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Hockey Players + Golf = Raising Money for CureDuchenne at the Getzlaf Golf Shootout

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The Castelli Family

### Scientist of the Month

Eric Hoffman, Ph.D

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Tailgate to CureDuchenne, Fall 2012, Austin, Texas

Tinker Bell Half Marathon, January 18-20, 2013, Anaheim, Calif.

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

## Exon Skipping Strategies for the Treatment of Duplication Mutations in Duchenne Muscular Dystrophy



*A new colony of lab mice are being bred to study exon skipping as a possible treatment for Duchenne muscular dystrophy.*

Our knowledge of exon skipping has evolved over recent years to the point where the first drugs targeting a single exon are poised for approval. This will benefit a sub group of boys with deletions that can be helped by an exon 51 skipping drug.

However, a significant number of our boys have duplicated exons; and while the science of exon skipping has continued to mature, drug development programs have not yet begun to target this population of boys. CureDuchenne

has invested in cutting edge science to bridge this gap by supporting the development of a critically needed new in vivo mouse model, as well as in vitro assays in human cell lines that will help accelerate the development of new strategies for the skipping of duplicated exons.

Two AON exon skipping drugs are currently being studied in late-stage clinical trials for deletion mutations associated with exon-51. These drugs target and skip a single exon only, and expectations are high for the first one to be approved within the next few years. This will be followed in subsequent years with additional AON drugs targeting deletion mutations associated with other exons.

A flurry of recent publications have highlighted successful multiple exon skipping in dystrophic mice and dogs that open the door to being able to treat a broader patient population with deletion mutations as well as the ability to target a subset of DMD patients that harbor one or more duplicated exons.

Unfortunately, no preclinical animal model of DMD is known to harbor a duplicated exon. To this end, CureDuchenne partnered with Dr. Kevin Flanigan and colleagues at Nationwide Children's Hospital at Columbus, to support the development of the first mouse model in which an exon is duplicated. This is intended to offer a platform for in vivo testing of duplication strategies for the first time and our goal is to make the model available to the broader research community. This mouse model carries a duplication of exon 2 within the *Dmd* locus (*mdxdup2* mouse) as this mutation represents the most common human duplication and additionally it results in a relatively severe DMD.

The group have recently succeeded in producing for the first time, male mouse pups that carry a confirmed tandem exon 2 duplication (RT-PCR analysis), and they are currently expanding this mouse colony in order to provide enough animals for detailed analysis and future studies.

They expect to establish the phenotype of the *mdxdup2* mouse early next year, assessing dystrophin expression in skeletal muscle, as well as assessing the severity of muscle disease using physiological and pathological assessments as the mice grow and mature. Once adequate numbers of male *mdxdup2* mice are available, the group will immediately begin (for the first time) the key exon skipping studies using both antisense oligomers as well as U7 snRNA in an exon-duplication model. But this is only the beginning.

In addition to the new mdxdup2 mouse model, CureDuchenne is supporting the group to develop and characterize patient-derived fibroblast cell lines representative of a broad variety of duplicated exons. This critically important step will add to our knowledge of skipping duplicated exons from patient derived cells and will allow us to test the effectiveness of different exon skipping strategies, as well as the efficiency of U7 snRNA-mediated exon skipping on human duplication mutations.

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## CIRM Awards \$6 Million Grant for Duchenne Research at UCLA

Congratulations to Dr. Stanley Nelson, Dr. Carrie Miceli and Dr. Michelle Spencer, all from the Center for Duchenne Muscular Dystrophy at UCLA, who received a \$6 million grant from the California Institute of Regenerative Medicine (CIRM) to develop a combination therapy for Duchenne muscular dystrophy. The study will use patient-derived stem cells as a novel therapeutic strategy.

Many parents and advocates, including CureDuchenne, successfully appealed for this funding during the last CIRM meeting in Sacramento. CureDuchenne continues to support all the great work being done at the Center for Duchenne Muscular Dystrophy at UCLA.

For more information, [click here](#).

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## Join the CureDuchenne Crusaders in the Tinker Bell Half Marathon on January 20, 2013



*"All you need is faith, trust and little bit of pixie dust."* Tinker Bell



A glimpse at the 2011 Tinkerbell 1/2 Marathon.

We are on the run of our life to find a cure for Duchenne muscular dystrophy.

Join the CureDuchenne Crusaders team and run the Tinker Bell Half Marathon on January 20, 2013 to raise awareness and funds to find a cure for Duchenne. With your participation, you can support CureDuchenne and raise money to fund critical research to help save this generation of Duchenne boys.

