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

Benefits of Rare Disease FDA Designations in Drug Development

In November of last year, Odylia announced that our lead gene therapy program was granted Orphan Drug and Rare Pediatric Disease Designations by the FDA. You might wonder what these designations are and why they can help our RPGRIP1 gene therapy program move faster and attract potential partners over time.

In 1983 Congress signed the Orphan Drug Act (ODA) into law with the intention of incentivizing rare disease drug development. Congress recognized the limited profit a company would make from sales of a rare disease drug and the need to motivate companies to continue developing drugs to prevent or treat rare diseases.

"there is reason to believe that some promising orphan drugs will not be developed unless changes are made in the applicable Federal laws to reduce the costs of developing such drugs and to provide financial incentives to develop such drugs; and it is in the public interest to provide such changes and incentives for the development of orphan drugs."

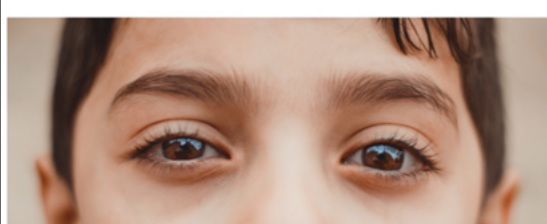
The ODA established the criteria for the Orphan Drug Designation and the benefits offered to those programs which include:

- waiver of the prescription drug user fee (estimated at \$3.1 million in 2022)
- tax credits (up to 50% of clinical testing expenses within the same tax year)
- waiver of NDA/BLA user fees (value of approximately \$2.9 million in 2021)
- eligibility for 7-year marketing exclusivity upon marketing approval
- ability to apply for research grants to support clinical studies through the Office of Orphan Products Development

Different from the Orphan Drug Designation, the Rare Pediatric Disease Designation provides access to a priority review voucher (PRV) if the drug is approved. A PRV provides the holder with an accelerated 6 month timeline for review of a drug once clinical trials are completed. In addition to the obvious benefit to patients and families of speeding-up the regulatory process, for industry, an accelerated approval holds financial value by putting a drug on the market earlier and therefore accelerating the time to sales and revenue generation, longer patent life protection (longer on the market), and earlier market entry relative to competitors. For this reason, the RPDD increases the value of the rare disease program and if awarded, the PRV can be sold to generate revenue.

These two designations increase the value of the RPGRIP1 program, decrease the costs of bringing the program through clinical trials, and increase Odylia’s access to conversations with the FDA about this program. Collectively, the financial and strategic impact of these designations will accelerate the timelines for moving our RPGRIP1 gene therapy program through clinical trials and position the program to benefit from early regulatory feedback. Additionally, they make the program more attractive for potential partners interested in accelerating therapeutic development.

Given the obstacles in the rare disease space, Odylia’s goal is to decrease the risk and increase the value of our gene therapies, in order to attract funders or industry partners to invest in each program. In the long term, we hope to reshape the conversation around ‘value’ in drug development for rare diseases. By developing a network of like minded groups, we are confident that we can develop innovative partnerships to ensure continued investment in therapeutic development for rare diseases.



The Path Forward – Funding the RPGRIP1 Gene Therapy

Odylia Therapeutics has a goal of raising \$1.95 million this year to fund the crucial steps needed to move the RPGRIP1 Gene Therapy toward clinical trials. We hope YOU will help.

We've set up a Peer-to-Peer Fundraising platform to make it easy. You can set up a fundraising page, tell your story about why you are raising funds, and then send it out to your network to ask for support.

Instructions to get started are [HERE](#).



Fundraising & Awareness Resource Page

Odylia Therapeutics has launched a resources page on our website with information that can be shared about Odylia and our rare disease gene therapy programs. You can also find tips and info to help raise funds. [Visit the Page](#) to download fact sheets, social media posts, and more.

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

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