

## Research

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Editing Restores Dystrophin  
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Raises More than \$11,000

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## Family of the Month

The Cook Family

## Scientist of the Month

Thomas Rando, M.D.,  
Ph.D.

## Upcoming Events

Saks Fifth Avenue  
Shopping Extravaganza,  
June 15-19, Costa Mesa,  
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Knoxville Brewfest, June  
29, Knoxville, Tenn.

Getzlaf Golf Shootout,  
September 7 and 8, 2013,  
Costa Mesa and Dana  
Point, Calif.

Dealing for Duchenne  
Philadelphia, November 16,  
2013, Philadelphia

Champions to  
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On May 24, [Prosensa filed for an IPO on NASDAQ](#). Prosensa is an early pioneer in exon skipping for Duchenne and has partnered with Glaxo Smith Kline since 2009 to develop their lead compound, drisapersen. CureDuchenne will watch closely to see the impact this has on additional drugs that Prosensa has in development. CureDuchenne is very encouraged that the financial markets are taking notice of companies that develop drugs for Duchenne. The expertise and resources that biotech and pharmaceutical companies bring to drug development will be crucial in order to commercialize Duchenne treatments.

This has been an exciting and hopeful time for families who are affected by Duchenne. Recent developments have shown how much promise exon skipping has for this devastating disease and how close we are to actually having drugs to treat it. CureDuchenne funded Prosensa in 2004 to develop their drugs for Duchenne. And, in 2010 when AVI Biopharma, now Sarepta, was on clinical hold, CureDuchenne collaborated with Children's National Medical Center and Foundation to Eradicate Duchenne to provide the funding necessary for the company to do the studies which enabled them to progress to human clinical trials.

CureDuchenne will continue to think outside the box in order to get treatments to all the Duchenne patients that so desperately need them. CureDuchenne is proud to be pioneers in exon skipping with both Sarepta and Prosensa and we expect that this disease will have treatments in the future. The question is how many of our sons must die before we get these drugs into our children? A lot depends on aggressive thinking and funding.

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## Reading Frame Correction by Targeted Genome Editing Restores Dystrophin Expression in Cells From Duchenne Muscular Dystrophy Patients

A recent paper from Gersbach et al. ([Molecular Therapy, 4th June, 2013](#)) entitled "Reading Frame Correction by Targeted Genome Editing Restores Dystrophin Expression in Cells From Duchenne Muscular Dystrophy Patients" highlights the recent advances in approaches to correct genetic mutations in patient derived cells using an engineered nuclease.

The study exploits the effect of two separate TALEN's, (an engineered fusion protein known as a transcription activator-like effector nucleases) that work together to selectively identify and bind specific sequences of DNA, ultimately leading to the creation of a double strand break in the region that the TALEN's bind. This targeted break in the DNA stimulates the cells own repair mechanisms and leads to the formation of multiple new forms of the original DNA that contains microinsertions and microdeletions at the previous DNA breakpoint.

In Duchenne muscular dystrophy, the deletion of certain "in-frame" exons (particularly in the long central rod domain) has been associated with the much milder form of the disease known as Becker muscular dystrophy. This flexibility of function of the dystrophin protein is important, because it is critical to the approach used by Gersbach et al. The micro changes in the DNA (insertions and deletions) imparted by the TALEN approach need to produce a functional form of novel dystrophin.

To demonstrate its utility, the team designed TALEN's that targeted exon 51 of the dystrophin gene and transfected cells with the TALEN encoded plasmids. They identified a specific TALEN pair, designated TN3/8, which were active and well tolerated in the cellular assay. TN3/8 was used to correct the reading frame and restore protein expression in immortalized human myoblast cells derived from a Duchenne patient with a reading frame-disrupting deletion of exon 48 to exon 50. Similar dose-dependent results were obtained in primary DMD dermal fibroblasts carrying a reading frame-disrupting deletion of exon 46 to exon 50. This is an exciting and powerful example of gene editing technology that could provide a permanent gene correction approach and offer an alternative strategy to antisense based exon skipping drugs.

