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Getzlaf Golf Shootout, September 8-9, 2012, Dana Point, Calif.

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TREAT-NMD TACT Review



Debra Miller, founder and CEO of CureDuchenne; Kate Busby, TREAT-NMD project coordinator; and Dr. Michael Kelly, chief scientific officer at CureDuchenne, during the TREAT-NMD TACT Review meeting in Washington, DC on April 28 and April 29.

CureDuchenne was honored to participate in the TREAT-NMD TACT review for Duchenne research held on April 28 and 29 in Washington, DC. TREAT-NMD (<http://www.treat-nmd.eu/>) is a European-based network for the neuromuscular field that is focused on creating infrastructure, scientific review and guidance to ensure that the most promising new therapies reach patients as quickly as possible. It was an international cooperative meeting thanks to Kate Busby, TREAT-NMD Project Coordinator, Professor of Neuromuscular Genetics - Newcastle University, and her wonderful team, including Cristina Csimma, and many other world renowned

scientists, clinicians and statisticians. CureDuchenne and PPMD co-sponsored the meeting.

Debra Miller, founder and CEO of CureDuchenne, and Michael Kelly, Ph.D., Chief Scientific Officer of CureDuchenne, participated in the review. Dr. Kelly was able to offer his 25 years of drug development experience to the review committee.

Of the many promising research results presented at conferences, published in journals and hailed as the basis for possible future treatments and cures, only a handful progress into clinical trial. Evaluating the therapeutic potential of drugs seemingly ready for this step is a challenge not only for the patients who build hope on preclinical results and for the potential funders and industry sponsors of the research, but also for the researchers themselves. In this context, the TREAT-NMD TACT review process is a critical component of identifying, prioritizing and helping the most promising projects. [Read more information on TACT.](#)

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**Duchenne Muscular Dystrophy Research Program
(DMDRP) Review**

Debra Miller, founder and CEO of CureDuchenne, participated in the Department of Defense Duchenne Muscular Dystrophy Research Program (DMDRP) review on April 30 at Fort Detrick, Md. Representatives of the NIH, PPMD, and MDA, along with Dr. Justin Fallon, Brown University; Dr. Charles Thornton, University of Rochester; and Cristina Csimma, independent consultant, participated in the yearlong collaboration which will fund impactful Duchenne research projects. DMDRP will fund three projects this year. The committee also discussed Duchenne research priorities for next year.

DMDRP was established in fiscal year 2011(FY11) to promote the understanding, diagnosis, and treatment of Duchenne Muscular Dystrophy. The FY11 appropriation is \$4 million. The vision of the FY11 DMDRP is to extend and improve the function, quality of life and life span for all individuals diagnosed with DMD. As such, the DMDRP mission is to fund research to accelerate the development and clinical testing of new therapeutics and increase our understanding of successes and failures of therapeutics in clinical trials.

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Dealing for Duchenne Charity Poker Tournament and Auction



presented by  Raines Feldman LLP
Lawyers

Duchenne parents Chris & Amy Martin and Scott & Cathy Jones are hosting the 5th annual Dealing for Duchenne event on June 2, 2012 at 6 p.m. at the legendary Sony Pictures Studios in Culver City. The evening will sparkle with Hollywood magic and includes a Texas Hold 'Em tournament, amazing auction, special live musical guest and dinner by Wolfgang Puck.

The star-studded honorary event committee includes Joel Murray, Josh Malina, Anne Heche, James Tupper, David Foster, Seal, Paul Reiser, Parker Stevenson, Kelli Williams, Mandy Moore and Oliver Hudson. Proceeds will benefit the Center for Duchenne Muscular Dystrophy at UCLA.

The evening will honor Josh Bohbot who lives with Duchenne and his parents George and Tyla. Josh Bohbot is a 22-year-old UCLA student majoring in Design media arts. Josh was diagnosed with Duchenne at age 6 and began using a wheelchair at age 10. He loves music, game design, French cuisine and has a great sense of humor. His insight, life experience and participation with the CDMD have led the way in establishing comprehensive Duchenne care and rehabilitation and raised Duchenne awareness at UCLA. He serves as a role model for men with Duchenne and other disabilities on the UCLA campus and beyond, redefining boundaries of independence, academics and achievement. We admire his courage, strength, and positive outlook on life. His parents, George and Tyla Bohbot,



Josh Bohbot, 22-year-old UCLA student

facilitated early and continued financial support raising the first substantial gift for the CDMD at UCLA. We are very grateful for their visionary support. They are an example to us all for their admirable job of raising Josh to be the man he is, and for their tremendous support of the CDMD at UCLA; a place where their impact will continue to serve the thousands of patients diagnosed with Duchenne every year.

