



Hi!

I hope you are enjoying the start of fall. The rest of summer brought much desired progress on the customized therapeutic development front. We are moving full steam ahead with growing Cure Rare Disease and driving the development of cutting-edge science into the clinic so that today's patients see tomorrow's cures.

We can't wait to see everyone at our annual gala on October 24th at the Boston Wyndham Beacon Hill. Through events like these, we power the progress of customized therapeutics. Below, I've shared key updates on both the development of our science and Cure Rare Disease. I hope you enjoy the fall edition of our newsletter. As always, please feel free to reach out with questions or comments!

All the best,
Rich Horgan

Third Cure Rare Disease Annual Gala

Believe it or not, our third annual gala is right around the corner! **The gala will be held on Thursday, October 24th, 2019 in Boston at the Wyndham Boston Beacon Hill. Ticket sales end on October 21st!** The event starts at 5:30PM (VIP reception) with general reception starting at 6PM. The night will include an open bar reception, plated dinner, live music, an auction and my personal favorite, a world-renowned magician, Gary Ferrar. You can see more info and purchase tickets [here](#). **This year is particularly special as we will award one of our top researchers at Yale School of Medicine the first Cure Rare Disease fellowship for her work in advancing customized therapeutics while also debuting our second foundation video!**

The funds raised from this event will help power our first customized therapeutic into the clinic. It's truly a historic time for Cure Rare Disease and rare diseases as a whole. I hope you will join me in making history happen. If you cannot make it to the gala and still would like to support us, you can make a donation [here](#).



Thank you to our wonderful sponsors for supporting our gala!

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Research update

In the last several months, the state of customized therapeutics has advanced rapidly. We've had several major wins including:

Awarding the Lek Lab at Yale School of Medicine \$150,000 to support customized therapeutic development!



The establishment of our humanized mouse colony

Given that we are developing human-based therapies, it was critical to establish a humanized Duchenne mouse colony so that we can successfully determine the proper dose of our customized therapeutic and understand any toxicity associated with it. Thank you to our friends at UCLA and the Spencer Lab for providing the breeding mice to enable this!

Finalizing the therapeutic construct

We are finishing the validation of the therapeutic that will go into our first patient. This is a particularly sensitive and critical step as this will be the clinical agent that helps the person suffering from the disease and on which the dose-finding and toxicology studies are based.

Dose studies to begin late Q4 2019/early Q1 2020

Once the therapeutic is finalized, we will have it inserted into an AAV vector for testing on the humanized Duchenne mice. This will allow us to understand the proper dose to deliver for therapeutic benefit and the dose at which we need to understand associated toxicities.

Be on the lookout for a new publication in Nature Biotech regarding the customized therapeutic work being conducted by the Lek Lab, our investigators at Yale.

We are on track and targeting a 2020 patient dosing!

Corporate Partnerships

We are excited to share that we are continuing to scale our corporate partnerships to support the development of customized therapeutics. We recently concluded our campaign with Global Partners which ran a \$1 Ask and coinbox donation campaign through the month of August, raising over \$65,000! A BIG thank you to our partners and store associates at Global who helped make this campaign a wild success!

In addition to our recent successes, we have launched additional campaigns in Connecticut with our great partners, Mystic Oil and Murphy Distributors. Mystic Oil is generously donating 25% of profits per gallon of gas pumped at their flagship locations. Concurrently, Murphy Distributors has enlisted 100 beverage stores with donation boxes and is also donating \$5/case of select wine through November.

Be on the lookout for additional campaigns coming soon. You can read more about our current campaigns [here!](#)



Cure Rare Disease Muscles for Max Golf Tournament

A big THANK YOU and congratulations to our board member, Stephanie Herzog and the Branford, CT community for hosting the inaugural Cure Rare Disease Muscles for Max Golf Tournament on August 26th. The event hosted over 360 people and raised almost \$400,000 – all to support the development of customized therapeutics. This capital will enable the growth of our mouse colonies and help support our first dose-finding studies as we push forward into the clinic. We are already looking forward to next year's event at the Pine Orchard Country Club!

Industry news & Speaking Opportunities

Catabasis Pharmaceuticals

Catabasis is happy to share that the Phase 3 PolarisDMD trial for edasalonexent in Duchenne muscular dystrophy has completed enrollment and exceeded the target enrollment. For more information, please see the Catabasis press release [here](#).

Sarepta Therapeutics

For more information, please see the Sarepta press release [here](#).

Look for Cure Rare Disease at events coming up this fall!

- [Neuromuscular Disease Summit](#) – October 25th, 2019 – Boston MA
- [Charles River “Harnessing the Entire Immune System”](#) – October 25th – Cambridge MA



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