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Getzlaf Golf

**Join Ryan Getzlaf at the 3rd Annual Getzlaf Golf Shootout**



*The 3rd Annual Getzlaf Golf Shootout will be held at Monarch Beach Golf Links in Dana Point, Calif. on September 8*

Ryan Getzlaf, captain of the Anaheim Ducks, is hosting the third annual [Getzlaf Golf Shootout](#) on September 7 and 8 benefitting [CureDuchenne](#). The Getzlaf Golf Shootout is an opportunity to interact with your favorite players, play golf and support a great cause. This two-day charity golf event includes a reception at Sutra Lounge in Costa Mesa and a golf tournament held at the Monarch Beach Golf Links in Dana Point. Each foursome will be teamed up with a professional athlete or celebrity for a unique and exciting day on the green.



*CureDuchenne Co-founder Paul Miller and Anaheim Ducks Captain Ryan Getzlaf at last year's Getzlaf Golf Shootout*

"Duchenne is a devastating disease that impacts boys and robs them of the ability to walk, run and play sports," said Getzlaf. "My wife, Paige, and I are committed to support CureDuchenne and their efforts in finding a cure for Duchenne. It is urgent to find a cure now in order to give these boys a chance. We hope you'll join us at the golf tournament and provide hope for the 300,000 boys living with Duchenne."

The Getzlaf Golf Shootout brings together athletes, celebrities and community leaders teaming up in support of CureDuchenne. The course will include a variety of fun activities including a hockey stick putting contest, longest drive contest and dunk tank as well as plenty of food and drink. Click [here](#) to

watch a video of last year's event.

Foursomes cost \$2,800 and include a celebrity or athlete as a fifth golfer plus eight tickets to the pre-golf dinner reception. Meet and mingle with hockey players at the reception on September 7 at Sutra.

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There will be a live and silent auction. Individual tickets to the reception are \$100. Click [here](#) to register now. For sponsorship information, please contact Karen Harley, 949-872-2552 or [karen@cureduchenne.org](mailto:karen@cureduchenne.org).



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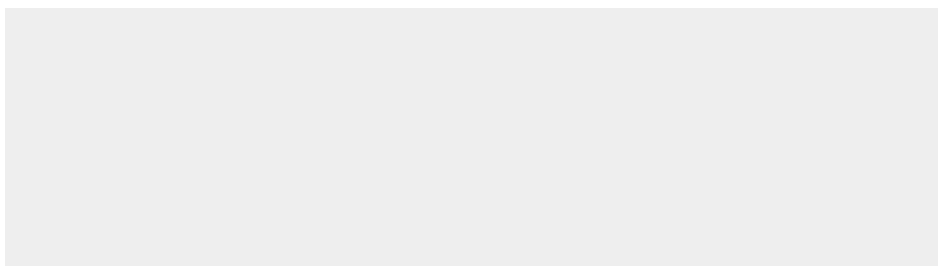
Wil Cook, Brian Cook, Tiffany Cook, Nikki Sixx from Mötley Crüe, Paul Miller, Debra Miller at Rockin' to CureDuchenne.

More than 200 people were Rockin' to CureDuchenne on June 2 at Stonegate Elementary School in Irvine, Calif. The benefit concert raised more than \$11,000 to help increase awareness and fund research to find a cure for Duchenne. Rockin' to CureDuchenne featured three local Orange County bands who donated their time and talent to perform a family-friendly concert.

Rockin' to CureDuchenne included a surprise appearance by Nikki Sixx, co-founder and bassist of the band Mötley Crüe. Sixx generously donated and autographed his guitar for CureDuchenne to auction off at a later date to raise additional funds.

