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The Sahagun Family

Scientist of the Month

Jeffery Molkentin, PhD

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Gala, March 22, 2013,
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Dealing for Duchenne, May
11, 2013, Los Angeles,
Calif.

CureDuchenne Hosted FDA Webinar on Accelerated Access

CureDuchenne was honored to host a webinar on February 20, in collaboration with PPMD and MDA, to discuss accelerated approval for drugs for rare diseases. Dr. Robert Temple, director of the Office of Medical Policy of FDA's Center for Drug Evaluation and Research presented detailed information on the different options available to speed the approval of drugs. Dr. Temple also answered questions posed by the 277 participants who logged on to the webinar.

To logon to the recorded webinar please click [here](#).

A quick summary of the discussion:

- The FDA Safety and Innovation Act (aka FDASIA) amends the expedited drug development and review process by providing a fast track designation for drugs with "serious or life-threatening disease" indications and those defined as "breakthrough therapies."
- Breakthrough designation, fast-track designation, and the accelerated approval process are expedited review programs that primarily differ in the effectiveness that the product must demonstrate against a serious or life-threatening disease or condition.
- For designation as a **breakthrough therapy**, preliminary clinical evidence must indicate that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. Drugs that are given breakthrough therapy designation will benefit from (among other things) an organizational commitment from the FDA involving senior FDA managers and experienced review staff.
- A **fast-track** product must demonstrate the potential to address unmet medical needs.
- For **accelerated approval**, there must be evidence that the product has an effect on an endpoint that is reasonably likely to predict clinical benefit (i.e. an effect on a clinical endpoint other than survival or irreversible morbidity).
- Accelerated approval includes fast track products indicated for a serious or life-threatening disease or condition, and approval under this mechanism comes with a requirement that the drug be studied post-approval to confirm its clinical benefit.
- **Priority review** means review and action on the application no later than six months after FDA's receipt of the application.
- The FDA does not require a phase III trial, their focus is on having enough data available to make the best decisions.
- Patients/Parents can contact the FDA through the Office of Special Health Issues: oshi@oc.fda.hhs.gov, 301-796-8460

Thank you to Dr. Temple, Dr. Pariser, Mr. Bauer and Ms. Furia-Helms from the FDA and also Dr. Michael Kelly at CureDuchenne for facilitating this webinar.

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Vince Young To Host Champions to CureDuchenne Austin Gala



The 4th Annual Champions to CureDuchenne Austin Gala will be held on Friday, March 22 at 6:30 p.m. at the University of Texas Golf Club. This year's event is hosted by Vince Young. All proceeds will benefit CureDuchenne.

The evening will be filled with entertainment, live and silent auction and great food from Austin's best chefs. Enjoy food from the Cookwell & Company, The League Kitchen & Tavern, Max's Wine Dive, Moonshine Patio Bar & Grill, Galaxy Moviehouse & Eatery, Steiner Ranch Steakhouse, University of Texas Golf Club and Vince Young Steak House. Drink sponsors include Dulce Vida Oranagic Tequila, Tito's Handmade Vodka, Thirsty Planet Brewing Company and Duchman Family Winery.

RBC Wealth Management is the presenting sponsor of the Champions to CureDuchenne Austin Gala. Other sponsors include SoCo Mission, AT&T, Covert Cadillac, Carino's Italian, HEB, Laura & Tim Revell, Lisa and Dennis Hobbs, Pam and Rick Rhodes, Rusty and Bill Duvall, Armbruster Consulting, The Pisticks, the Munoz Family, the White Family, Deuce Brand Watches, Summit Stoneworks, Austaco, Altura Healthshare, FSG, KXAN, ASID, Horns Illustrated, Adventures Outback, Russeell Korman Fine Jewelry Diamonds & Watches, Lee Warth Designs, Bliss Blooms and Gene Arant Real Estate Team.

Put on your "cowboy chic" attire and be a champion for CureDuchenne. Tickets are \$150 per person. Table sponsorships start at \$1,000. [Click here](#) to buy tickets.