"We look forward to another successful Dealing for Duchenne event," said Amy Martin, co-chair of Dealing for Duchenne. "We put our heart and soul into this event because we believe real treatments are on the horizon, and we are grateful to CureDuchenne and the CDMD at UCLA for working together to provide a place where research and translational care can coexist and eventually treat our children."

One of the auction items includes a Yellowstone Club Winter Private Powder Package in Montana that includes four days of skiing at Yellowstone Club's private ski mountain and three nights lodging in a mid-mountain, ski accessible cabin.

Dealing for Duchenne is being presented by Raines Feldman LLP. Other sponsors include: Gibson Dunn, CD Photocopy Service Inc., Trace 3, O'Melveney & Myers LLP, Wolfgang Puck Catering, Bacardi USA and Heineken. Invitations were donated by ErinCondren.com.

Join us for a fabulous evening and help us raise funds to find a cure for Duchenne.

**BUY
TICKETS**

Can't attend but still want to help fund critical Duchenne research? Donate:

DONATE



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The 19,341 Foot Challenge

Eight-year-old Tyler Armstrong's goal is to become the second youngest person ever to summit Mt. Kilimanjaro. Tyler, of Yorba Linda, Calif., is climbing Mt. Kilimanjaro to raise awareness and funds to find a cure for Duchenne muscular dystrophy. He already set a record as the youngest person to climb Mt. Whitney in California in a single day the highest peak in the contiguous United States. While Tyler takes on the physical challenge of the climb from June 25 - July 2, 2012, he challenges others to donate a dollar to CureDuchenne for every foot he climbs.



Tyler Armstrong, 8, will climb 19,341 feet to the top of Mt. Kilimanjaro to benefit CureDuchenne.

"I'm climbing 19,341 feet to the top of Mt. Kilimanjaro for boys with Duchenne who can't," said Tyler. "If people donate one dollar for every foot I climb, we can raise a lot of money to help find a cure for Duchenne."

Tyler's climb is being coordinated through CureDuchenne, as part of the fourth annual [Climb to CureDuchenne: Pick Your Peak](#) event. The funds raised for Climb to CureDuchenne support the most promising research projects aimed at treating and curing Duchenne.

To learn more about Tyler visit [TopWithTyler.com](#). To support Tyler and help him reach his \$19,341 fundraising goal, [donate here](#).

[Travel Dirty](#) is the official travel sponsor for the Mt. Kilimanjaro climb. The international travel agency caters to those who want their vacations to include both stunning locations and athletic adventures.

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There is Still Time to Form a Climb to CureDuchenne Team



Last year's team Hawken's Heroes climbed Grand Teton in Wyoming as part of the Climb to CureDuchenne Pick Your Peak.

We have teams from coast to coast ready to Climb to CureDuchenne on June 23 or June 30. There is still time to form a team in your community. If those dates don't work for you, pick a date that is more convenient.

Join us for the fourth annual [Climb to CureDuchenne: Pick Your Peak 2012](#). You can help find a cure for Duchenne muscular dystrophy. It's simple, it's local and it's fun. Choose a mountain, hill or tall building to climb in order to increase awareness about Duchenne

and help raise funds to support our promising research projects and find treatments to save our sons' lives. With your help, we can make it to the peak. There are no geographical or athletic ability limitations. You pick where you want to walk, hike or climb.

We hope you'll set your own personal climbing challenge, big or small, to help us conquer Duchenne. Encourage your friends, family and colleagues to sponsor your climb. You can register and set up your fundraising page by [clicking here](#). It costs \$25 to participate, which includes a CureDuchenne t-shirt. Or be a virtual climber and [donate](#) to Climb to CureDuchenne.

To get motivated, watch our Pick Your Peak [video](#).

For more information and resources to promote Climb to CureDuchenne in your community, call Karen Harley at 949-872-2552.

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The Boys are the Champions at the Austin Gala



Mack Brown, host of the 3rd Annual Champions to CureDuchenne

This year's Champions to CureDuchenne Austin Gala was a huge success. More than 450 people attended the casual evening at the luxurious University of Texas Golf Club on April 27. They enjoyed food from amazing Austin restaurants while walking around in cowboy boots and jeans on a lovely spring evening.