Rockin' to CureDuchenne was organized by Tiffany Cook, whose son Wil lives with Duchenne, and Harmony Hager, Wil's sixth grade teacher at Stonegate. Wil celebrated his 13 birthday at the event.

The concert kicked off with the band The Hager Boyz. Band members include four Hager brothers and their dad who write and play their own original music. The second set was Johnny and the Gentlemen, a local pop rock band. The third band, Breach the Summit, is an indie rock band with members from Northwood High School. The bands were jamming and had the crowd on their feet during the concert.



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*The Hager Boyz perform at Rockin' to CureDuchenne.*

In between sets, concert goers enjoyed food and drinks provided by the Irvine High School football team. The high school student volunteers manned the snack bar. The event also included raffle items and a silent auction.

Click [here](#) to view a video clip of Rockin' to CureDuchenne.

Rockin' to CureDuchenne sponsors include ART Janitorial Services, KIP, McNeill Travel Plaza, Tim Horn, Title Associates and Wells Fargo. The following companies donated auction or raffle items: Big Bee Photography, Chick-Fil-A (Woodbury Town Center), Jon Pettey Custom Goldsmith, Sport Clips (Woodbury Town Center) and Carl's, Jr. (Woodbury Town Center).

Thank you to Stephen M. Hager and Danny Gray for donating their time as stage manager and sound engineer respectively.

Thank you to the faculty and staff of Stonegate Elementary for hosting Rockin' to CureDuchenne. Wil's educators volunteered at the event and donated an auction item.

"We want to give a huge shout out to Tiffany and Harmony for creating and organizing Rockin' to CureDuchenne," said Debra Miller, founder of CureDuchenne. "It is events like this that help get the word out about Duchenne and allow us to fund research to find a cure for this devastating disease."

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**Time to Climb to CureDuchenne**

Climb to CureDuchenne season has begun. Several teams have already held their walk/hike/climb. Other teams are picking their peak and planning for their upcoming climbs.

**Team Altura: Carter's Climbers**

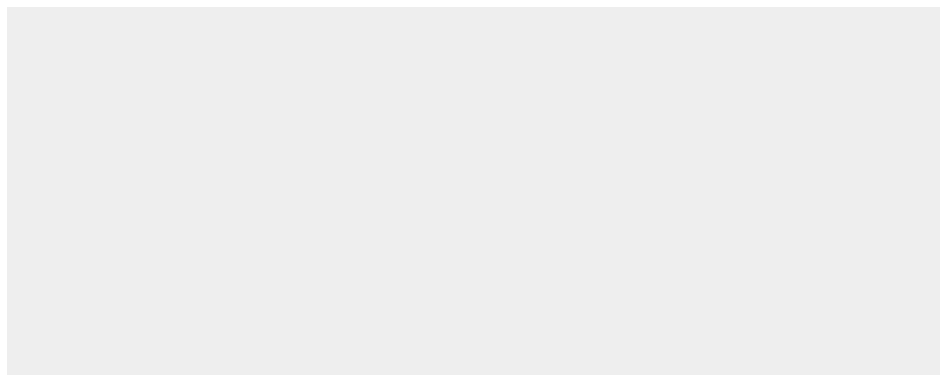


Team Altura: Carter's Climbers

Rhiannon Ramkisson organized Team Altura: Carter's Climbers for the third year in a row. Forty-eight people participated in the family-friendly hike to beautiful Mt. Rubidoux in Riverside, Calif., on June 1. The hike began at Altura Credit Union's 14th Street branch in downtown Riverside. The 5-mile loop was suitable for all fitness levels and stroller/wheelchair accessible. As part of the fundraising effort, the team held a bake sale and sold refreshments to raise much needed funds for research for Duchenne muscular dystrophy. Team Altura: Carter's Climbers raised nearly \$2,800.

