1.5 million grant for RPGRIP1 Gene Therapy Program) (2024-2026)
- Advancement of Odylia's RPGRIP1 Gene Therapy Program (OT-004), with positive FDA feedback in Odylia's first Pre-IND meeting with the FDA (2025)
- Official announcement of third pipeline program: advancing hope for people with vision loss caused by NPHP1 mutations (2025)
- Official partnership with Comend to expand Odylia's work empowering Patient Research Groups (2025)
- Odylia mission video received *Award of Distinction* from The Communicator (2025)



Visit The Odylia
Library



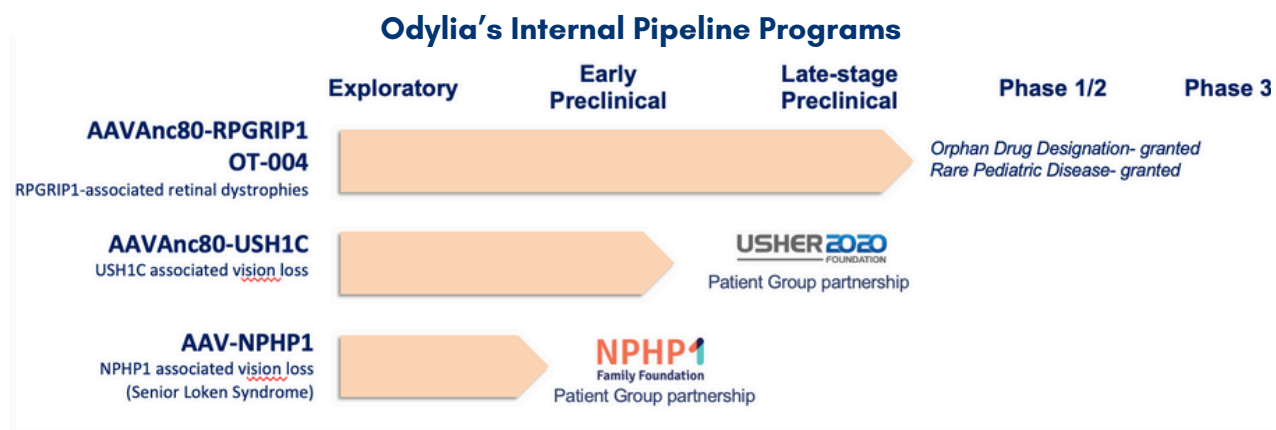
Watch the Odylia
Mission Video

ODYLIA'S PIPELINE

Odylia's internal pipeline is strategically focused on the development and delivery of gene therapies to treat the underlying genetic cause of three rare inherited retinal disorders. Each program is built in close partnership with the patient communities we serve.

We believe collaboration is the catalyst for progress. Each of our programs brings together a dedicated network of partners — from families and advocacy groups to researchers, foundations, and industry allies. This unified, cross-sector approach breaks down the traditional silos of drug development, enabling us to share knowledge, accelerate timelines, and maximize every dollar invested.

What sets Odylia apart is our ability to navigate complexity with confidence. Our team brings together deep scientific expertise, operational precision, and the lived experience of those directly impacted by rare disease. We lead with empathy and strategy — designing streamlined development plans that reflect both the urgency of patients' needs and the realities of a resource-constrained environment.



BRYDGE SOLUTIONS

Through the Brydge Solutions Program, Odylia streamlines the path from early therapeutic discovery to clinical trials for our partner patient groups who are investing in drug development directly. Utilizing our expertise in rare disease research, drug development, and program management, we empower our partners to embrace alternate approaches that will accelerate their progress. Each year, the number of groups we support continues to grow—but demand for our help far exceeds our current capacity. Additional resources are essential to meet this need and expand our impact. We are committed to making a meaningful difference for rare disease patients.

Odylia's Growing Network



Mike Kaplan, MD

President & CEO, JEM Therapeutics

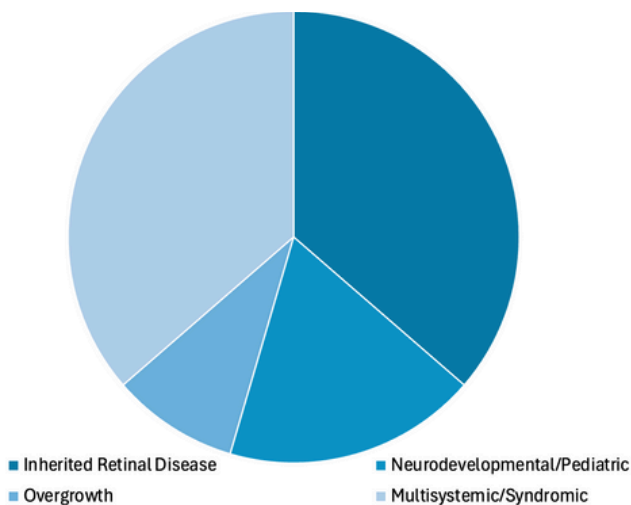
Our goal is to accelerate progress as quickly as possible so we sought a partner who had the depth of expertise to provide guidance on scientific strategy, but who could also give us insights into the financial and operational aspects of our company's growth. But what really stood out about Odylia was their accessibility and personal touch. They are as committed as we are to rare disease patients and that's a quality that we highly value and is critical to our success.