Mack Brown, head coach of the University of Texas football team, and his wife, Sally, hosted the 3rd Annual Champions to CureDuchenne event presented by RBC Wealth Management. The evening included a silent and live auction and an inspirational program including remarks by Mack Brown. The event raised more than \$200,000 for research to find a cure for Duchenne.

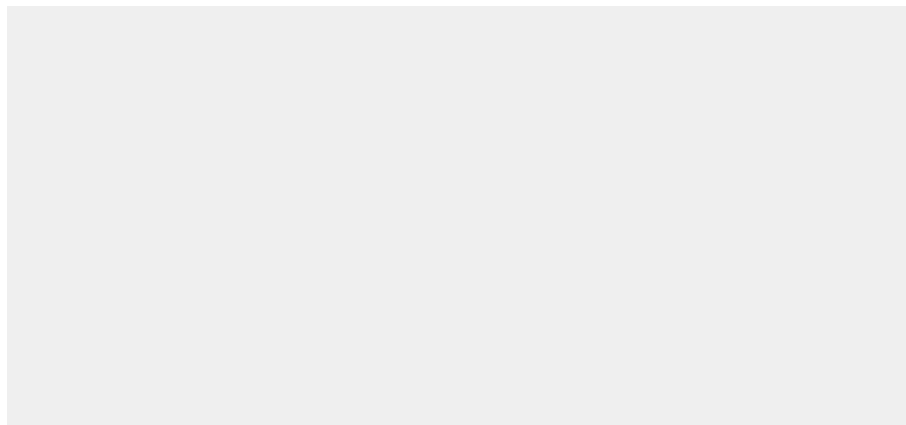
"Timothy was the highlight of the night," said Sally Brown. Eight-year-old Timothy Revell, who lives with Duchenne, thanked the guests for coming and for their support. Timothy represented the 300,000 boys worldwide with Duchenne.

"Duchenne boys are the champions for what they face every day," said Tim Revell, Timothy's father and the event organizer. "Guests were moved by the message and want to help fund research to find a cure for Duchenne."

Thank you to presenting sponsor RBC Wealth Management and other sponsors including Visa, Covert Cadillac, AT&T and Carino's Italian.

Participating Austin area restaurants included: Vince Young Steakhouse, Max's Wine Dive, Steiner Ranch Steakhouse, Zax, Cook Well & Co., The League, Moonshine and University of Texas Golf Club. The Yellowstone Club also provided a chef station. Guests enjoyed live music from the Hot Club of Cowtown, wine from Duchman Family Winery, beer from Thirsty Planet Brewing, and signature Bevo-ritas with Dulce Vida Organic Tequila.

Sponsors and restaurants were honored to be part of such a great event and support CureDuchenne's mission to raise awareness and funds to find a cure for Duchenne.



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Tim and Laura Revell (center kneeling) are surrounded by friends and supporters at the Champions to CureDuchenne Austin Gala.



Guests have a good time at the Champions to CureDuchenne Austin Gala.



Champions to CureDuchenne guests listen to the program and are inspired to help find a cure for Duchenne.



Guests enjoy the Champions to CureDuchenne Austin Gala on April 27 at the University of Texas Golf Club.

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Advocate Outreach Session



Seavey Castelli, who organized the Advocate Outreach Session in Phoenix, and her son Chase.

More than 40 people attended our Advocate Outreach Session at Phoenix Children's Hospital on April 24. CureDuchenne's Chief Scientific Officer Dr. Michael Kelly talked about the science behind the disease and how this forged CureDuchenne's mission and strategy. He discussed the latest exon skipping trial results, rare mutations and future direction for research. We received feedback from parents that the session was extremely valuable, and it also provided an opportunity for clinicians, parents and patients to connect, ask questions and get a more detailed understanding of ongoing research and development activities. Thank you to Wells Fargo for supporting the event.

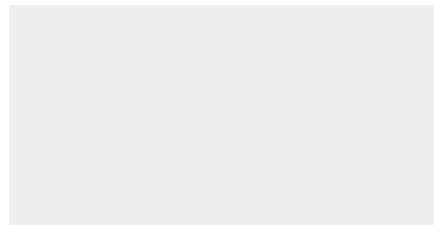
The event was organized by Duchenne parent Seavey Castelli, who is also hosting "CureDuchenne Indulge," a cupcake and wine tasting event in Tempe, Ariz. on

Friday, June 8.