"My 9-year-old son Carter has Duchenne muscular dystrophy. It is devastating and 100% fatal," said Ramkisson. "Our family was shocked to learn the diagnosis, less than a month after I started with Altura Credit Union in July 2007. I've been incredibly blessed by the support of my Altura family throughout our journey (which is only just beginning) and honored that we came together as Team Altura: Carter's Climbers to raise money and awareness for Duchenne."

**Team Bailey**





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*Team Bailey*

Jacqueline Hislop, Preschool/Grade 2 Special Education Teacher at Bailey Elementary School in Lowell, Ma, organized a Climb to CureDuchenne event. There are two students at Bailey Elementary School with Duchenne. Team Bailey hiked the Welch-Dickey trails in Waterville Valley on May 26.

We encourage you to pick a mountain, hill or tall building to climb to raise funds and increase awareness of Duchenne. Anyone, regardless of athletic ability, is encouraged to participate individually or to form a team and secure personal sponsors/contributors for completing the climb.

Click [here](#) to register your Climb to CureDuchenne team.

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**Family of the Month - The Cook Family**

*Tiffany, Wil, Emily and Brian Cook*

When Wil Cook was diagnosed with Duchenne at age 7 the Cook family lived in Florida and didn't know a single person with Duchenne.

"It was the most horrific day and timeframe of my life," said Tiffany Cook, Wil's mom. Cook is a speech-language pathologist who works with special needs children primarily with autism. "I never thought in a million years that I would become the parent of a special needs child."

The Cook family Brian, Tiffany, Wil and sister Emily moved to Irvine, Calif., three years ago and have met quite a few Duchenne families since then. They have also created a strong network of friends, colleagues and neighbors.

"I have three boys in my life with Duchenne: my son, my friend, and my student," said Cook. "This is pretty amazing for such a rare disease. When we were in Florida we felt more isolated because we didn't know anyone else who was living with Duchenne. Now we have made connections with others who are going through the same challenges we are."

Wil, 13, has had a rough period the last several months.

"I watch Wil every morning get out of bed and it is a struggle," said Cook. "He has to figure out how to get his body out of bed."

The Cook family has been actively fundraising for CureDuchenne since moving to Southern California. They organized a Climb to CureDuchenne event last year and just recently organized a Rockin' to CureDuchenne benefit concert (see related article) to help raise awareness and fund research to find a cure for Duchenne. The Cooks have also attended the Champions to CureDuchenne Newport Beach Gala and the Scientific Summit the past two years.

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The Cook family is featured in the CureDuchenne [video](#). The video had a big impact on the Cook's network of family and friends as well as others who have seen it.

"I decided to not only share the video with family and friends, but to, for the first time, include the families of the children I service," said Cook. "This video captured the essence of what all parents will do, no matter the disability, for their children."

Here are just a few of the comments the Cook's received after people viewed the video:

*"This video touched my heart and taught me a lot about Duchenne. I'm in awe of the determination and courage of the boys, their families, and their parents- perhaps especially the parents! I hope the video has a far reaching impact."* -A coworker

*"I pray that there will be a cure for Duchenne - the positive outlook you and your family will be a reality. We will be making a donation."* -Parent of son with Autism whom I am the Speech Therapist

*"The tears of sadness for the children fade with the hope of a cure. It's a tremendous blessing to see a cure be available soon. My donation may not be huge but I know the power of everyone getting involved so I have forwarded to those I know. I am sure God will provide for your family, and will pray for the children facing this. I am rooting for you and them!"* -Parent of child with Down syndrome whom I am the Speech Therapist

*"I cried watching it. I also shared link to others. Hope to find the cure soon."* -Parent of a friend of Wil

*"I truly appreciate you bringing this to my attention. This is all you said, in addition to the Kids, it also presents the parents, who in my view are so underappreciated for what they endure in emotions and worry, yet go from day to day giving all they have in loving and caring for their kids, rarely revealing the concern and worry each of you carry with you each day. Hopefully this message of hope will connect with those of extraordinary means who with a few words and actions can bring more attention to the strides made toward a treatment and the funding needed to make hope a reality."* - Wil and Emily's Pediatrician

*"This video brought tears and sadness, but in the end I felt a great deal of hope!"* -A mom of a friend of Wil

*"What brave families and especially the boys afflicted by this disease!"* -Family friend

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## Scientist of the Month - Thomas Rando, M.D., Ph.D.