EMPOWERING PROGRESS

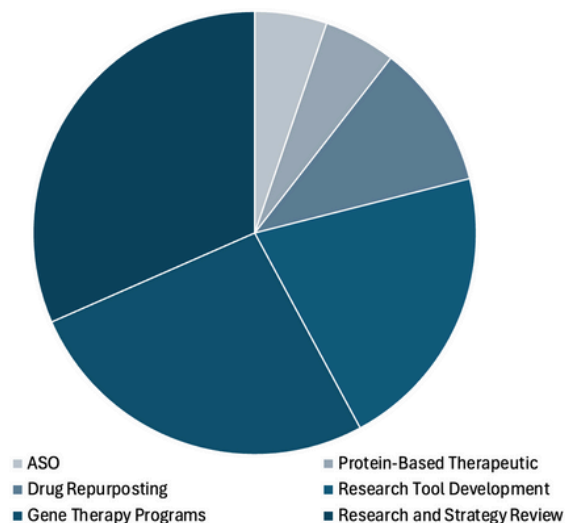
Working closely with patient groups, the Brydge Solutions program provides direct access to the drug development process and expanded organizational capacity. We help groups focus on prioritizing development of therapies that are safe and effective. We offer them tools to identify and prioritize what matters most to their communities: to advance the most promising therapies and to focus their limited resources on the science that holds the greatest potential impact. They are the next generation of rare disease drug developers.

Brydge Solutions: Disease Areas & Therapeutic Technologies Portfolio

Disease Area



Therapeutic Technologies



Kristen Davis

Executive Director,
CLOVES Syndrome Foundation

Having a child with a rare disease means needing to become an expert in drug development. But there are so many roadblocks and things to learn. Our partnership with Odylia's Brydge Solutions helped us better understand the science of CLOVES Syndrome and gave us a fresh perspective on therapeutic options and potential collaborators for the future.



Allison Kaczinski

President,
SATB2 Gene Foundation

Odylia, through Brydge Solutions, really helped the SATB2 Gene Foundation focus and start moving in the direction we need to be moving in. Their landscape analysis broke everything down into workable goals in language that could be shared with our communities.

EXPANDING ACCESS

Odylia believes in empowering others through information sharing. We build free or affordable resources for the benefit of the broader rare disease community.

The Odylia Library



A publicly accessible library to help drug development programs locate the most recent research and studies created by Brydge Solutions.

Odylia Youtube Channel



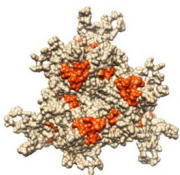
Educational videos, community updates (Odylia's pipeline programs), and other resources from creators in the field.

Comend (previously, The Odylia Collective)



A centralized marketplace and project and asset management hub to make essential resources more accessible for patient groups and rare disease researchers.

Anc80 Access Program



Odylia has exclusive rights to the Anc80 AAV vector for use in developing gene therapies to treat rare and ultra-rare retinal diseases. Anc80 was discovered in 2015 in the lab of Luk H. Vandenberghe, PhD, at Massachusetts Eye and Ear, and can effectively transduce the retina, central nervous system, muscle, liver, heart muscle, and cells of the inner ear, to name a few. Odylia is developing tools to enable the use of the Anc80 capsid for therapeutic delivery of DNA, and is developing a unique access model to ensure non-profit patient groups can utilize Anc80 for their drug development programs.

ODYLIA BY THE NUMBERS

Odylia continues to expand its reach, sharing resources and knowledge. Our vision includes increasing access and expertise across the rare disease ecosystem.

