Odylia THERAPEUTICS

IMPACT REPORT

Transforming Drug Development for Rare Diseases

odylia.org

WE ARE A FORCE FOR CHANGE

Odylia is a nonprofit organization, dedicated to accelerating treatments for rare diseases. Odylia develops transformative new therapeutics, while working hand-in-hand with patient groups to advance their drug development efforts. We are on a mission to make the drug development process accessible to rare disease groups. While it may seem straightforward, the reality is anything but simple.

The limitations of existing funding frameworks hinder for-profit biotech and pharmaceutical companies from pursuing viable treatments. While these companies are eager to make a positive impact, they must operate within the constraints of financial return on investment (ROI). Developing therapeutics for rare diseases necessitates community involvement, proactive risk management, and innovative business models.

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 redefining how gene therapies for rare diseases are developed. Our mission is to deliver life-changing therapies to patients often overlooked by traditional drug development.

We drive therapeutic advancement through two core initiatives: the Odylia Pipeline and Brydge Solutions. Our internal 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 in the rare disease space.

MISSION

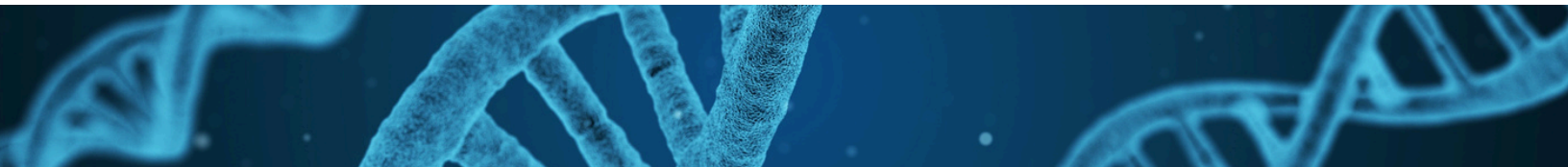
Odylia accelerates therapeutic development for rare diseases

VISION

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

... A DIFFERENT KIND OF BIOTECH

Programs need more than scientific expertise, they need dedicated champions who will drive them all the way to clinical trials.



RECOGNITION & MILESTONES

Impact for rare disease patients in just 7 years

- Lead pipeline program 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 2024 Amgen Prize, Concordia Summit presentation
- TRAP Award recipient from the Foundation Fighting Blindness ($1.5 million grant for RPGRIP1 Gene Therapy Program) (2024-2026)
- Advancement of Odylia's lead gene therapy program (OT-004), with positive FDA feedback in Odylia's first pre-IND meeting with the FDA (2025)
- Official announcement of 3rd 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
- Odylia mission video received Award of Distinction from *The Communicator*

FDA

BRYDGE SOLUTIONS Odylia

2024 AMGEN PRIZE AMGEN x SOLVE

FOUNDATION FIGHTING BLINDNESS

Visit The Odylia Library

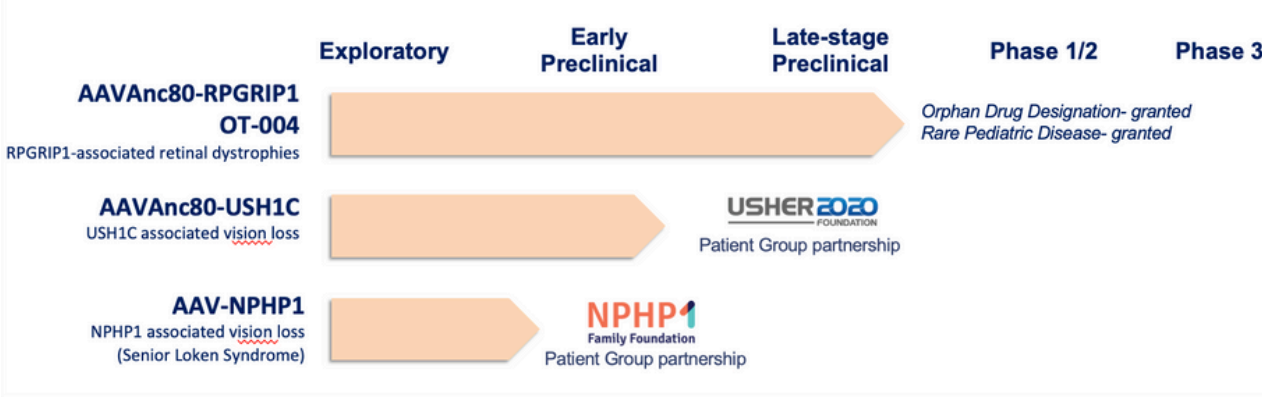
Watch the Odylia Mission Video

ODYLIA'S PIPELINE

Shifting investments in the life sciences sector has led to a continuous cycle of shelving promising therapeutics for rare diseases. Life-changing treatments should not stall because of limited financial profit.

The Odylia nonprofit model starts by identifying a viable therapeutic approach. From there, the Odylia team builds a collaborative network of partners and a strategic plan for each program that is fueled by support from a broad community of stakeholders including philanthropic organizations and families as well as industry supporters. To build the right team to advance each program, Odylia breaks down competitive barriers, bringing together a coalition of patient advocacy groups, researchers, industry partners, and funders who play pivotal roles in moving each development program forward.