Thomas Rando, M.D., Ph.D., professor of neurology and neurological sciences at Stanford University School of Medicine and director of Stanford's Glenn Laboratories for the Biology of Aging, has focused his entire career researching muscular dystrophy.

The main areas of interest of the [Rando Laboratory](#) are muscle stem cell biology, muscle stem cell aging, muscular dystrophies, tissue engineering and basic muscle cell biology. Dr. Rando's research focuses on the restorative and repair mechanism of stem cells. The lab has a long-standing interest in understanding the mechanisms of cell injury and cell death in muscular dystrophies and the development of novel therapeutics. The long term goal is to develop stem cell therapies for Duchenne muscular dystrophy.

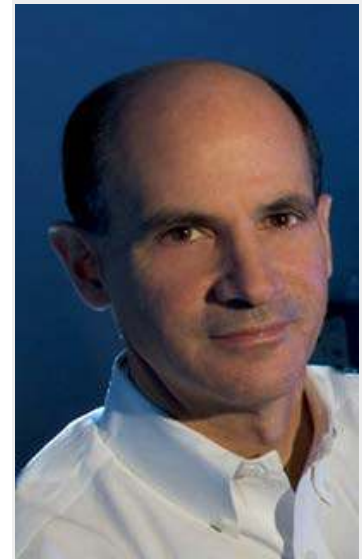
The Rando Laboratory has seen success in mouse models. They have obtained muscle biopsies from patients, isolated them and grown them extensively to transplant the cells into mice to test them. Their research on muscle stem cells is turning toward therapeutic applications. A major emphasis of their recent effort is pre-clinical studies of using muscle stem cells in models of cell transplantation to treat muscular dystrophies with an eye toward future human trials.

Dr. Rando is the senior author of a study that was published online recently in the Journal of Clinical Investigation. His lab created a mouse model of muscular dystrophy in which regenerating muscle tissues gives off visible light from luciferase, the protein that causes fireflies' tails to glow. When the luciferase gene is activated in a cell it glows whenever the mice are given a compound that gives off light in the presence of luciferase. So as the muscular dystrophy progresses the muscle that is being repaired becomes increasingly luminescent. This mouse model paves the way to quicker, cheaper and more accurate assessment of the efficacy of therapeutic drugs. It is already being used to test stem cell and gene therapy approaches for muscular dystrophy. Click [here](#) to read the abstract.

"These are reporter mice because they report on a disease progression," said Dr. Rando. "Using mice allows us to see the progression of the disease in a compressed time scale and be able to test other approaches."

Research takes time but progress is being made in the Duchenne field.

"We know the time pressure for parents with children with Duchenne is profound and that sense of urgency is shared by scientists," said Dr. Rando. "I know the slow pace of research can be frustrating. You never know which avenues will lead to treatments and sometimes they come from avenues that are unexpected. That is why it is good to do a combination of both targeted therapeutic research and basic research that could lead to new therapeutic opportunities. We are optimistic that stem cell therapies will be in more and more trials that will make a huge difference in the lives of boys with Duchenne."



Thomas Rando, M.D., Ph.D.

Dr. Rando is a member of the CureDuchenne Scientific Advisory Board.



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**Saks Fifth Avenue Shopping Extravaganza, June 15-19, Costa Mesa, Calif.**

Help support CureDuchenne by shopping at Saks Fifth Avenue at South Coast Plaza in Costa Mesa, Calif., between Saturday, June 15 and Wednesday, June 19. Just tell the associate at check out that you would like to support CureDuchenne and 10% will be donated.

