CURE RARE DISEASE

Summer 2019 Newsletter

Greetings!

Much has happened since our last newsletter in the Spring. Progress in developing our customized therapeutics platform is moving forward at an accelerating rate. Moreover, we've added several new board members to help guide and grow our organization rapidly. We also welcomed a new staff member, Jenny Richard, who is leading our community engagement work. This is an exciting stage for Cure Rare Disease and we look forward to the coming months as the vision of customized therapeutics continues to approach reality. We are currently working to grow our board and event committees. If you, or someone you know, is interested in helping Cure Rare Disease grow, please reach out and let's talk!

Below, you'll find the updates of our summer 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

Foundation Updates

We released our first foundation video sharing our story and our mission. The video featured our founder, Rich, his parents and brother Terry, as well as some of our researchers. It has been incredibly well received and we look forward to continuing to spread the message and vision of customized therapeutics. A big thanks to everyone who contributed in sharing it.

A big thank you to our friends at Mirabito Energy for their recent golf classic to support CRD! This was Mirabito's 29th annual fundraiser tournament. 300 golfers attended to raise funds for neuromuscular disease research.

Customized Medicine Update

Hear from one of our researchers, Dr. Keryn Woodman who's a member of the Lek Lab at Yale Medical School, on the recent progress we've made on developing our customized therapeutic platform:

"As a postdoctoral scientist at Yale in the Lek lab I am incredibly excited to be working on this project generously supported by Cure Rare Disease. Our goal is to develop personalized therapies for DMD mutations, firstly. Over the last several months, I have been working in the lab on the first patient's cells and using a new genetic technique to boost levels of dystrophin in his cells. We have some very promising results and are repeating these experiments to get the best conditions possible before moving forward.

Our next steps will be to transition this into an AAV (adeno-associated virus) vector which has shown promise in delivering genes for sustained improvement. We will first test this approach on mice to show safety, dosage and efficacy. While to the public science can seem slow, we are really progressing quite rapidly, but ultimately we want the treatment to be safe and give the best response possible for the first patient to prove the concept and allow us to begin to scale the process to others."

With the support of our collaborators at the University of Massachusetts Medical School, we are planning to engage the FDA in the coming months in parallel with continued development. Our goal is to get their guidance on approaching our first clinical trial as efficiently as possible. Stay tuned for more updates!

New Staff

Jenny Richard - Director of Community Engagement

Jenny comes to CRD full of passion and drive. With two close friends impacted by muscular dystrophy, rare disease research strikes home for her. Jenny's extensive background in grant writing and fundraising has prepared her well to reach new fundraising milestones for CRD. Fostering relationships and spreading awareness to build a mutual passion is one of her strengths. Having raised funds for 43 types of muscular dystrophy, she has a keen sense of the critical impact of funding research. Jenny is incredibly eager and feels very fortunate to be able to support the development of cures for those impacted by rare diseases.

New Board Members

Mark Smith- Mark has over 35 years of progressive experience in all aspects of the energy industry. He currently serves at the senior executive vice president and chief financial officer of California Resources Corporation. During fourteen years of corporate and investment banking with BMO Capital Markets (formerly Bank of Montreal and affiliates), Mark held advisory roles to a diverse range of energy companies including Texaco, Union Pacific Resources, Oryx, Pennzoil, and Anadarko.

Mr. Smith holds a B.S. degree in Petroleum Engineering from the University of Oklahoma, where he was a distinguished scholar, and an MBA with highest honors from Oklahoma City University. He has also completed executive business development programs at Harvard Business School and Stanford Graduate School of Business. Read Mark's fill bio on our website.

Karen Morales- Karen Morales spent over 20 years in nationally recognized agencies solving Fortune 500 marketing and advertising challenges before she founded her own company, Marketing Magnet, in 2017. Marketing Magnet provides in-depth agency services focused on efficiency, fun, and fast results for businesses of all sizes.

Karen was also diagnosed with LGMD2b when she was a junior in college. Two decades later, she is a mom, survivor and advocate for those with health challenges. She is eager to bring hope and change to the disease community after spending so much of her time and personal resources on navigating the unpaved medical road of a rare disease.

New Partnership with YourDNA

YourDNA

To help make cutting-edge genetic research more accessible, and raise awareness of rare diseases such as Duchenne muscular dystrophy, YourDNA and CRD are joining forces. There is a great deal of misinformation and misunderstanding about the emerging technology and what it can accomplish. YourDNA and Cure Rare Disease together seek to focus on how genomics can benefit rare-disease communities. You can read more about the partnerships here.

Upcoming Events

The Power In Community Conference 2019 is an annual conference that brings together families, clinicians and pharmaceutical companies within the Duchenne and Becker's muscular dystrophy space together to learn from one another.

When: Sunday, July 21, 2019

Where: Museum of Science Boston | Registration begins at 9AM and kickoff is at 10 AM

Thank you to our Power in Community Conference sponsors!

Pfizer

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THEIR FUTURE - OUR FOCUS

More info and FREE tickets here!

For the month of August, Cure Rare Disease is teaming up with Global Partners, a family of convenience stores in New England, for a dollar ask campaign. The ad featured below will be on a donation box on the counters of hundreds of stores across the Northeast! **Find an XtraMart near you and tag us on social media with picture of our box in your store! Use #CuringRare!**

Find an XtraMart near you

The Ride to Cure Rare Disease is a 25 or 50 mile bike ride on the Cape Cod Rail Trail, beginning at Bike Zone in South Yarmouth, MA. Riders of all ages and experience levels can ride as little or as much of the trail as they like. After the ride, there will be a reception at Sea Dog Brew Pub, where riders and guests can enjoy an afternoon of free food, drinks, raffle prizes and entertainment!

When: September 21st | Registration begins at 9AM
Where: South Yarmouth, MA

Register here!

Don't want to ride but also don't want to miss out on the fun? Tickets to the reception are available!

Reception Tickets

Industry News

- Check out Catabasis's latest newsletter. They're currently recruiting boys ages 3-7 with any Duchenne mutation type for their PolarisDMD trial of "edasa". Also find useful tips on teaching young kids how to swallow capsules!
- Audentes Therapeutics has taken a license from Nationwide Children's Hospital to develop the work of Dr. Kevin Flanagan. The plan is to develop vectorized exon skipping which uses an AAV vector to deliver an antisense sequence designed to induce cells to skip over faulty or misaligned sections of genetic code, leading to the expression of a more complete, functional protein. Read more here.
- Vertex Expands into New Disease Areas and Enhances Gene Editing Capabilities Through Expanded Collaboration with CRISPR Therapeutics and Acquisition of Exonics Therapeutics. Read more here.
- Sarepta Announces Agreement with Nationwide Children's Hospital for Rights to its Gene Therapy Program to Treat Limb-Girdle Muscular Dystrophy Type 2A, the Most Common Form of Limb-Girdle Muscular Dystrophy. Read more here.
- Santhera Submits Marketing Authorization Application to the European Medicines Agency for Puldysa® (Idebenone) in Duchenne Muscular Dystrophy. Read more here.

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 thousands – 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 to save lives***.
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.

Thank you to our national sponsors!

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For more information: Follow & share!

Cure Rare Disease | http://www.cureraredisease.org/