The State of Cure Rare Disease during COVID-19

A Message from the Founder

Dear All,

I hope this newsletter finds you safe and well during these trying times. With the world, and especially the rare disease community, impacted by COVID-19, community is more important now than ever. It’s critical that we come together, check-in on one another and support our family and friends in any way we can. These times are tough for everyone, our small team included. As we all know, rare disease doesn't stop for anything, and neither can we. Cure Rare Disease is adjusting our strategies to ensure our research does not slow down during these unprecedented times. Above all, I want you to know that Cure Rare Disease, and I, are here for you during this tough time. We aren't going anywhere and the science won't be slowing down.

All the Best,
Rich Horgan, Founder & President
Announcements & New Fundraising Strategies

During these uncertain times, Cure Rare Disease is staying vigilant in following all CDC guidelines on social gatherings and social distancing. This, unfortunately, means we have had to adapt and postpone several of our in-person community events. Cure Rare Disease will continue to follow CDC updates and guidelines and keep the community updated on the rest of our in-person events for 2020.

Cure Rare Disease was on The Today Show!
CRD made its first appearance on TV! If you missed it, you can see the piece here!

Signature Cure Rare Disease wine available nationally!

While we’re all getting used to this new normal, help support the mission of CRD by purchasing a case or two of specially blended and bottled Cure Rare Disease wine. We have partnered with Murphy Distributors to sell signature Cure Rare Disease red and white wine. They are now available for purchase online and shipping nationwide. Share with your wine-drinking friends! And be on the lookout for Cure Rare Disease branded spirits coming soon!
Cure Rare Disease Partners with Gaming Organizations to Raise Awareness & Support

Gaming can be a great escape for those impacted by Duchenne. Cure Rare Disease has partnered with professional video game organizations and streamers to host charity streams and fundraise on behalf of CRD. With everyone spending their time at home, there’s no better time to do some good while gaming. You can help too! Stream your favorite game with your friends! Learn more about our initiative [here](#)

If you’re a gamer like Terry, pictured below, and want to support CRD through streaming, sign up [here](#) on Softgiving! We’ll try to announce your stream over social media to support you!

![image of a gamer streaming]

Fritz&Friends Strength Is More Than Muscle Masks:

Now more than ever, it’s important to protect your loved ones from getting sick. The CDC recommends wearing a face mask to lower the chances of getting COVID-19. Our partners at Fritz&Friends are selling masks that you can check out on their [website](#). All of the proceeds go toward funding the process of developing customized CRISPR based therapy for those living with rare disease. These comfy cotton masks include their signature phrase on both adult and child masks, “strength is more than muscle.” Thank you to Fritz&Friends for making this possible! Another example of how collaboration can yield incredible results!

![image of a mask]

Shop Now!
Research Update
Our research continues to move closer to the clinic. We've provided a summary of the accomplishments below. A separate newsletter will provide even greater scientific detail soon!

Biodistribution study and exploratory mouse dosing study complete
- For our biodistribution study, the team injected the experimental mice with the AAV which included the muscle promoter and a tracer protein to measure dose response and tissue localization.
  - Results are promising and showed that the AAV and muscle promoter express well in muscle tissue. This helps to convince us that the drug is safe since it expresses where we want it to do so. Credit to Dr. Keryn Woodman.
  - A visual example of the results is provided above. It shows localization in the heart and skeletal muscle tissue (leg) of the experimental mouse.
- Moreover, our experimental mice were dosed with the experimental drug. Tissues are being analyzed for evidence of dystrophin upregulation.
- We are incredibly excited about the results – stay tuned for our science newsletter to see more!

Clinical site established for infusion
- We are proud to announce that we've established our clinical infusion site at the University of Massachusetts Medical School with Dr. Brenda Wong as our clinical investigator. We are grateful for Dr. Wong’s support in bringing her knowledge, empathy and passion to advance the paradigm of customized therapeutics.
  - “I am inspired by the potential that this customized approach holds to not only help a subset of DMD mutations in the short term, but for the potential that this new approach could enable for other DMD patients, especially those with rare mutations. This partnership with Cure Rare Disease is unique and I am excited to be part of the collaboration.” – Dr. Brenda Wong

We are only $100,000 away from being able to produce the human-grade therapeutic for our first, and unprecedented, human dosing with a CRISPR drug. Please consider supporting us in this critical time. We are incredibly close and relying on your support to get us to the first finish line! If you're able to donate, please consider doing so below. Thank you!

Donate