Dear friends,

A phone call in late 2017 changed my life forever.

Just two years ago I was uncertain of what I wanted to do after business school, not believing that a single person could help end a disease that threatened my brother, Terry. That all changed after answering the phone and hearing Terry had taken a serious fall.

That phone call started what would become a race to form an organization with the capacity to effect change for the ones we love.

Two years later the organization that began in a dorm room has blossomed into a fighting force that's drawn people together to end rare disease through the development of customized therapeutics.

With each day, we draw closer to ending Duchenne and providing the world a method by which customized drugs can be rapidly designed, developed and, with approval, administered to patients who need them most.

The road is long and often times weary, but I draw strength from this group in my darkest hours.

I have never been more confident that this path we are forging has the potential to radically impact drug development so that today's patients can see tomorrow's cures. While the coming year will be one of the most defining for this collective group, I trust that we are capable of anything.

This Year's Accomplishments

- **Built the best scientific team for Duchenne:** Formed an unprecedented research collaboration spanning 7 institutions and over 15 researchers
- **Developed a therapeutic to reverse the disease:** Showed in-vitro proof of dystrophin restoration through our custom CRISPR therapy
- **Created a customized animal model for drug testing:** Established our humanized DMD mouse colony for preclinical testing in early 2020
- **Established a patient pipeline:** Enrolled 5 additional patients into our custom medicine process
- **Engaged payers on drug coverage:** Began discussions with leading national payers to set groundwork for a customized therapeutic reimbursement mechanism
- **Built a diverse and experienced team:** Organizationally, we added 7 new key board members and 2 staff members to expedite our growth and development of customized therapeutics

Into the Future

In 2020, we will prove that we can stop a disease that's claimed far too many lives and show the world that passion, dedication and collaboration are the strongest weapons in the fight against rare disease.

- **Set replicable dosing guidelines:** Successfully complete dose-finding for efficacy and toxicology studies on our first custom drug
- **Get FDA approval for administering to Terry:** Obtain FDA approval to dose our first patient, Terry, with a first-in-man administration of our customized therapeutic
- **Create drugs for 5 additional patients:** Advance our first cohort of patients to the preclinical stage with the expectation of dosing in 2021
- **Expand our organization with funding and staff:** Grow our board and fundraising ability to support a larger second cohort of patients
- **Create FDA approval policies for custom drugs:** Modify existing FDA regulations to create a long-term pathway for custom therapies
- **Get custom drugs approved with payers:** Activate insurance reimbursement of customized therapeutics as we scale the process

We have accomplished so much, and so quickly, but we need your continued help to grow at this fast pace. **It was with the 100,000 donors who stand with us that we made progress this quickly.**

Our mission is to transform how rare disease is treated so that patients don't have to wait 10 years to receive a cure.

I hope you'll consider continuing to support this work or, if you're just learning about Cure Rare Disease, that you'll

consider seeing the impact that your donation has to help change the world and protect our loved ones from disease.

With deepest gratitude,

Rich Horgan

Donate