Odylia THERAPEUTICS

*re**Imagining*** drug development for rare disease

From the Desk of CSO, Ashley Winslow, PhD **September 2021**

Why is Drug Development for Rare Diseases so Challenging?

Why is it so hard to develop treatments for rare disease? Well, the answer is simple. It's not. You can make a fair argument that it is easier. The majority of rare diseases are thought to be caused by genetic variation, or mutations. For many rare diseases, mutations in one gene are known to cause that disease. These diseases are called monogenic. When we know the cause of a disease it is usually easier to develop new treatments because we can design drugs to target that dysfunction. In more common diseases where the cause is not genetic or is the result of a complex interaction of genes, it is much harder to develop effective therapeutics. So arguably, for many rare diseases that are monogenic, we have fought half the battle when we know what to target.

So why aren't companies jumping headlong into drug development of rare diseases? Well, some are, but most still battle with a business model where the low prevalence of rare disorders cannot generate enough profit to support the expected return on investment. This is the reason Odylia was created. Our nonprofit was born from the frustrations of a father who was tired of dead ends and a scientist who knew we could do better. Odylia seeks to change the traditional business model to one where profit is not the primary driver. To achieve this goal, Odylia partners with patient groups early in the research and development phase to fund programs through 'the valley of death,' a term used to denote the late-stage preclinical space often inclusive of efficacy and toxicology testing before clinical trials. Commercial interest in a new therapeutic often increases after efficacy and safety have been proven in animals. By de-risking these programs through this critical phase, programs are far more likely to succeed, and therefore, should be more appealing to potential partners.

> "What good does it do to create life-altering treatments and never bring them to patients?"

Another important role Odylia plays is as champion of our programs. Oftentimes commercial business strategies change, causing promising therapeutics to be jettisoned or shelved. With each of our programs, we feel it is important to protect against this possibility and to act as a continued home base for a program. We ensure each program has a full development plan and we work hard to minimize stall times by initiating work as soon as we have funding.

Odylia's lead program, a treatment for Leber Congenital Amaurosis 6 (LCA6), is a gene therapy to provide patients with a functional copy of the *RPGRIP1* gene. Mutations in *RPGRIP1* cause LCA6 and result in infantile and pediatric loss of vision which progresses over time. Odylia is continuing to advance this program and working to ensure that this promising therapeutic has the best opportunity to reach patients. Odylia's RPGRIP1 program has strong efficacy and preliminary toxicity data. We have built a full development path for the program through IND submission, and we are actively looking for commercial and non-commercial partners, grants, strategic partners, and donations to support the continued movement forward. The 'how?' is not a problem, identifying the funding to do so is the challenge. Share this newsletter with your network and join us in thinking creatively about what is possible, and how we can reimagine drug development for rare disease.

Be a Champion- You can support rare disease treatments

Odylia Therapeutics is a nonprofit organization and relies on support from a variety of sources to fulfill our mission. Donations are vital to our work but in addition to making a gift, there are other ways you can make a difference.

- Make a donation here
- Email info@odylia.org with questions or suggestions
- Follow us on social media LinkedIn and Twitter

Join Us for an Informational Meeting about RPGRIP1

Odylia Therapeutics will present a program about gene therapy and our work on a treatment for LCA6 to address vision loss caused by RPGRIP1 mutations. Chief Scientific Officer, Ashley Winslow, will talk about gene therapy drug development and update attendees on the status of a possible treatment for LCA6.

To register for the zoom, click HERE.

Please share our newsletter with others who may be interested in finding treatments for rare diseases.

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