Odylia THERAPEUTICS

Delivering on the promise of treatments for rare disease

October 2023

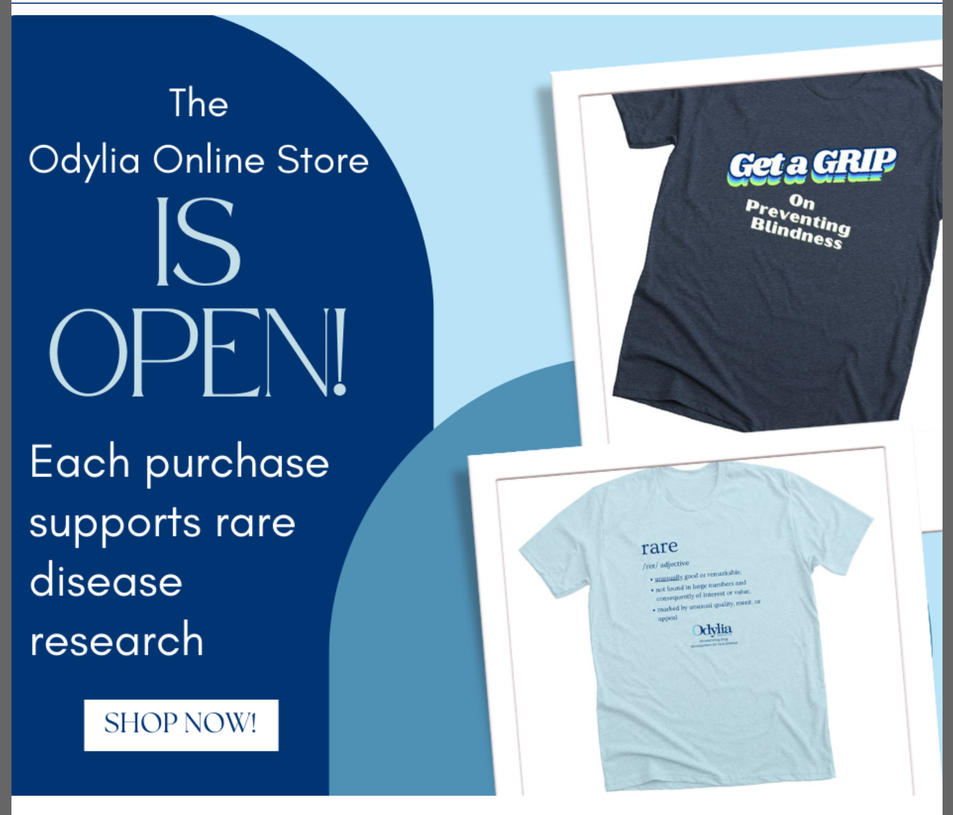
From the Desk of CEO, Ashley Winslow, PhD

Patient Groups Tackling Challenges Pharma Isn't Prepared to Face

Odylia Therapeutics works with some amazing patient groups. Having personally worked with rare diseases for almost two decades, I have seen patient groups fundamentally change how the entire life sciences landscape operates to bring a drug to market for rare diseases. For example, a single patient group brought competing pharma behemoths together to overcome roadblocks that are usually impassable. Patient groups have created their own successful companies that are better positioned to navigate the usual setbacks, thereby ensuring promising therapeutics continue to move forward. They have licensed the technologies directly to pharma, and more recently, salvaging their assets from crumbling BioPharm portfolios. Patient groups have grown from being the great motivator to being the great organizer and leader.

The most successful patient groups bring together key leaders who don't normally align with one another- scientists from academia, the medical community, pharma, donors, consultants, regulators, family members. Patient groups build productive teams that leverage the expertise of each individual role and contributor. But R&D is an expensive process and requires a significant amount of money. For this, patient groups create investment opportunities, investing in the early stage R&D and keeping scientists focused on what matters most. To this end, utilizing the power of academic research can be an incredible advantage, but it is not without its challenges. Incentives in academia are usually grants and publications, rather than treated patients and approved drugs. Through our work at Odylia we have the privilege of working hand-in-hand with patient groups that are boldly steering academic partners. Their unrelenting drive has prompted entire groups and university departments to diversify their traditional funding sources towards patient group awarded grants, service agreements, or licensing agreements. This speaks volumes to the sophistication of these organizations. Patient groups have moved from trying to attract interest in their disease, to funding the next generation of drugs, to developing and licensing those drugs, to founding and running the next generation of BioPharma to bringing those drugs to market.