"Together we can CureDuchenne"

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New CureDuchenne Video

A new video from CureDuchenne illustrates both the urgency and hope for a cure for Duchenne. The emotional video features several Duchenne families and demonstrates the urgency of finding a cure for Duchenne by showing the progression of the disease. The video also highlights hope for Duchenne families that there are several promising drugs going through human clinical trials.

Click [here](#) to view the video.

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**Champions to CureDuchenne Newport Beach Gala
Raises Almost \$800,000**



Debra Miller, founder of CureDuchenne, Lisa and Scott Niedermayer

CureDuchenne held its annual [Champions to CureDuchenne](#) Newport Beach Gala on Saturday, February 9 at the Balboa Bay Club & Resort. The event raised almost \$800,000 toward research to find a cure for Duchenne, a progressive muscle-wasting disease that impacts 1 in 3,500 boys.



Balboa Bay Club Executive Chef Josef Lageder and Guest Chef Stan Frazier

More than 300 people attended the CureDuchenne gala. Guests were treated to a special dinner menu designed by Stan Frazier, former drummer for rock band Sugar Ray, co-owner of A Restaurant in Newport Beach and the most recent winner of "Chef Race." The menu included Geviche and Little Gem Lettuce; Black Sea Bass on Braised Rainbow Chard with Meyer Lemon Buerre Blanc, Braised Short Ribs with Truffled Mascarpone Polenta and Rosemary Balsamic Syrup; and Chocolate Banana Bread Pudding. Two of the courses on the menu were items Frazier created during Chef Race challenges.

The Champions to CureDuchenne event featured an inspirational program that began with CureDuchenne founders, Debra and Paul Miller, and their 16-year-old son Hawken, who lives with Duchenne.

"We now have visibility to a cure," said Debra Miller, founder and CEO of CureDuchenne. "Our organization doesn't need to raise the hundreds of millions of dollars it takes to develop a drug; we only need about \$6 million dollars over the next two years to initiate the research so that we can turn them over to the large drug developers."

CureDuchenne honored Scott and Lisa Niedermayer for

2013, Los Angeles, Calif.

their strong commitment to CureDuchenne over the years. Niedermayer is one of the most decorated hockey players of all-time. Niedermayer, recently named assistant coach of the Anaheim Ducks, is the only individual to win every major North American and international championship including the Memorial Cup, World Junior Championship, World Championship, World Cup, four Stanley Cups and two Olympic gold medals.



Honoree Scott Niedermayer with Debra and Paul Miller, founders of CureDuchenne

A tribute video showed highlights of Niedermayer's hockey career and how he is a champion on and off the ice. Niedermayer has supported CureDuchenne by climbing Mt. Shasta and the Grand Tetons to raise awareness and funds. Lisa Niedermayer has served on the Newport Beach Gala committee and inspired others to get involved. Lisa's cousin has FSHD, a form of muscular dystrophy, and she is also an advocate for that disease.

Dr. Michael Kelly, CureDuchenne's chief scientific officer, gave guests an overview of the state of Duchenne research and CureDuchenne's comprehensive strategy of treating the whole disease. Dr. Kelly explained that based on the current science that Duchenne could become treatable for this generation of boys. CureDuchenne is helping to accelerate this research. CureDuchenne funded two companies early on that are on the way to becoming the first to seek FDA approval for drugs to treat Duchenne.



Mary Sahagun

Mary Sahagun, a Duchenne parent, shared her story about losing her son Kenny to Duchenne at age 16. "It is too late for my son, but it is not too late for other boys with Duchenne," said Sahagun.