On June 19 from 12:30 p.m. to 2:30 p.m. there is a special Dior beauty presentation at Saks. Enjoy light bites and refreshments while you shop. Space is limited. Please e-mail [alicia@cureduchenne.org](mailto:alicia@cureduchenne.org) to R.S.V.P.

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Rockin' to CureDuchenne  
Raises More than \$11,000

Time to Climb to  
CureDuchenne

**Family of the Month**

The Cook Family

**Scientist of the Month**

Thomas Rando, M.D.,  
Ph.D.

**Upcoming Events**

Saks Fifth Avenue  
Shopping Extravaganza,  
June 15-19, Costa Mesa,  
Calif.

Knoxville Brewfest, June  
29, Knoxville, Tenn.

Getzlaf Golf Shootout,  
September 7 and 8, 2013,  
Costa Mesa and Dana  
Point, Calif.

Dealing for Duchenne  
Philadelphia, November 16,  
2013, Philadelphia

Champions to  
CureDuchenne Newport  
Beach Gala, March 29,  
2014, Newport Beach,  
Calif.

**Knoxville Brewfest, June 29, Knoxville, Tenn.**

The 3rd Annual Knoxville Brewfest will be held Saturday, June 29, 2013 in downtown Knoxville, Tennessee, from 4:00-8:00 p.m.

We'll be celebrating great beers. Beers, breweries and beer enthusiasts from the region will gather on a summer afternoon for a sampling of fresh beers of all colors, styles and tastes.

Knoxville Brewfest will be held outdoors at the historic Southern Railway Terminal at 306 Depot Avenue, Knoxville, Tennessee.

All net proceeds will benefit CureDuchenne.

Click [here](#) to learn more and buy tickets.

**Research**

New Developments with  
Prosensa and Sarepta for  
Exon Skipping in Duchenne

Reading Frame Correction  
by Targeted Genome  
Editing Restores Dystrophin  
Expression in Cells From  
Duchenne Muscular  
Dystrophy Patients

**News**

Join Ryan Getzlaf at the 3rd  
Annual Getzlaf Golf  
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Beach Gala, March 29,  
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**Getzlaf Golf Shootout, September 7 and 8, 2013,  
Costa Mesa and Dana Point, Calif.**

Save the Date for the 3rd Annual Getzlaf Golf Shootout benefiting CureDuchenne. Join Ryan Getzlaf captain of the Anaheim Ducks, NHL All-Star and Olympic Gold Medalist at the Getzlaf Golf Shootout, a two-day charity golf event, including a reception and a golf tournament, that brings together athletes, celebrities and community leaders, all teaming up in support of CureDuchenne.

On Saturday, September 7 there is a MVP reception at Sutra Lounge in Costa Mesa, Calif. On Sunday, September 8 the golf tournament will be held at the Monarch Beach Golf Links in Dana Point, Calif.

Click [here](#) to register. Each foursome will be teamed up with a professional athlete or celebrity for a unique and exciting day on the green.

For sponsorship information, please contact Karen Harley, 949-872-2552 or [karen@cureduchenne.org](mailto:karen@cureduchenne.org).

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**Dealing for Duchenne Philadelphia, November 16,  
2013, Philadelphia**

Save the Date. Dealing for Duchenne Philadelphia will be held on November 16 at the Hyatt Regency Philadelphia. This charity casino night will benefit CureDuchenne.

For sponsorship information, please contact Karen Harley, 949-872-2552 or [karen@cureduchenne.org](mailto:karen@cureduchenne.org).

Click [here](#) to learn more about Dealing for Duchenne Philadelphia.

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**Champions to CureDuchenne Newport Beach Gala,  
March 29, 2014, Newport Beach, Calif.**

Save the Date. The Champions to CureDuchenne Newport Beach Gala will be held on March 29, 2014 at the Balboa Bay Club.