A MESSAGE FROM THE FOUNDER

Dear all,

This past year was filled with significant challenges, but thanks to our community's support and our researcher's tenacity, CRD achieved scientific advancements despite the headwinds. As we move into the final stage of dosing our first patient, my brother, Terry, I would like to thank our community for your continued support. We are pleased to bring your our winter update for 2021, including a recap of what you may have missed since the last newsletter, an exciting save the date for our much-anticipated gala, and a research progress update—all made possible because of you.

All the Best,
Rich Horgan, Founder & President
Research Update

Our research took a BIG step towards the clinic! We’ve provided a summary of the accomplishments below.

**Kinea Biosciences partnership established to support preclinical development**
- For patients possessing an exon duplication, it is near impossible to create a mouse model to test therapeutic efficacy
- In partnership with Kinea Biosciences, we have opened a new avenue to test therapeutics using 2D and 3D patient tissue modeling of skeletal and cardiac muscle
- This allows CRD to dose a patient skeletal / cardiac muscle cell line and measure functional changes when mouse models aren’t possible to use

**Neutralizing Antibodies (NAb) research study tops 25 patients**
- NAbs prevent the successful delivery of AAV drugs including CRISPR. Currently estimated at 30-50% of the population and a major hurdle.
- Research study continues with over 25 patient samples analyzed for NAbs.
- Early analysis of data indicates there is limited correlation between age and level of AAV NAbs. For more info or to participate, click here.

**Manufacturing & Mouse models**
- Packaging of the plasmid continues for Terry's drug - anticipated to be complete in 3 months
- Genetic off-target study for Terry's drug has commenced - allowing us to understand the genetic safety profile of his custom drug
- Engineering begins for the 2nd wave of (4) mutation-specific mouse model intended to support preclinical development for the next wave of patients

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**SAVE THE DATE**

**Cure Rare Disease Annual Gala**

Our annual benefit gala will be going virtual this year!

**JUNE, 3 2021 • MORE DETAILS COMING**
Fundraising Efforts

We are pleased to have ended the year as strongly as we started it despite significant headwinds. The final months of 2020 and the beginning of 2021 brought some exciting press, virtual events, and fundraising efforts that we are glad to share here.

End of Year Giving

In the last three months of 2020, our grassroots efforts raised over $91,000 through our end-of-year campaign and over $150,000 through our esports efforts. Thank you to all who helped us finish the year as strong as we started.

2021 Webinars

This year, we have hosted two webinars: "What's Next for 2021?" and "The COVID-19 Vaccine's Impact on Gene Therapy" featuring experts on neutralizing antibodies. The replays are available here.

2020 Press Wrap

In 2020, we were thrilled to spread awareness for our cause in a variety of media and publications. Read about our top 10 proudest press moments of 2020 on our blog, here.
Get your monthly donation matched!

2021 is a defining year for Cure Rare Disease as we prepare to dose our first patient. Support CRD with a monthly donation and have it matched up to $45,000 this year AND receive our exclusive monthly donor newsletter with patient spotlights and research updates.