

March 2021

## Changing the Paradigm

The latest news from Odylia Therapeutics about science for rare disease treatments

### From the Desk of CSO, Ashley Winslow, PhD

While 2020 was nothing like we expected, I'm pleased to share updates about the progress Odylia was able to make during the year and a few things we are looking forward to in 2021.

Our lead program, a gene therapy to treat vision loss caused by mutations in the [RPGRIPI](#) gene, is progressing rapidly. In 2020, promising preclinical data were generated supporting the therapy's ability to slow progression of vision loss. In 2021 we will find partners to support our goals to initiate key toxicology studies and to begin large scale manufacturing of the gene therapy in preparation for eventual clinical trials.

This past summer, Odylia officially launched a gene therapy development program for [Usher Syndrome](#) Type 1C in collaboration with the Usher 2020 and FAUN Foundations. We expect to generate the first set of proof-of-concept data by the end of the year, and our goal is to bring an USH1C gene therapy for vision loss to clinical trials by 2023. This accelerated timeline is possible through an impressive network of strategic partnerships and advisors.

Along with many other challenges, 2020 highlighted some specific roadblocks for rare disease treatment development. More support is needed for earlier stage treatment programs led by Patient Groups. These groups are seeking scientific and strategic guidance, and have limited bandwidth. Odylia created [Brydge Solutions](#), a service that provides help with navigating science, strategy, and opportunities for Patient Groups and their specific program or their research portfolio. During the coming months, we plan to work with more Patient Groups to leverage and deploy their resources appropriately and efficiently to further accelerate research.

Odylia was created to find innovative solutions and partnerships to accelerate treatments for the rare disease community. To this end, we are actively exploring new partnerships with patient groups, academic researchers, industry, and manufacturing vendors. Together we can create a path forward for rare disease therapies.

[Read More](#)

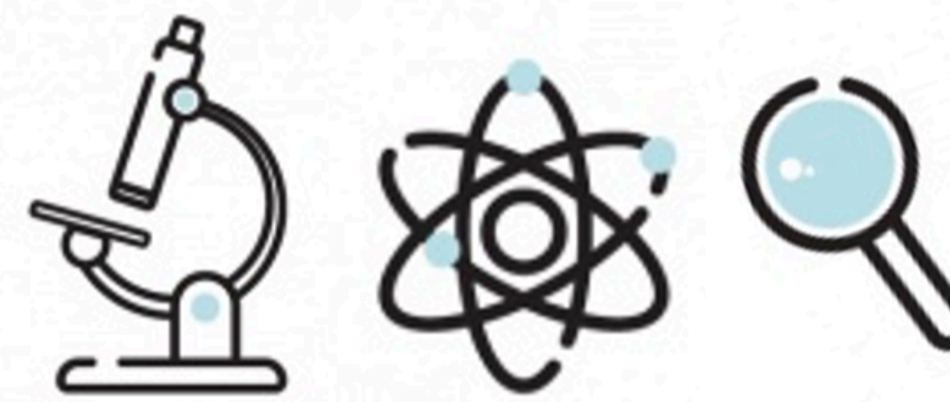
## What Does Treatment Mean to You?

In the lead up to Rare Disease Day, February 28, 2021, Odylia asked patients and their families, "What does treatment mean to you?" Their responses were shared on our social media platforms and remind us that the simple, little things that most of us take for granted each day are the most meaningful. Visit our website to see all of the photos and heartfelt responses.

Odylia's goal is to bring proven therapeutics to patients regardless of the number of people with the disease or the potential for profit. Developing treatments for rare diseases is not easy but we will stay on course to ensure therapies make it to the patients who need them.

Follow us on [LinkedIn](#) and [Twitter](#) to learn more.

[Read more](#)



### 2020 Highlights

The year did not turn out as expected, but Odylia did have many accomplishments:

[Tiasen Li, NIH researcher, joined the Advisory Board](#)

[In conjunction with the Foundation Fighting Blindness, Odylia hosted a four-part preclinical and translational research webinar series.](#)

[A new website was launched that includes a donation portal to make it easier to support Odylia](#)

[Odylia announced new partnership with the SATB2 Gene Foundation](#)

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