Delivering on the promise of treatments for rare disease

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From the Desk of CEO, Ashley Winslow, PhD

Successful Gene Therapy May Impact RPGRIP1

From *"Gene Therapy Restores Hearing in Deaf Mice...Down to a Whisper"* to *"Gene Therapy Allows an 11-Year-Old Boy to Hear for the First Time."* These are the headlines marking the journey of AK-OTOF a gene therapy to treat *OTOF (otoferlin)*-mediated hearing loss utilizing the Anc80 capsid, the same capsid Odylia is using in our gene therapy pipeline. At the core of AK-OTOF is the hope-turned-promise of the Anc80 capsid and its ability to deliver the genetic payload to the targeted cells. Similar to Odylia's approach with our RPGRIP1 program, AK-OTOF uses the Anc80 capsid to deliver a functional copy of the otoferlin gene to the inner ear cells in patients with mutations in otoferlin. AK-OTOF returned hearing to a patient who has never heard before. We have a similar hope for OT-004. Using the Anc80 capsid, we hope to return or stabilize vision in patients with RPGRIP1-related vision loss, or for those with USH1C-related vision loss (Odylia's second gene therapy program). From a scientific perspective, the success of the AK-OTOF program provides clear evidence that the Anc80 capsid can deliver genes to the human body where they are needed. For the RPGRIP1 program, this means the jump from animal-based studies to human clinical trials is not as theoretical as it was before the achievements of AK-OTOF

During its journey, AK-OTOF moved from academic lab, to start-up company, to the acquisition of that startup company and AK-OTOF by pharma company, Lilly. A circuitous path to the clinic but an all-too-common path for many gene therapies and rare disease drugs, if they do not fail or stall along the way. For this gene therapy technology to reach OTOF patients adequate funding had to be identified, businesses had to be built and acquired, then a path forward was forged, ultimately, reaching patients. There are numerous ways to get to the same ending, but there are just as many ways that companies fail along the same path. Odylia's nonprofit approach to drug development for rare diseases means we rely upon charitable donations and grants, not the promise of financial profit to fund drug development. While raising funding this way may be more challenging, being a nonprofit enables us to commit to our programs for the right reasons, safety and efficacy, while allowing us to remain flexible in how we partner in the broader biotech and pharma landscape. This combination of flexibility and a focus on the clinical impact of a new drug, puts the focus back on the patient's needs, ensuring that promising programs continue to advance.

A gene therapy's path to clinical trials and to patients requires money, innovation, a fearlessness to pursue that often circuitous path, and a passionate community of supporters. The success of AK-OTOF and Anc80 in hearing loss carries with it excitement not only for the OTOF patients but also for our community. Congratulations and thank you to the many people who were involved! We look forward to watching the impact of Anc80 has on future programs.

Odylia Program Updates

In partnership with the Usher2020 Foundation, FAUN Foundation, and our collaborators in the Czech Republic, Germany, and the United Kingdom, Odylia continues to develop a gene replacement therapy to treat vision loss caused by mutations in the USH1C gene.

- The final phase of our current USH1C study is underway and we have begun collecting final measurements, a process that will span 6-9 months.
- Once all the results have been collected and samples analyzed, Odylia along with our partners will assess the data and shift into a new phase of development for this program.
- We are seeking a new dynamic QA specialist to join our robust team. Please forward any inquiries to **jklein@odylia.org**

OT-004, Odylia's RPGRIP1 gene therapy program continues to make progress towards the clinic.

- Odylia's next RPGRIP1 Q&A session will be held in April. Be on the lookout for further details, we will be updating on the progress of the gene therapy, future plans, and how you can help get ready for our 2024 RPGRIP1 Fundraising Campaign!
- Odylia has completed process development for the RPGRIP1 gene therapy with Anc80 capsid. This crucial manufacturing step enables batch scale-up to a larger size for our toxicology studies.
- Odylia spoke with Ian Taylor, Co-Founder and CEO of Medibanx about our partnership and how Medibanx helps RPGRIP1 patients and families contribute to longitudinal data that is critical for understanding disease progression. Please check out that interview **HERE**.
- The pre-IND continues to make great progress with anticipated submission in Q2 2024.

BRYDGE SOLUTIONS

Odylia partners with Patient Groups, academic institutions, and industry to accelerate therapeutic development for rare disease through our Brydge Solutions program. Together we CAN make a difference!

- Odylia will be attending Global Genes Rare Drug Development Symposium in Philadelphia on April 29th-May 1st and ASGCT Annual Meeting in Baltimore on May 7th-11th so **please reach** out if you will be at either of these conferences, we would love to connect!
- Two of our current Brydge Solutions partners are open to partnering their programs or investment. **Reach out** today if you are interested in learning more about therapeutics in development for a pediatric neurodegenerative disorder or vision loss and we will connect you directly.
- Please **check out our video series** that aims to address many of the common questions and roadblocks that rare disease patient advocacy groups face.

Brydge Solutions 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**.

YOU can make a difference. Set up a fundraising page and share with your friends, relatives, co-workers, and neighbors. It is easy to set up and you can help raise funds for your gene therapy program. Follow this link for **easy-to-follow instructions**.

What content is most important to you?

- RPGRIP1 Program
- USH1C Program
- Brydge Solutions
- General Rare Disease
- Ways to Get Involved

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