Approximately 40 people attended our Advocate Outreach Session at the Hilton Garden Inn in Perrysburg, Ohio (near Toledo) on April 26. The event was organized by Duchenne parent Tammy Henegar. Dr. Michael Kelly updated the audience on current preclinical and clinical research and shared details of CureDuchenne's scientific mission and strategies. The Q&A session was very interactive and informative, and gave the audience an opportunity to cover every possible Duchenne related topic. We were so pleased that evening to meet Tom Sulfaro and his family. Tom is 40 years old and living with Duchenne. He is an incredible example of faith and positive attitude...with a very good dose of humor rolled in...a true inspiration.



Debra Miller, founder and CEO of CureDuchenne, and Tom Sulfaro, who lives with Duchenne, at the Advocate Outreach Session in Perrysburg, Ohio.





Dr. Michael Kelly, chief scientific officer, CureDuchenne and Debra Miller, founder and CEO of CureDuchenne, with Duchenne parent Tammy Henegar at the Advocate Outreach Session on April 26.

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The Revell Family



Tim and Laura Revell with their sons Andrew and Timothy (left to right).

Tim and Laura Revell, from Cedar Park, Texas, have hosted the Champions to CureDuchenne Austin Gala for the past three years. This is just one of the many ways the Revell family actively raises funds to find a cure for Duchenne.

The Revell's have two sons, Timothy, 8, and Andrew, 5. Timothy was diagnosed with Duchenne at 2-years-old. Timothy is in second grade and has an electric personality. He loves all forms of transportation such as airplanes and trains. Timothy is also captivated by weather and likes watching thunderstorms.

In addition to the Champions to CureDuchenne Gala, Tim also ran the Austin Marathon in February and carried his son Timothy over the finish line. "It was a remarkable experience," said Revell.

"There are certain moments in life when everything is right, and crossing the finish line was one of those for me and Timothy," said Tim Revell. "Timothy got to walk the finisher row, get the finisher metal and be in the finisher picture. For a moment, he ran his first marathon and will now forever be a finisher. As a parent of one of these little guys, our daily lives are full of survival, so on the day of the marathon we won a small battle and it was joy."

Recently, Revell was selected as a Duchenne parent advocate to review 13 proposals for the Department of Defense Muscular Dystrophy Research Program (see related article). He, along with other select Duchenne parents, provided an analysis of the projects worth investing in from a parent perspective. The DMDRP has \$4 million to grant for Duchenne research, which is seed money for research projects. This gave Revell first-hand knowledge of how costly it is to fund research projects.

"Our family is making the transition from planning and participating in events to trying to make contacts for even larger donations," said Revell. "Our priority is to find a way to make an even greater impact sooner. We encourage other Duchenne parents to get involved and do something big or small to help raise money to find a cure for our boys."

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Thank you Tim and Laura for all that you do to help find a cure for Duchenne.

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Jill A. Rafael-Fortney, Ph.D., is an Associate Professor in the Department of Molecular and Cellular Biochemistry at Ohio State University. She has been working in the field of Duchenne Muscular Dystrophy her whole career. She became interested in Muscular Dystrophy at the age of 6 when she saw children her own age who couldn't do the same things as she could.

Rafael-Fortney worked on a study conducted at Ohio State University that recently showed a combination of the FDA approved drugs spironolactone and lisinopril improves both skeletal muscle and heart function and histology in a mouse model of Duchenne.

Cardiomyopathy in Duchenne muscular dystrophy contributes to heart failure and is a leading cause of death. Duchenne patients show myocardial fibrosis well before functional impairment and early treatment using drugs with an antifibrotic effect may be beneficial.



Jill A. Rafael-Fortney, Ph.D., associate professor in the Department of Molecular and Cellular Biochemistry at Ohio State University.

"The funding from CureDuchenne has allowed us to test whether spironolactone can improve muscles in Duchenne muscular dystrophy mice in combination with other standard-of-care drugs," said Rafael-Fortney. "Since spironolactone is routinely used in pediatric patients with heart diseases; showing improved efficacy of this drug treatment compared or combined with other standard-of-care drugs could quickly translate to better treatments for Duchenne patients."

According to Rafael-Fortney, CureDuchenne's support has allowed them to examine many different combinations of approved drugs compared to the effect of prednisone, the standard of care, in the mdx-mouse model of DMD. Funding from CureDuchenne has accelerated this research by one or two years and has hastened its transition from the research laboratory to the clinic.