And now roles are beginning to flip. I have seen drug developers turn to patient groups to identify resources that are lacking and to organize pre-competitive efforts in clinical trial design and biomarkers. The student has become the teacher, the principal, and in many cases the entire school board. The example patient groups set in how they leverage expertise across the drug development landscape is already changing how pharma thinks. I am convinced they will continue to have a positive impact by de-siloing information, data, and learnings so programs that do not fit as cleanly in the commercial model can still move forward. While the approach may seem grassroots on the surface, patient groups are leveraging the same experts, scientists, and technologists, but in a way that hones and hyper focuses efforts. In my opinion, patient groups are organizing the life sciences landscape of experts in a way that tackles the big challenges that much of pharma is unwilling to face. I can't wait to see what they will do next.



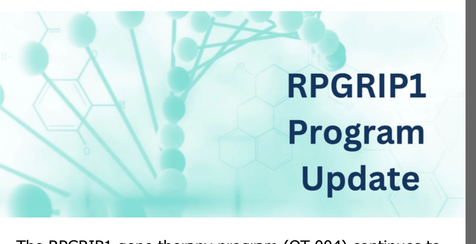
Announcing the Odylia Online Store

Odylia has opened an online store. Each purchase helps raise awareness about rare diseases and helps raise funds. Want to get some cool swag? Need the perfect gift? Check out our store HERE.

If you have an idea about a design we could offer, please email our Director of Development, Heather Greene.

Odylia Program Updates

In partnership with the Usher2020 Foundation, FAUN Fondation, and our collaborators in the Czech Republic, Germany, and the United Kingdom, Odylia continues to explore a gene replacement therapy to treat vision loss caused by mutations in the USH1C gene. As a part of this work, we are developing a more complete understanding of the USH1C genetic model to help guide future safety studies. Preliminary results from our current study are expected in the first half of 2024.



The RPGRIP1 gene therapy program (OT-004) continues to make great progress as we head into fall.

- Odylia's next RPGRIP1 Q&A session will be held on November 8th at 1:00 pm EST. Sign up here.
- Odylia is planning for preliminary engagement with the FDA later this year and beginning of next year.
- Odylia has partnered up with Medibanx to collect longitudinal patient data. This will be extremely beneficial to understanding how mutations in RPGRIP1 affect vision throughout people's lives. Follow the link to learn more and feel free to reach out if you have questions.

BRYDGE SOLUTIONS
Odylia THERAPEUTICS

Odylia partners with Patient Groups, academic institutions, and industry to accelerate therapeutic development for rare diseases through our Brydge Solutions program. Together we CAN make a difference!

- Odylia attended the Global Genes Rare Advocacy Summit in September where we had the opportunity to meet with many rare disease advocates and discuss ways we can work together to accelerate drug development.
- We have a new video series out that aims to address many of the common questions and roadblocks that rare disease patient advocacy groups face. Our first video covers free resources that can help patient groups get started. Check out our video series HERE.
- Brydge Solutions 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.

RPGRIP1 Gene Therapy Program Campaign

Our goal is to reach clinical trials in 2025. Thanks to amazing and generous donors we are getting closer. People in communities all over the world came together to raise funds this summer. We now have over $175,000 raised toward our goal.

Gavin Birch, in the UK, raised more than US$12,000 through a variety of fundraising events and his friends, including Rob Dixon and Jason Harrison, organized a charity football match and raised almost US$2,000.

Melissa Matias, in Texas, organized a fundraiser at MOD Pizza. The restaurant donated a portion of sales one evening in August and raised over $500 for the RPGRIP1 Gene Therapy Program.

Thank you to everyone who donated and worked to organize events or recruit others to support this effort. We are making progress! There is still more to do. We must reach our overall goal of raising $400,000 to continue to move forward. Just like you, Odylia won't quit. We will continue to do all we can and we will make sure the program doesn't stop. For fundraising ideas, visit this link.

If you know of someone who may be interested in donating please send them HERE.


Greg Spitznagel, #5 for the NAHL Philadelphia Rebels

Greg is donating $10 for EACH goal he scores to Odylia. Help him make an even bigger impact by making a donation HERE. ALL funds raised will be used to support the development of genetic treatments for rare diseases like RPGRIP1.

Let's Get Social

Odylia is now on Instagram and Facebook.
Follow us and tell you friends.

Mark your calendar

October - Vision Awareness Month
World Blindness Awareness Month

Is this a pass-along copy? Click to subscribe today.

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

Want to find out more? Visit our Website.

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