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From the Desk of President & CSO, Ashley Winslow, PhD

### An Innovative Approach to Bring Treatments to Rare Disease Patients

The scientific understanding and technology exist to treat many genetic causes of disease. However, the commercial model used to develop drugs often falls short when it comes to rare diseases. This does not just impact the patients, but the families, caregivers, and communities around them. Though patient populations for any one specific disease may be small, the aggregate is costly.

*One study estimated total 2019 U.S. costs for rare diseases at \$966 billion-including direct medical costs (drugs, dr visits) and other non-medical/indirect costs (loss of income).  
- The US Government Accountability Office*

Odylia’s mission of accelerating drug development for rare diseases does not begin and end with our organization, rather, our organizational strength lies in strategic community building. For each new treatment program in our pipeline, we bring together the experts and organizations needed to move it to the next stage of development.

To overcome the weaknesses often found in the traditional drug development process, like the ‘stalling’ or ‘shelving’ of promising treatment programs, Odylia leverages the strength of our community partners. Pharmaceutical companies sometimes make the tough decision to de-prioritize promising therapeutics, in order to deploy their budgets behind the therapeutic that is most likely to succeed in their portfolio. This means that a promising treatment may never make it to clinical trials.

Through Odylia’s unique approach to partnerships we champion promising therapeutics throughout the development cycle. When we work with industry partners, we take important steps to ensure programs are not shelved, unless they are shown to be unsafe or ineffective. These steps include incorporating unique contractual terms, being a critical and involved resource to the industry partner, and in some cases, continuing to lead the program through a hybrid approach to partnerships. Importantly, as a nonprofit, we prioritize the patient, and are not dependent on delivering profits to investors or shareholders.

I’ve recently had the opportunity to speak in more detail about Odylia’s nonprofit approach to drug development. Check it out [here](#), [here](#), and [here](#) if you would like to learn more.



#### RPGRIP1 Patient Advisory Board Interest Form



Odylia is forming an RPGRIP1 Patient Advisory Board (PAB). The PAB will bring together a group of informed individuals that represent a diversity of age and geography within the RPGRIP1 community to share knowledge and personal experience that will be used to inform clinical trial design. If you are interested in being a part of the PAB, please [complete this form](#).

Patient advocacy organizations are taking progressive steps to drive drug development programs. But drug development is difficult, and many groups come upon unforeseen challenges. Odylia’s Brydge Solutions program provides rare disease communities with expertise that reduces inefficiencies in translating new therapeutics from bench-to bedside, increases preclinical productivity, accelerates the path to clinical trial, and reduces program development costs. [Learn more](#).

**This fall, we are asking everyone who gets our newsletter to share it with at least two people who might be interested in our work.**

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