Your donations will fund research and can help turn that pixie dust into viable treatments and ultimately a cure for Duchenne. Even though Peter Pan never wants to grow up we want to give Duchenne boys that opportunity.

For more information and to register, [click here](#).

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**Hockey Players + Golf = Raising Money for CureDuchenne at the Getzlaf Golf Shootout**



Ryan and Paige Getzlaf with Debra and Paul Miller, co-founders of CureDuchenne, and their son Hawken Miller, 15 who lives with Duchenne muscular dystrophy.

**Ryan Getzlaf**, captain of the Anaheim Ducks, and his wife Paige, were gracious hosts of the second annual Getzlaf Golf Shootout on September 8 and 9 to benefit **CureDuchenne**. The Getzlaf Golf event raised nearly \$200,000 to help fund research to find a cure for Duchenne.

The weekend kicked-off with Getzlaf and other Anaheim Ducks and Los Angeles Kings hockey players, celebrities and community leaders at a pre-golf party at Sutra in Costa Mesa. More than 185 people partied with the players; enjoyed a buffet dinner and cocktails; bid on silent and live auction items; and pledged support to CureDuchenne.



Silent auction items included autographed Anaheim Ducks jerseys.

Kent French, the voice of the Anaheim Ducks, welcomed the crowd and introduced Hawken Miller, son of CureDuchenne founders' Paul and Debra Miller.

Hawken, 15, congratulated Getzlaf for being "a good role model on and off the ice" and thanked him for helping to raise awareness and funds to find a cure for Duchenne.

Getzlaf shared how they met the Millers and learned about CureDuchenne. They were touched by how Duchenne impacts young boys. As parents, with a young son and another child on the way, Ryan and Paige wanted the Getzlaf Golf Shootout to benefit CureDuchenne to help give this generation of Duchenne boys a chance.

The next day, this spectacular charity event teed-off at the Monarch Beach Golf Links in Dana Point. Each golf foursome included a pro athlete or celebrity as a fifth player such as:

- Ryan Getzlaf, Anaheim Ducks

- Bobby Ryan, Anaheim Ducks
- Teemu Selanne, Anaheim Ducks
- Sheldon Souray, Anaheim Ducks
- Scott Neidermayer, Anaheim Ducks (retired)
- Dustin Penner, Los Angeles Kings
- Mike Richards, Los Angeles Kings
- Bruce Boudreau, coach of the Anaheim Ducks
- Kent French, the voice of the Anaheim Ducks
- Paul Bissonnette, Phoenix Coyotes
- Kevin Connolly, actor

## Getzlaf Golf Shootout Benefits CureDuchenne

It was a gorgeous day on the golf course. In addition to a great round of golf, players also enjoyed fun activities on the greens including a putting contest using a hockey stick; a black jack table; closest to the pin contest; longest drive contest; opportunity to win a Maserati, BMW or Harley Davidson on three hole-in-one holes; and the popular dunk tank. Golfers also enjoyed food and drinks throughout the course.



*Actor Kevin Connolly of Entrourage throws a ball at dunk tank during the 2nd Annual Getzlaf Golf Shootout to benefit CureDuchenne.*



*Joey Sparks, Scott Sawicki, Scott Neidermayer (retired Anaheim Ducks player), Brian Bachhuber and Jim Sardina are ready to tee-off at the 2nd Annual Getzlaf Golf Shootout to benefit CureDuchenne.*



*Paige Getzlaf, Raymond Swain, Sami Vatane (Anaheim Ducks), John and Marianne Bonciatto, Mark Harding are the winning foursome, along with Ryan Getzlaf and Ryder at 2nd Annual Getzlaf Golf Shootout to benefit CureDuchenne.*

*CureDuchenne's co-founders Debra and Paul Miller, and their son Hawken, are surrounded by Anaheim Ducks and Los Angeles Kings hockey players who came out to play golf for Ryan and Paige Getzlaf's 2nd Annual Getzlaf Golf Shootout to benefit CureDuchenne.*

Thank you to our sponsors [The Sports Corporation](#), [WHGC](#), and [Garth Brooks Teammates for Kids Foundation](#). Supporters included The Hundred Acre Wine Group and Straub Distributing. Hole sponsors included: Tres Sietes/Cabo Chips, Deuce Brand, Metrix, Integrated Oncology Network, Eaglerider, The Meathouse, Jagermeister, Straub Distributing, Realty Co-Op, The Cannery; Manly & Steward, Dr. Alexandra Chebil, CNC Motors and Ferrari of Newport Beach. We appreciate your support and generosity.

