Odylia THERAPEUTICS

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

March 2023

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

I am often asked, "Why a nonprofit?"

Nonprofit organizations improve our quality of life, often fulfilling needs that are underserved by both the private sector and government. Our communities are supported by a three-legged stool composed of businesses, government, and nonprofits. The private sector, or businesses, addresses consumer needs and is incentivized by financial gain. Government, or the public sector, addresses needs that are usually unprofitable but necessary for the larger public good. Nonprofit organizations feed, heal, shelter, educate, inspire, enlighten, and nurture people of every age, gender, race, and socioeconomic status – fulfilling needs that are often too specific to be considered for the 'public good', and for which a profit-driven model does not work.

Odylia Therapeutics was founded as a nonprofit for just this reason, to serve the rare disease community. Each rare disease has a small number of patients, meaning a return on investment is often limited, and government's ability to impact a large number of people is low. However, the societal impact of treating rare diseases is tremendous, and often measured in lowered healthcare costs, increased employment opportunities, decreased psychiatric effects, and lives saved and lived.

I often find that people misconstrue the definition of nonprofit to mean the organization cannot make money. In fact, nonprofits can and should bring in excess revenue which is then driven back into fulfilling the mission and expanding the reach of their services. While the funding model is more challenging than that of a for-profit business, nonprofits have the added benefit of focusing 100% of the funds raised on the organization's mission, rather than diverting a portion of revenue to investors. This allows us to operate on a more streamlined budget, and critically, it allows Odylia to make decisions differently, and remove some of the critical barriers traditionally present in the rare disease space.

Odylia exists to develop safe, effective treatments for rare diseases ensuring this next generation of transformative medicines move to clinical trials and to the patients who need them. A nonprofit model enables us to focus on developing treatments for rare disease patients through a drug development process unhindered by commercial constraints on decision-making.

Our lead program is a prime example. We prioritize the questions of safety and efficacy, over profitability. Over the last five years, our RPGRIP1 gene therapy program would have been shelved or terminated numerous times, but Odylia has kept the program moving. Our criteria for moving a program forward centers on three questions: Do we have the technology or scientific know-how to make a therapeutic? Is it safe? Is it effective? These three criteria drive decision-making. We work on programs that do not usually fit into industry's model, to ensure the entire community is served. Our work compliments, expands, and enhances the work of our partners in the private and public sectors.

It may not be the easiest approach, but it needs doing!

For a fascinating review of philanthropy that dates back to the 1500's, visit the National Philanthropic Trust's *History of Giving* interactive timeline. https://www.historyofgiving.org/

"I said "Somebody should do something about that." Then I realized I am somebody."
- Lily Tomlin

Odylia Therapeutics was launched five years ago by our co-founders, Scott Dorfman and Luk Vandenberghe. Luk and Scott knew there was a better way to develop treatments for rare diseases. When the idea for Odylia was born, they realized things that also remain true today:

- The traditional commercial model does not work for many rare diseases because of limited financial profit, so many treatments are never made.
- Rare disease patient advocacy groups often lack the bandwidth and scientific expertise needed to run effective and efficient drug development programs to advance their own treatments, resulting in lost time and money.
- Hundreds of viable treatments for genetic diseases are left sitting in academic labs instead of saving sight and saving lives. Often, the science demonstrates that a treatment should work, but there remain obstacles in the path to clinical trials, and ultimately to patients.

In 2018, Odylia became a first-of-its-kind nonprofit biotech company. Since then we have advanced gene therapy programs for vision loss caused by mutations in the RPGRIP1 and USH1C genes and helped numerous rare disease patient advocacy groups navigate and accelerate their own research, in order to better serve rare disease patients.

We are excited by all that has been accomplished in five years and look forward to even more in the coming years. Help us celebrate by making a gift of $55.55 to support our mission to accelerate drug development for rare diseases.

Odylia Program Updates

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 is excited to partner with JEM Therapeutics to develop and implement their scientific strategy for therapeutics to treat TECPR2-related disease. Read more.
- 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. More details.
- In May of this year Odylia will be at the ASGCT conference discussing our gene therapy programs and Brydge Solutions, please reach-out to find a time to meet-up if you will be attending.

USH1C: Odylia is developing a gene replacement therapy to treat the vision loss caused by mutations in the *USH1C* gene. Odylia has several strategic partnerships in the Czech Republic, Germany, and the United Kingdom and with our funding partner, the Usher 2020 Foundation, that ensure continued advancement of this therapeutic for patients with Usher Syndrome Type 1C.

- Clinical candidate selection and efficacy testing: In the past year, we have begun testing several designs of a gene therapy to identify which is the most effective for treating or preventing vision loss.
- All aspects of our efficacy study are now underway and we anticipate results in 2024, with the potential for early findings later this year.

The RPGRIP1 gene therapy program (OT-004) continues to progress through new partnerships and the start of manufacturing.

- The initial phase of manufacturing for Odylia's gene therapy to treat vision loss caused by *RPGRIP1* mutations is underway with our contract development and manufacturing (CDMO) partner, Andelyn Biosciences out of Ohio.
- Odylia has begun preparing for preliminary discussions with regulators about the RPGRIP1 gene therapy program later in the year
- Odylia continues work with the Patient Advisory Board and Clinical Advisory Group. This diverse group of patients, parents, caregivers, clinicians, and researchers continues to provide valuable insight which we are incorporating into our clinical trial design

Odylia will be at these upcoming events

March 21-23
Global Genes Conference

May 1-3
Rare Drug Development Symposium

May 16-20
ASGCT Conference

Mark your calendar

March 23 - World Optometry Day

May 16-23 - Inherited Retinal Disease Genetic Testing Week

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