Spironolactone is being used in conjunction with Lisinopril on DMD patients once they already have shown heart problems, although Spironolactone is not being used in a preventive setting. Clinical studies are being designed in parallel to the mouse testing, and in this context they are currently planning clinical trials in collaboration with Nationwide Children's Hospital to look at the effects of spironolactone on skeletal muscle fibrosis which happens much earlier than the heart disease.

"We are in this field because we care about improving the lives of Duchenne patients," said Rafael-Fortney. "Since these drugs are already used in pediatric patients with heart disease they are likely to improve heart function and we should know their effect on DMD skeletal muscles in the next year or two."

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Calif.

Dealing for Duchenne

Champions to CureDuchenne Los Angeles Gala, June 2, 2012, Los Angeles

The 5th annual Dealing for Duchenne event is June 2, 2012 at 6:00 p.m. at the legendary Sony Pictures Studios in Culver City. The evening will sparkle with Hollywood magic and include a celebrity poker tournament and charity gambling, amazing auction, special live musical guest and dinner by Wolfgang Puck. Proceeds will benefit the Center for Duchenne Muscular Dystrophy at UCLA.

[Click here](#) to register.



"Together we can CureDuchenne"

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Getzlaf Golf Shootout,
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Point, Calif.

Champions to
CureDuchenne, Newport
Beach Gala, February 9,
2013, Newport Beach,
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CureDuchenne Indulge!, June 8 in Tempe, AZ.

Join us for an evening of cupcakes and wine pairings to benefit CureDuchenne's mission to raise funds and awareness for Duchenne muscular dystrophy on Friday, June 8 at 7 p.m. in Tempe, AZ. The evening includes a silent auction. The cost is \$50 per person. Ticket purchase includes cupcakes and wine tastings, hors d'oeuvres and two beer tickets per person. The event is being coordinated by Duchenne parents Seavey Castelli. It is sponsored by Our Lady of Cupcakes and Seavey Winery.

[Click here](#) to register.

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Climb to CureDuchenne, Pick Your Peak, June 23 and June 30, Nationwide

Teams of people from around the United States pick a mountain, hill or tall building to climb to raise funds and awareness for Duchenne. The fourth annual event features the Elite Team, led by 8-year-old Tyler Armstrong, of Yorba Linda, climbing 19,341 ft. to the summit of Mt. Kilimanjaro in Africa. Armstrong is one of the youngest to climb Mt. Kilimanjaro and is serving as the Honorary Spokesperson for the 2012 event.

- **What:** Teams of people across the country climb a mountain, hill or tall building to raise funds and awareness for CureDuchenne. This is the fourth annual event and hundreds of people will participate. All funds donated will be used for research and to raise awareness for Duchenne.
- **Where:** No geographic limitations. Anyone, regardless of athletic ability, is encouraged to participate individually or to form a team and secure personal sponsors/contributors for completing the climb.
- **When:** June 23, 2012 or June 30, 2012. If those dates do not work for you, please select another day that is more convenient. (Elite team to climb Mount Kilimanjaro June 25 - July 2, 2012)
- **Cost:** Registration fee is \$25, includes CureDuchenne t-shirts.

[Click here](#) to register

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Getzlaf Golf Shootout, September 8-9, 2012, Dana Point, Calif.

The Getzlaf Golf Shootout is a spectacular, two-day charity celebration held at the beautiful Monarch Beach Golf Links in Dana Point and Sutra in Costa Mesa. Gold medalist and NHL All-Star Ryan Getzlaf, along with a host of other NHL stars, celebrities and community leaders, will come together in support of CureDuchenne. Foursomes cost \$2,800 and include a celebrity or athlete as a fifth golfer plus eight tickets to the pre-golf dinner reception.

A pre-golf dinner and live auction will be held on Saturday, September 8 from 5:30 p.m. 9:30 p.m. at Sutra in Costa Mesa. The golf tournament is Sunday, September 9 at Monarch Beach Golf Links in Dana Point. Registration is at 9 a.m., shotgun at 10:30 a.m. and awards ceremony is at 4 p.m.

[Click here](#) to register.



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Champions to CureDuchenne, Newport Beach Gala, February 9, 2013, Newport Beach, Calif.

Save the date for the Champions to CureDuchenne Newport Beach Gala on February 9 at the Balboa Bay Beach Club in Newport Beach, Calif.