Check out some more photos from the event:

[Reception & Auction at Sutra](#)

[The Shootout at Monarch Beach Golf Links](#)

[Photos of the Foursomes at the Shootout Tournament](#)

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**Family of the Month - The Castelli Family**



From left to right: Jim, Chase, and Seavey Castelli

Seavey and Jim Castelli have a license plate that says CureDMD. That is one of the many ways that they get the word out about Duchenne muscular dystrophy. Their 5-year-old son, Chase, was diagnosed with Duchenne at the age of three.

The Castellis were heart broken when Chase was diagnosed with Duchenne but they were not surprised. Chase had delays in walking and talking and had large calves. The Castellis researched and asked their pediatrician if Chase could possibly have Duchenne.

Two pediatricians said no, if it doesn't run in your family you don't need to worry about it. How wrong they were.

"Watching your child live with a progressive disease is torture," said Seavey. "We try to make the most of every day with Chase while he can still do things. You learn not to take life for granted."

Chase is very energetic and active for a Duchenne boy. He loves jumping and swimming. Chase likes to draw, watch movies and play with Legos. In addition to Duchenne, Chase also has autism. This makes it hard for him to understand why he has to do therapy and stretching and often wants the "boots off" at night.

The Castellis try to educate everyone they meet about Duchenne. "I talk to customers, strangers at the grocery store or the guy who gave us a quote for new counter tops about what Duchenne is and how it affects our son," said Seavey. "We all need to be advocates for Duchenne boys. If all Duchenne parents, grandparents and other family members would talk to one new person every day about Duchenne, think about the impact we would have."



The Castelli Family's CureDMD license plate.

People need to know about the disease to fund Duchenne. “Even though Duchenne is a rare disease it is taking the lives of too many boys,” said Seavey. “Just think of how much closer we could be to treatment if there was more funding.”

In addition to increasing awareness of Duchenne, the Castelli’s have helped raise funds for CureDuchenne. They hosted a cupcake and wine fundraiser in April and are organizing a “Climb to CureDuchenne: Pick Your Peak” climb in November in Scottsdale, Ariz. They are also planning a piano bar fundraiser in the fall. Chase’s cousin, Connor Pyle, has been spreading the word and fundraising for CureDuchenne while on the Legend Car driving circuit.

“You can’t give up hope because there are so many potential treatments,” said Jim Castelli. “The sooner they find a cure the better. My hope is they find a treatment and Chase lives a long, happy life.”

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## Scientist of the Month - Eric Hoffman

This month we have a featured Q&A with Eric Hoffman, Ph.D, director, Research Center for Genetic Medicine, George Washington School of Medicine and Health Sciences, Children's National Medical Center, Washington DC. Dr. Hoffman shares his insights on where we are with Duchenne research.



*Eric Hoffman, Ph.D*

**Q: Tell us about the projects you are currently working on.**

**A:** We have about 50 clinicians, scientists, and clinical trialists working specifically on DMD, covering many topics from what dystrophin normally does in muscle and what goes wrong when it is missing (basic science), developing new approaches to therapy (translational research), and bringing potential therapies to clinical trials (clinical research). We have also spun off a drug development company focused on DMD (ReveraGen Biopharma), and work with Sarepta on development of exon skipping (pharmaceutical projects).

Our two major pharmaceutical projects are on VBP15 and exon skipping. VBP15 (ReveraGen Biopharma) appears to have a number of activities that appear beneficial to dystrophin-deficient muscle. It is an inhibitor of NFκB (a key inflammatory pathway in dystrophic muscle), and helps stabilize dystrophin-deficient muscle plasma membranes. Pre-clinical studies in mouse models have shown impressive results, and we expect to be in human trials in 2013. With regards to exon skipping we work closely with Sarepta on a number of projects. We lead Department of Defense supported projects that enabled the IND for the current exon 51 phase 2 trial (Jerry Mendell), and are similarly working on bringing exon 45 drug to trial. We run two large NIH-supported projects on exon skipping one optimizing dose schedules, and a second looking at predicting the clinical response to different skipped (Becker-like) dystrophin proteins. The latter includes the first large natural history study of Becker muscular dystrophy through our CINRG clinical trial network ([www.cinrgresearch.org](http://www.cinrgresearch.org)). CureDuchenne helps support these pharmaceutical interactions via support of Abby Bronson (previously of MedImmune) in our shop (together with Foundation to Eradicate Duchenne).