We take pride in our team's deep expertise in drug development, genuine empathy, and firsthand experience with rare diseases. Our team's depth in scientific and operational strategy enable us to remain agile in the constantly evolving landscape of therapeutic development.



BRYDGE SOLUTIONS

Through Brydge Solutions, Odylia streamlines the path from early discovery to clinical trials by reducing common inefficiencies. Utilizing our expertise in rare disease research, drug development, and program management, we empower partners to accelerate their progress. Each year, the number of groups we support continues to grow—but demand far exceeds 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 and their families.

Odylia's growing network of Patient Research Group & Nonprofit Partners

jem THERAPEUTICS

PBD project

PURA Syndrome FOUNDATION

Saving Jordan Avi Ogman Foundation
Research & Development of Cures for TECPR2 Genetic Disease

THE RDH12 FUND FOR SIGHT

Smith-Kingsmore SYNDROME FOUNDATION

CLOVES SYNDROME COMMUNITY

Open Treatments

FOUNDATION FIGHTING BLINDNESS

NPHP1 Family Foundation

RPGRIP1
Preventing Vision Loss

USHER2020 FOUNDATION

SNOW FOUNDATION FOR WOLFRAM SYNDROME RESEARCH
A GLOBAL VOICE FOR RARE DISEASE

sat gene b2 foundation

Mike Kaplan, MD
President & CEO, JEM Therapeutics

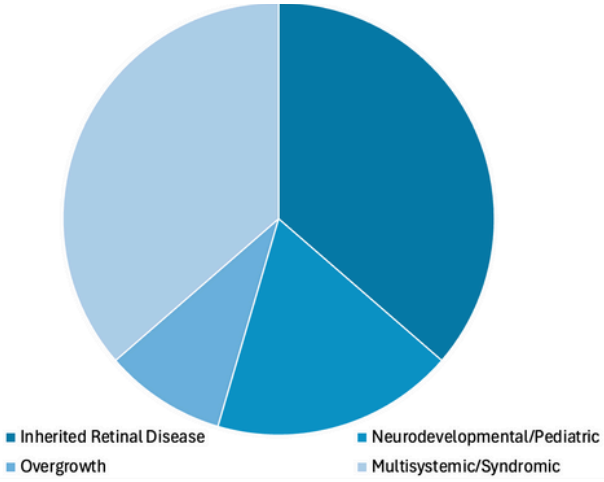
Our goal is to accelerate progress as quickly as possible so we sought a partner who had the depth of expertise to provide guidance on scientific strategy, but who could also give us insights into the financial and operational aspects of our company's growth. But what really stood out about Odylia was 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

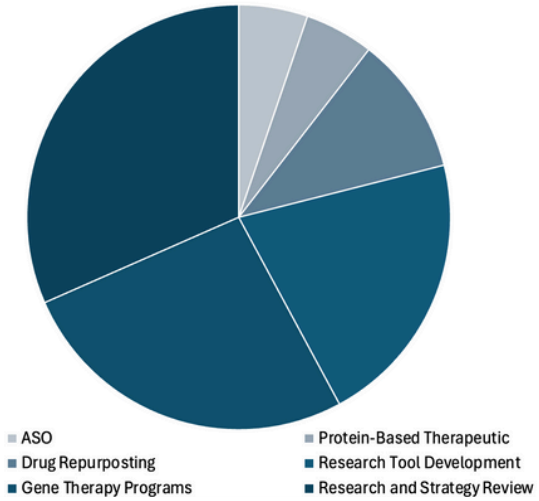
Working closely with patient groups, we provide direct access to the drug development process. We help groups focus on prioritizing development of therapies that are safe and effective. Our goal is to bring scientific expertise to these groups - the next generation of rare disease drug developers.

We empower them to identify and prioritize what matters most to their communities, advance the most promising therapies, and focus limited resources on the science with the greatest potential impact.

Current Therapeutic Areas



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 Kaczenski
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.

INCREASING 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 of resources from our Brydge Solutions partnerships, to help other drug development programs move faster towards the patients that need them

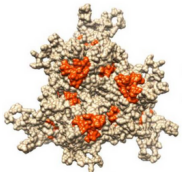
Odylia Youtube Channel

Educational videos, community updates for Odylia's pipeline programs, and essential resources from other creators in the field

The Odylia Collective Now, Comend!