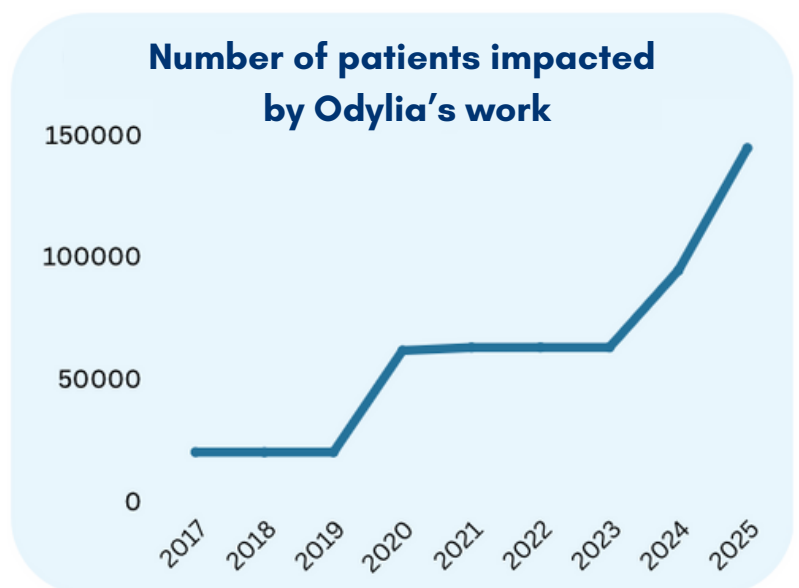
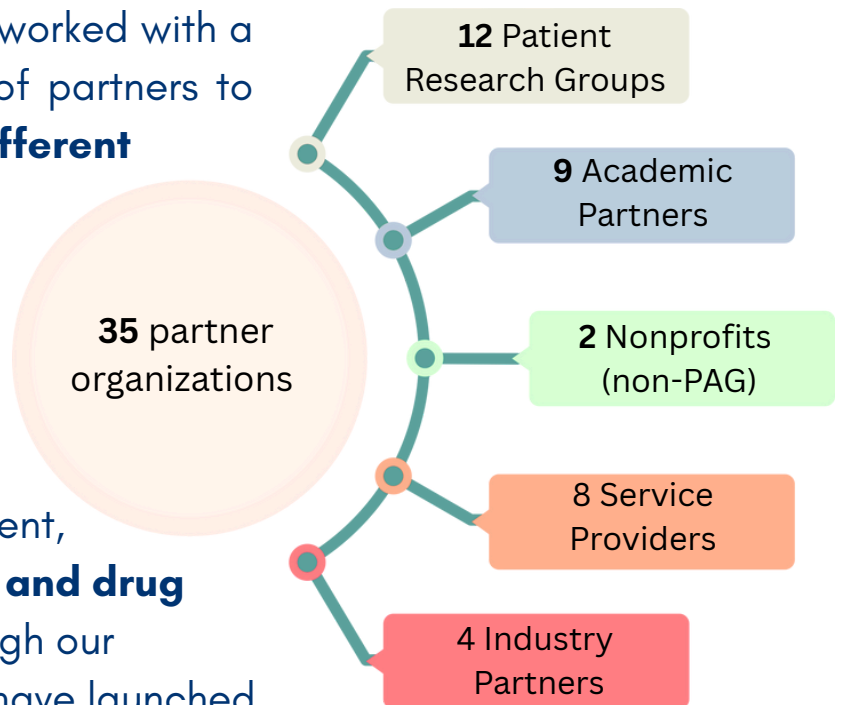
Since its inception, Odylia has worked with a diverse and growing network of partners to further its mission, serving **14 different rare disease communities**.



The Odylia pipeline has **3 gene therapies** in development, and has advanced **11 research and drug development programs** through our Brydge Solutions partners. We have launched **4 initiatives** that expand access to free or affordable resources for the broader rare disease ecosystem.

As of 2025, Odylia's mission has the potential to change the lives of more than **140,000 rare disease patients worldwide**.

With your help, we can positively impact the lives of those with rare diseases left behind by the commercial drug industry.



TURNING HOPE INTO REALITY

Odylia is driven by a singular purpose: to turn passion into impact for rare disease patients. Every breakthrough we achieve and every patient group we support brings us closer to life-changing treatments.

Companies, individuals, foundations, and investors can be a part of the solution. Together we can build a new path to bring treatments to those in need. Odylia is addressing critical challenges to ensure treatments are developed for rare diseases.

Curing rare diseases transforms lives, restores hope, and reshapes the future of medicine. Over 400 million people worldwide are affected by a rare disease – nearly half are children. The need is urgent.

The impact is profound: healthier futures, stronger communities, and a world where no disease is too rare to matter.

We asked our partners what a treatment means to them:

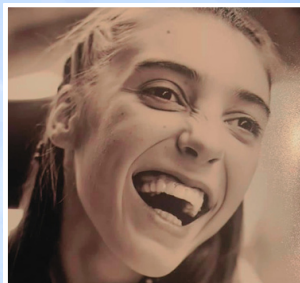
What does a treatment mean to you?



*a treatment keeps Davidson
playing baseball*

NPHP Family Foundation **Odylia** THERAPEUTICS

What does a treatment mean to you?



*improved quality of life, lasting solutions,
& more of these beautiful smiles!*

PURA Syndrome FOUNDATION **Odylia** THERAPEUTICS

What does a treatment mean to you?



*less worry, less pain,
and more joy!*

Smith-Kingsmore SYNDROME FOUNDATION **Odylia** THERAPEUTICS

If Not Us, Then Who?

At Odylia, patients are at the center of everything we do. We are committed to changing the future for families affected by rare diseases worldwide.

Why does it matter? Because if it was your child, your parent, your spouse with a life-altering rare disease, you would want everything possible done to help them, and so do we. Odylia moves safe, effective treatments forward.

Philanthropic investment is critical to advancing promising therapeutics. The rare disease community is changing the narrative—bringing ingenuity and urgency to redefine the future of what is possible in medicine. It is about patients, not profits.

**ODYLIA, CHANGING
THE OUTCOME
FOR EVERY
RARE DISEASE PATIENT**





BE A PART OF SOMETHING RARE

CONTACT US



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