Accelerated Approval Process is Working for Rare Diseases

A few weeks ago, I wrote an opinion piece on LinkedIn responding to a recent approval of a gene therapy for Duchenne’s Muscular Dystrophy (DMD). The decision was controversial because the Director of CBER at the FDA, Peter Marks, overrode the recommendation from his internal committee. Marks sided with an external panel of experts with backgrounds in DMD, and sided with the recommendation that the Accelerated Approval Program would work best for this particular therapy. It is important to note that the FDA did not expand its approval to Duchenne DMD, but it is important to note that the FDA did approve the treatment for a number of rare diseases and has shown a willingness to approve these types of therapies.

Marks’ decision appears regulatory flexibility. In this case, I am referring to the regulatory flexibility of the FDA (Food and Drug Administration) which oversees new drug approvals in the United States. In recent years, rare diseases have become a focal point for drug development. The gene therapy to treat Duchenne muscular dystrophy is one of many that have been approved by the FDA in recent years. The FDA has approved treatments for rare diseases and has shown a willingness to approve these types of therapies.

This flexibility allows for the approval of drugs for rare diseases. The FDA has approved treatments for rare diseases and has shown a willingness to approve these types of therapies.

To address these questions, time is needed. Time to see if the treatment works and time to assess how the benefit will impact symptoms of the disease. To this end, building a more flexible regulatory system is absolutely necessary to the success of genetic targeting therapeutics. The FDA instituted the Accelerated Approval Program to allow “for earlier approval for drugs that treat serious conditions, and fill an unmet medical need based on a surrogate endpoint.” When companies can’t afford to invest in lengthier trial designs, they move onto other therapeutic areas that have less risks, and higher potential payoffs. By remaining flexible through the AAP process, the FDA is allowing for changes in intermediate biomarkers to serve as early indicators that the drug is working. These initial signs, if successful, can allow the FDA to provide early approval, which allows a company to generate revenue with the efforts cannot be monitored, and imprecise data is collected to place one year of the treatment. Imprecision means that companies cannot commit to developing new treatments for rare diseases.

Research shows...

Our Goal is to Reach Clinical Trials in 2025

$2,625.38 of $400,000.00

Clinical trials in 2020: That’s our goal for the RPGRIP1 Gene Therapy Program but we won’t reach it without you. We must reach $400,000 by September 1, 2023.
Please consider making a donation today. You can make even more of an impact by inviting friends, family, neighbors, or colleagues to make a gift too.
Donations will ensure RPGR, the gene therapy to treat vision loss caused by mutations in the RPGRIP1 gene, will continue efficiently with a commitment to patients.
To help us reach our goal, a very generous donor has issued a challenge grant.
All donations made to the RPGRIP1 Program before September 1 will be matched dollar for dollar, up to $25,000.
That means your gift of $50 becomes $100.
You can make even more of an impact by inviting friends, family, neighbors, or colleagues to make a gift too.
Together we can make this happen and prevent vision loss.

Odylia Program Updates

Odylia in collaboration with Usher 2020 and RSGF Foundation is continuing to develop a gene replacement therapy to treat visual loss caused by mutations in the TECPR2 gene, which is associated with Usher Syndrome Type II. TECPR2 vision loss is associated with the decrease in photoreceptor function and visual acuity.

Odylia and the Usher 2020 Foundation have engaged a clinical development team to develop a clinical data collection strategy, crucial for any future regulatory filings.

The RPGRIP1 gene therapy program (OT-004) has made excellent progress this past year.
• Odylia present at initial manufacturing events with Andelyn Biosciences at the American Society for Cell and Gene Therapy Conference (ASCOT, 2023).
• Good results. Odylia has progressed to the next phase of the manufacturing process. Our gene production can be found on the Odylia YouTube channel.
• In May we held a Q&A session with members of the RPGRIP1 community. The video from this meeting can be found on our YouTube channel.
• The next Q&A will be held during the week of July 24.

Visit our website.