Our clinical and translational research projects include pre-clinical trials via a large DMD mouse drug testing facility (run by Kanneboyina Nagaraju) (about 40 trials done to date), and the Cooperative International Neuromuscular Research Group (CINRG) clinical trial network. The CINRG group is working on clinical and biomarker outcome measures for clinical trials in DMD, trains a network of clinical evaluators for trials, and assists pharmaceutical companies with designing and running their trials in DMD. I serve as Scientific Director of CINRG, Paula Clemens at Pittsburgh is the Medical Director, and Avital Cnaan in our shop runs the statistical and coordinating center for CINRG.

**Q: What are the goals and objectives of your research?**

**A:** To make DMD a much less severe disease. I made a commitment to myself to do this many years ago, otherwise I'm not allowed to retire.

**Q: Where are we currently with research related to exon skipping?**

**A:** All the animal studies done with the morpholino chemistry suggested that this approach should work well in DMD patients, including our trials in DMD dogs with Shin'ichi Takeda's group in Tokyo. The data from both Francesco Muntoni's and Jerry Mendell's trials are what we would expect to find, and all this is extremely encouraging. We need more trials with more exon skipping drugs in more DMD patients, and all this is expensive, but these are 'good shots on the goal'.

**Q: What are you excited about in terms of Duchenne research?**

**A:** I've been in DMD research a long time (since 1986), where I worked with Louis Kunkel as a post-doc to help identify the DMD gene and identify dystrophin. Once the gene and protein were identified, many folks said, "We know what causes DMD; we're done!" At the time, I felt, "Whoa, what about the DMD patients?!" The next 15 years was tough; funding was thin, and research seemed to be more similar to betting on horse racing than a 'broad healthy portfolio'. The last 10 years has seen a tremendous turn to a bright new future lots of really good approaches by a lot of different groups internationally, with some impressive support from many governments and foundations. The coordination and cooperation between groups is also impressive now (like it was in the 1980's). I guess I'm excited about everything Duchenne now!

**Q: What is your hope?**

**A:** That soon we'll just remember what DMD used to be like.

**Q: What are the next steps?**

**A:** INDs, trials, INDs, trials, repeat!

**Q: How did CureDuchenne support help you?**

**A:** Academic/industry interactions that are smooth and 'trans-culturally correct' are critical to move DMD therapeutics forward. CureDuchenne recognized this early on, and has helped support Abby Bronson at Children's National Medical Center to facilitate our interactions with industry. CureDuchenne also provided a critical loan to ReveraGen as it was getting started. This helped us leverage significant support from both NIH TRND program, and MDA Venture Philanthropy. Children's National Medical Center led a collaboration with CureDuchenne and the Foundation to Eradicate Duchenne to fund preclinical work for Sarepta (formerly AVI BioPharma) to proceed to human clinical trials.

**Q: What role does CureDuchenne play in drug development and discovery?**

**A:** CureDuchenne has been forward thinking in fostering industry involvement with DMD, such as the impressive Prosensa/GSK exon skipping initiatives.

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## Tailgate to CureDuchenne, Fall 2012, Austin, Texas

"We're Texas Tailgaters" are preparing for another exciting year of tailgating before University of Texas home football games. Join fellow football fans for food and refreshments at 15th & Trinity in Centennial Park near the University of Texas Tennis Courts.

"We're Texas Tailgaters" is supporting CureDuchenne, a nonprofit that raises awareness and funds research to find a cure for Duchenne muscular dystrophy.

"We're Texas Tailgaters" is growing in size and anticipate more than 130 people per home game. This is a high traffic area for tailgate parties so be sure to look for CureDuchenne's banner. Stop by for some fun and help out a good cause.

Home Game Tailgate Schedule:

- **October 10th:** West Virginia
- **October 20th:** Baylor
- **November 10:** Iowa State

For more details or questions, please contact Ivan Vires at 512-296-3752 or the CureDuchenne office at 949-872-2552.

[Click here](#) for more information or to donate.

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## Tinker Bell Half Marathon, January 18-20, 2013, Anaheim, Calif.

CureDuchenne is proud to be an official charity of the Tinker Bell Half Marathon. The Tinker Bell Half Marathon weekend is January 18-20, 2013.

**Disneyland® Resort** becomes Never Land for a magical weekend. It starts with a Family 5K and a Kids' Races and culminates with 13.1 mile run that weaves through **Disneyland® Resort**. To register, [click here](#) or for more information call CureDuchenne at 949-872-2552.



*"Together we can CureDuchenne"*

September 2012

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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, 2013 at the Balboa Bay Beach Club in Newport Beach, Calif.