Delivering on the promise of treatments for rare disease

July 2023

From the Desk of CEO, Ashley Winslow, PhD

Accelerated Approval Process is Working for Rare Diseases

A few weeks ago, I wrote an opinion piece on LinkedIn responding to a recent approval of a gene therapy for Duchenne's Muscular Dystrophy (DMD). The decision was controversial because the Director of CBER at the FDA, Peter Marks, overrode the recommendation from his internal committee. Marks sided with an external panel of experts with backgrounds in DMD, and leaned into the belief that the Accelerated Approval Program would prove to be the best testing ground for this novel therapeutic. I'd like to expand on why this is important to the rare disease community and Odylia's own gene therapies in development.

Marks' decision impacts regulatory flexibility. In this case, I am referring to the regulatory flexibility of the FDA (Food and Drug Administration) which oversees new drug approvals in the United States. In recent years, rare diseases have been the testing ground for genetic technologies such as AAV gene therapies, CRISPR, and antisense oligonucleotides (ASOs) to name a few. Despite a starting point in rare diseases, many biotech and pharma companies eventually move away from rare diseases and into more common disorders that have a higher potential for financial benefit or where the clinical trial design is easier to navigate. The quicker the path to drug approval and the lower the risks along the way, the quicker a new drug can generate revenue for a company.

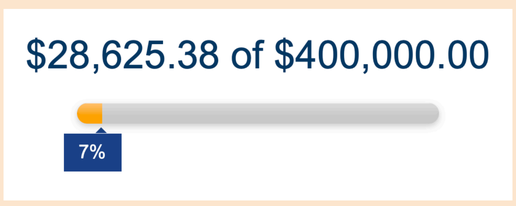
Compared to drugs that treat symptoms primarily, a genetic therapy that targets the genetic cause of the disease may take longer to see the benefit in patients. For example, Odylia's lead program is a gene therapy to treat vision loss caused by mutations in the RPGRIP1 gene. While we believe this gene therapy has the potential to prevent further vision loss, there are critical questions before testing this in a clinical trial. For example, we don't know how long it will take to see effects on visual function after we deliver a functional copy of the RPGRIP1 gene to the eye. Could this be a month? 3 months? A year? More? While the aim is to stabilize visual function at the time of treatment, is it possible to restore function that was lost prior to treatment? And which measurements will be the most sensitive to a beneficial effect of the treatment? Visual acuity? Or maybe an electroretinogram (ERG) could detect the earliest changes in photoreceptor function before full vision is affected.

Additionally, treating the genetic cause of disease can lead to an unpredictable cascade of events. For example, we are working with a patient group on a rare neurodegenerative disease called spastic paraplegia 49 (SPG49) caused by mutations in the TECPR2 gene, through our Brydge Solutions program. Symptoms like central apnea, recurrent respiratory infections, and global developmental delay are associated with TECPR2. Treatment with a gene therapy holds potential, but it can be hard to predict which one of these symptoms might change first and if all or only a few are treatable.

To address these questions, time is needed. Time to see if the treatment works and time to assess how the benefit will impact symptoms of the disease. To this end, building a more flexible regulatory system is absolutely necessary to the success of genetic targeting therapeutics. The FDA instituted the Accelerated Approval Program to allow "for earlier approval of drugs that treat serious conditions, and fill an unmet medical need based on a surrogate endpoint." When companies can't afford to invest in lengthier trial designs, they move onto other therapeutic areas that have less risks, and higher potential payoffs. By remaining flexible through the AAP process, the FDA is allowing for changes in intermediate biomarkers to serve as early indicators that the drug is working. Those initial signals, if meaningful, can allow the FDA to provide early approval, which allows a company to generate revenue while the effects continue to be monitored, and importantly, data is collected to prove over time if the therapeutic is effective. Importantly, this means that companies can remain committed to developing more treatments for rare disease.

Read the article.

Our Goal is to Reach Clinical Trials in 2025



Clinical trials in 2025! That is our goal for the RPGRIP1 Gene Therapy Program but we can't reach it without you. We must raise $400,000 by September 1, 2023.

Please consider making a donation today. You can make even more of an impact by inviting friends, family, neighbors, coworkers - everyone - to make a gift too.

Donations will ensure OT-004, the gene therapy to treat vision loss caused by mutations in the RPGRIP1 gene, will continue efficiently with a commitment to patients.

To help us reach our goal, a very generous donor has issued a challenge grant.
All donations made to the RPGRIP1 Program before September 1 will be matched dollar for dollar, up to $25,000.
That means your gift of $50 becomes $100.

We've made it easy for you to reach more people and help meet this important goal. You can set up a fundraising page and ask your network to contribute. More information and instructions can be found HERE.

Together we can make this happen and prevent vision loss.

Odylia Program Updates

Odylia in collaboration with Usher 2020 and FAUN Foundation is continuing to develop a gene replacement therapy to treat vision loss caused by mutations in the USH1C gene with our research partners in the Czech Republic, Germany, and the United Kingdom.

- Clinical candidate selection is ongoing, and we plan to complete the next set of efficacy testing this summer. Results from this study are anticipated in 2024.
- Odylia and the Usher 2020 Foundation have engaged a compliance leader to develop a solid data collection strategy, critical for any future regulatory filings.

The RPGRIP1 gene therapy program (OT-004) has made excellent progress this past spring.

- Odylia presented initial manufacturing results with Andelyn Biosciences at the American Society for Cell and Gene Therapy Conference (ASGCT). With great results, Odylia has progressed into the next phase of the manufacturing process. Our poster presentation can be found on the RPGRIP1 page of our website.
- In May we held a Q&A session with members of the RPGRIP1 community. The video from this meeting can be found on our YouTube channel.
- The next Q&A will be held during the week of July 24. Watch your email for more details.

BRYDGE SOLUTIONS
Odylia THERAPEUTICS

Through our Brydge Solutions program, Odylia partners with Patient Advocacy Organizations, academic organizations, and industry to accelerate therapeutic development for rare diseases. Together we CAN make a difference!

- Odylia launched our Odylia Library this spring. Through our own drug development experience and our work with partners through Brydge Solutions, we are building a publicly accessible library of resources in hopes that our learnings can help other drug development programs move faster towards the patients that need them.
- Odylia will be attending the Global Genes Rare Advocacy Summit in September to network with other rare disease organizations and share our experience and learnings.
- Odylia is looking for new partners. If you know a patient group or rare disease company that is looking for a strategic or operational partner please share our information.

Odylia will be at the Global Genes Rare Advocacy Summit September 19-21

If you would like to meet with us before or during the conference to learn more about how we can support your rare disease work, please complete this form.

Mark your calendar

September - National Guide Dog Month (US)

October 12 - World Sight Day

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Support Odylia! Together we can develop treatments for rare diseases.

Want to find out more? Visit our Website.

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