



Dear families and friends,

It's an exciting time in the field. With fall upon us, I want to share with you the events that happened over the summer. Below, you'll find our newsletter that talks about TFMD's efforts along with industry-wide developments. I hope you find it helpful. As always, please feel free to reach out with questions or comments. Our goal is to distill helpful information for you and your family.

All the best,

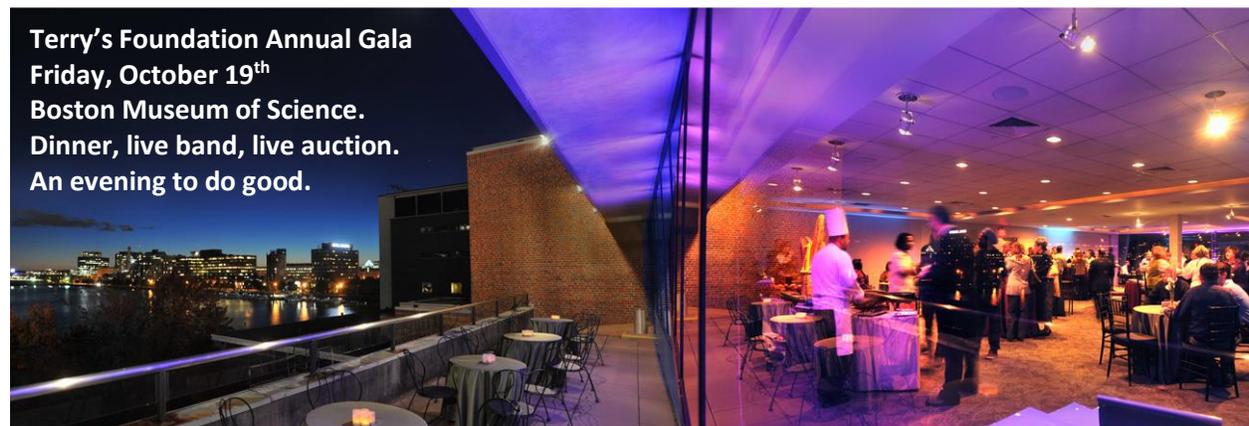
-Rich Horgan  
Founder & President

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### **Terry's Foundation Annual Gala, October 19<sup>th</sup>, 2018**

Join us for our second annual gala to help raise money for DMD research. This year, we will be hosting the gala at **Boston's Museum of Science on October 19, 2018 from 6:00-9:00PM**. The gala will feature dinner, an auction (including tickets to Hamilton here in Boston!) and a keynote address. Come join us for a fun evening overlooking the Charles! **Tickets include on-site parking and museum access!**

All proceeds raised will be directed to cutting-edge academic research.



**Terry's Foundation Annual Gala  
Friday, October 19<sup>th</sup>  
Boston Museum of Science.  
Dinner, live band, live auction.  
An evening to do good.**

For more details: <https://tfmd2018gala.eventbrite.com>

## **Terry's Foundation Pin-up Campaign**

Our first large-scale pin-up campaign is about to begin! With support of our corporate partners, Terry's Foundation is launching a large-scale pin-up campaign beginning in November through the end of the year. In hundreds of convenience stores across the northeast, customers will have the opportunity to support Duchenne research by purchasing either a \$1 or \$5 'lightbulb' pin-up. All capital raised through the campaign will go to support groundbreaking personalized therapy developments at Boston Children's Hospital and the University of Massachusetts Medical Center. Be on the lookout if you shop at Shell, Gulf, Citgo, Xtramart, Mobil or many other convenience stores around the Northeast. An illustration of the lightbulb can be found below:



## **Terry's Foundation Funded Research**

### **Kunkel Lab - \$50,000 grant for 1 year**

- This project involves two main aims targeting the delivery efficiency of cell therapy, and the Kunkel lab is utilizing both in vitro and in vivo experimental approaches. In the 6 months of Terry's Foundation funding, we have performed pilot genome-wide screens to identify impactful genes. We have generated a list of gene targets and are now doing more replicates of the experiment to finalize the list. Then, we plan to validate and test each gene independently. For the in vivo engraftment screening experiments, we established a cell line and performed in vivo screening of the whole library in mouse models of DMD. However, the efficiency has been shown to be too low for reproducibility. To overcome this, we have now established a novel in vitro 3-D system to pre-screen the library cells for better ability to cross the vasculature when delivered in vivo. Once we have a list of genes that can promote cell extravasation, we will validate individual targets in vivo in our well established mouse models of DMD.
- The pilot grant from Terry's Foundation was critical in supporting the Kunkel lab to generate this promising preliminary data, which has now resulted in successful acquisition of additional grant research funding from Boston Children's Hospital and Small Business Technology Transfer (STTR) grant from NIH.

### **Boston Children's/University of Massachusetts - Personalized Medicine**

- Terry's Foundation is establishing a large-scale research agreement with Boston Children's Hospital to conduct a proof of concept approach of the development of personalized medicine for Duchenne. Our research team believes that it is possible to develop personalized therapeutics based on an individual's specific mutation. The foundation is spearheading this collaboration to further pursue this development.

## **Industry News**

- Pfizer terminates two ongoing clinical studies for domagrozumab (myostatin inhibitor). On August 30, Pfizer reported that the phase 2 study did not meet primary efficacy endpoints. Pfizer canceled both a Phase 2 safety and efficacy study (B5161002) and an open-label extension study (B5161004). [More Details Here](#)
- Sarepta and Jerry Mendell, M.D. from Nationwide Children’s Hospital to provide update on DMD Gene Therapy Program from World Muscle Society Annual Congress. The update will take place on Thursday, October 4<sup>th</sup> at 7AM EST. For those interested in listening, the update will be broadcasted on Sarepta’s website under the investor relations section.
- CRISPR Treatment for Duchenne Muscular Dystrophy Helps Dogs. By editing cells in one-month old beagles, serving as models of the disease, researchers boosted dystrophin expression to up to 92% of normal levels in some tissues. [More Details Here](#)
- Catabasis Pharmaceuticals initiated a single global Phase 3 trial, called PolarisDMD, to evaluate the efficacy and safety of edasalonexent as a potential novel treatment for Duchenne. The trial is enrolling approximately 125 boys ages 4 to 7 (up to 8th birthday) regardless of mutation type who have not been on steroids for at least 6 months. [More Details Here](#)

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