CURE RARE DISEASE

SPRING NEWSLETTER

2019

Dear Families & Friends,

Much has happened since our last newsletter in January. Last week, we shared an update on the big changes happening at the new, Cure Rare Disease. We're excited by the changes and our new team.

Below, you'll find the updates of our spring newsletter. As we continue to build progress and momentum, I encourage you to share our work and ask questions. As always, our goal is to distill helpful information for you and your family.

All the best,

-Rich Horgan
Founder & President
rich@cureRD.org

Cure Rare Disease: News

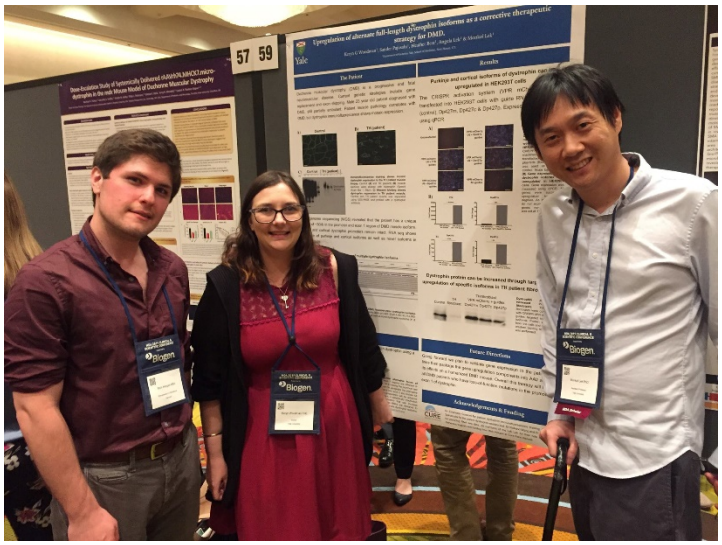
- In early April, Terry's Foundation for Muscular Dystrophy **rebranded to Cure Rare Disease**. By broadening our focus to include related rare diseases that are amenable to the customized therapy platform that our collaboration is developing, we can accelerate our progress.
    - For more details, visit our website: www.cureraredisease.org
- In addition to our rebrand, we've also **brought on staff who embody the passion of the Cure Rare Disease mission**. Please welcome Bill Patjane, our new Chief Development Officer. To read about Bill's story, and those of our other staff & board members, visit our website.
- Welcome to our new board members! **We are excited to announce the arrival of Stephanie Herzog, Carolina Alarco and Donna Izzo to the Cure Rare Disease Board of Directors**. Read more about their impressive backgrounds and accomplishments here.
- On March 22nd, **we had the opportunity to visit our friends at Exonics Therapeutics to share what it's like living with Duchenne.** Exonics is developing next generation CRISPR technology to help #endDuchenne. Thank you to our friends at Exonics Therapeutics for warmly welcoming Cure Rare Disease.

- Check out this feature on one of our collaborators, Dr. Monkol Lek. He talks about his motivations and his thoughts on the future of customized medicine. https://bit.ly/2SL3VAf

Cure Rare Disease visiting Exonics Therapeutics, March 2019

Customized Therapeutics Update

- First patient muscle cell line (patient in a dish) established and analyses to genetically and molecularly characterize it were completed.
- This critical step has enabled therapeutic development work to begin.
- With our collaborators at Yale, a custom CRISPR-construct was developed and successfully tested on the first patient's cell line.
- As was recently presented at the MDA Conference in Orlando, **our CRISPR-construct resulted in highly upregulated levels of dystrophin in the patient's cell line, back to normal levels**!
- This major achievement enables us to take the next steps as we drive towards an investigator-led clinical trial and begin to scale the approach to help others!
- **Thank you to our amazing researchers who work tirelessly to make this happen!** Stay tuned for more information as it becomes available.



Cure Rare Disease with the Lek Lab presenting results of our customized therapy development program at the MDA Conference, March 2019

Industry News

Duchenne muscular dystrophy

- The New York Times recently published an article highlighting the reality of clinical trials in Duchenne. https://nyti.ms/2TXRoi8
- Sarepta shared an update on their Phase 1 open-label study of micro-dystrophin gene therapy candidate in Duchenne. http://bit.ly/2Ou4fTo
- There was a study published recently that details how scientists were able to boost CRISPR efficiency while working to edit the dystrophin gene. https://bit.ly/2EVN7RV
- An article was published in Science Advances that describes how CRISPR-Cas9 can be used to correct Duchenne muscular dystrophy exon 44 deletion mutations in mice and human cells. http://bit.ly/2U7n18v
- Sarepta has announced that the FDA has accepted its New Drug Application (NDA) seeking accelerated approval for golodirsen (SRP-4053). https://bit.ly/2tmFe2T

Limb-girdle muscular dystrophy

- New Study Brings Good News for the People With Limb-girdle Muscular Dystrophy. https://bit.ly/2VvQEAW

Cystic Fibrosis

- Novel technique may enable personalized cystic fibrosis treatment. https://bit.ly/2GPTo3S

Sickle cell disease

- A newly published study presents new promising implications for the development of treatments in Sickle Cell Disease. http://bit.ly/2URoLUd

We need your help

Share.

Our community is rapidly growing. We encourage our friends and supporters to share our quarterly newsletter (http://www.cureraredisease.org/newsletters/) & progress with your network by word of mouth and social media. Together we can do much more and multiply our impact to end not only Duchenne but other rare diseases through customized therapeutics.

Join.

When the foundation was established in late 2017, we had a group of 5 forging the path forward. Since then, the number of people we touch, work with and benefit from has swelled into the hundreds –

ranging from the east coast to the west coast and internationally. If you are interested in working with the foundation to multiply our impact – let's talk.

Support.

To all of our sponsors and supporters: Thank You. It's with your support that in only slightly over a year, we've been able to form a world-class collaboration and **make significant achievements to develop customized medicine for our loved ones suffering from these diseases, *in time***.

For those thinking of supporting our mission, I encourage you to do so. Cure Rare Disease prides itself on having the most efficient capital utilization. No level of support is too small – from a small donation to a large donation, it all helps us get closer to ending rare disease. If you, or someone you know, is interested in running a fundraiser or donating, we are here to support you.

Upcoming Cure Rare Disease Events

Cure Rare Disease: Power in Community Conference

- Our second annual educational conference for families and caregivers will be held on July 21st at the Boston Museum of Science from 10AM – 4PM.
- We will host the leading companies, clinicians and researchers to discuss progress in the fields of Duchenne & Spinal Muscular Atrophy. This includes information on clinical trials enrollment, a chance to learn about industry developments & to get all of your questions answered.
- You can register & find more information on our new website here. Refreshments and lunch will be provided. The event is **free for families!**

Riding to Cure Rare Disease: First Annual Bike Ride

- Cure Rare Disease will host its inaugural bike ride on September 21, registration starting at 9AM. The event will take place on the Cape Cod Rail Trail and will begin at the new start of the trail in South Yarmouth. Riders will have the option to choose between a 25-mile ride or a 50-mile ride.
- Join us afterwards for what will be one of the most exciting celebrations on Cape Cod! All are welcome to join at the Sea Dog Brew Pub beginning at 1pm. Want to join but not ride? Tickets are available for purchase.
- You can find more details on the ride and event here.

Cure Rare Disease: Third Annual Gala

- Our 3rd annual gala will be on October 17th at the Boston Museum of Science. More details will be available soon but mark your calendars now!

For more information, follow & share!

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 https://www.linkedin.com/company/cureraredisease

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