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

Anc80 Access Program

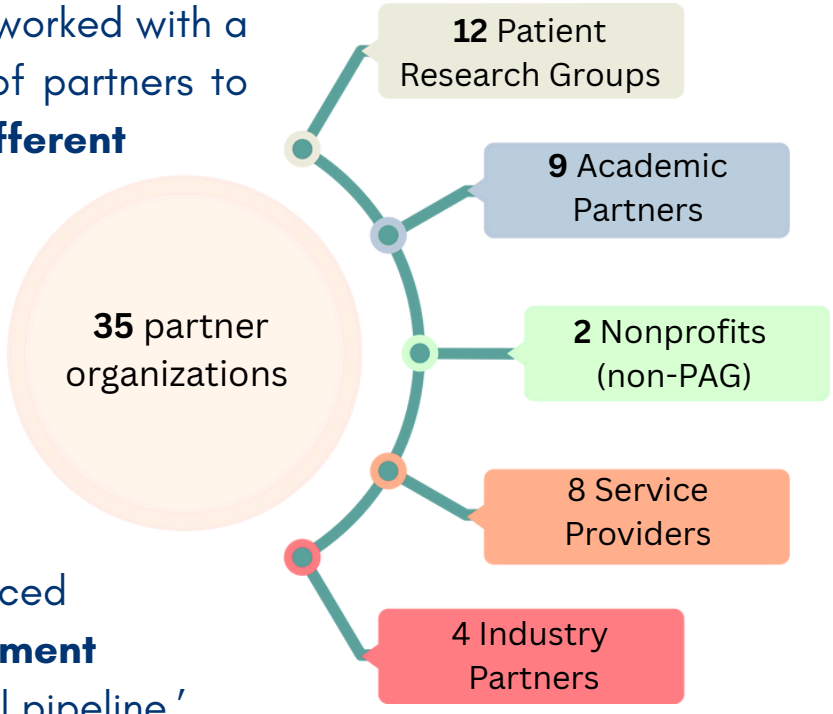


Odylia has exclusive rights to the Anc80 AAV vector for use in developing gene therapies to treat rare and ultra-rare retinal diseases. Anc80 was discovered in 2015 in the lab of Luk H. Vandenberghe, at Massachusetts Eye and Ear and can effectively transduce the retina, central nervous system, muscle, liver, heart muscle, and cells of the inner ear, to name a few. Odylia is developing tools to enable use of the Anc80 capsid for therapeutic delivery of DNA, and develping 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.

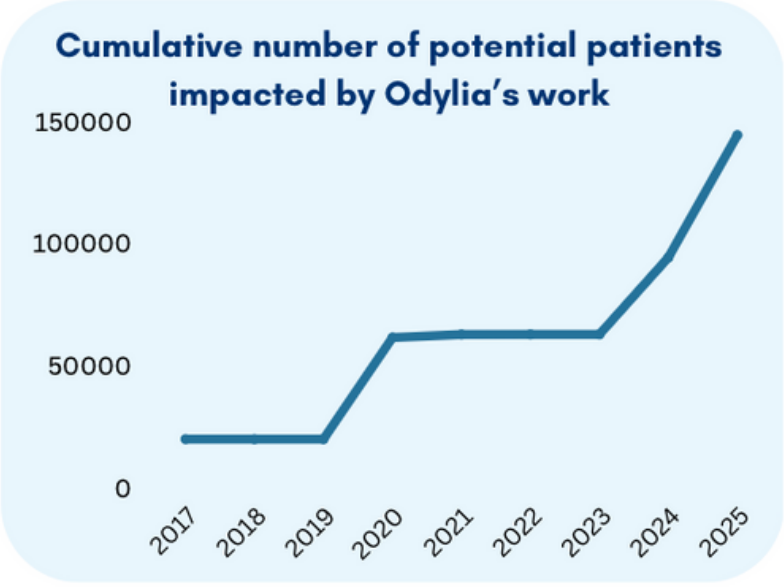
Since its inception, Odylia has worked with a diverse and growing network of partners to further its mission, serving **14 different rare disease communities**, and growing.



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

And 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 have incredible impact.



TURNING HOPE INTO REALITY

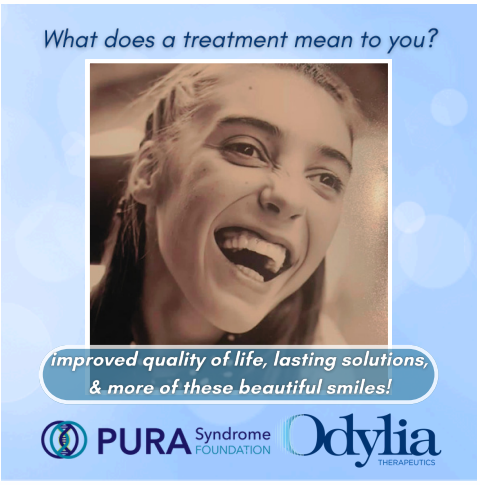
Odylia is driven by a singular purpose: to turn passion into impact for rare disease patients. Every breakthrough we achieve and every patient group we support brings us closer to life-changing treatments.

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.



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.

As a nonprofit, our work relies on charitable support to advance our mission. Progress takes time—sometimes years to move a treatment to clinical trials—but the wait is worth it. Without philanthropic investment, promising therapies risk being shelved simply because success is too often defined by profit, not patient impact. The rare disease community is changing that narrative—bringing ingenuity and urgency to redefine the future of drug development.

CHANGING HOW THE STORY ENDS FOR EVERY PATIENT WITH A RARE DISEASE

Odylia THERAPEUTICS

BE A PART OF SOMETHING RARE

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Platinum Transparency 2025 Candid.

Charity Navigator FOUR-STAR