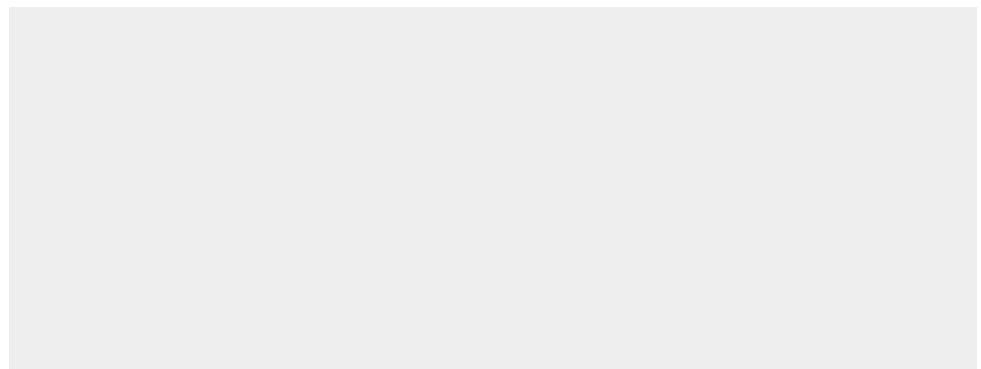
An emotional video featuring other Duchenne families illustrated the urgency of finding a cure for Duchenne by showing the progression of the disease. The video also highlighted hope for Duchenne families that there are several promising drugs going through human clinical trials.

The Champions to CureDuchenne gala ended with a pledge program and live auction. Live auction items included a La Jolla weekend escape; an ultimate sports experience; a Napa getaway with a private jet; an ultimate restaurant package; Fleetwood Mac Concert experience; a Bahamas Getaway at The Cove; and a Yellowstone Club, Big Sky

Montana vacation.

Champions to CureDuchenne event sponsors included Wells Fargo, Argyros Family Foundation, Peter & Virginia Ueberroth Family Foundation, Ducks, Honda Center, Hundred Acre Wines, PIMCO, Muscle Milk and Illumina.

Click [here](#) to see more event photos. The password is curenow.





Sage Hill moms at the Champions to CureDuchenne Newport Beach Gala



Special guests at the Champions to CureDuchenne Newport Beach Gala included the Matthews Family - Jennifer, Clay Matthews III, Leslie and Clay Matthews Jr.

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Strike...Bowling to CureDuchenne a Big Hit



Addie Thane, Rocket Ismail, Nathan Rothe and Greta Thane

Bowling with Dallas sports celebrities was a great way to raise awareness and funds for Duchenne research. The Bowling to CureDuchenne fundraiser, held on February 10 at Lewisville Lanes in Lewisville, Texas, benefitted CureDuchenne. More than 180 guests attended and the event raised nearly \$30,000.

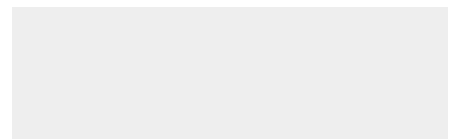
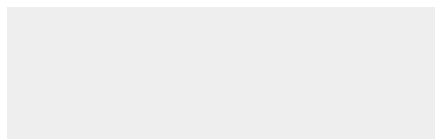


Jessica and Nathan Rothe

"The bowling event was fantastic," said Jessica Rothe, whose son Nathan, 10, lives with Duchenne. "It was a sold out event with great participation from celebrities, businesses and individuals in the community."

Dallas sports celebrities Rocket Ismail (Dallas Cowboys), Mark Brandenburg (Texas Rangers), Kevin Brooks (Dallas Cowboys), Tony Dorsett, (Dallas Cowboys), Billy Joe DuPree (Dallas Cowboys), Pete Hunter (Dallas Cowboys), Frank Lucchesi (Texas Rangers), Nate Newton (Dallas Cowboys), Ted Ovletrea (Dallas Cowboys), Carly Patterson (Olympic Champion), Drew Pearson (Dallas Cowboys), Preston Pearson (Dallas Cowboys), Jeff Russell (Texas Rangers), Don Stanhouse (Texas Rangers), Everson Walls (Dallas Cowboys) and Jen Welter (Dallas Diamonds)

bowled with community members to benefit CureDuchenne. The celebrities graciously interacted with the families, signed autographs and posed for photos with guests.



2013, Los Angeles,
Calif.



*Twins Lonnie and Larry Fikes and their brother Logan
with Tony Dorsett.*



*Nate Newton from the Dallas Cowboys and friends at
the Bowling to CureDuchenne event.*

In addition to bowling, the event included a short program and video on CureDuchenne, pizza and soft drinks, raffle prizes and a silent auction. Guests had a great time.

“Events like this help us raise awareness and funds to get closer to a cure for Duchenne,” said Debra Miller, founder and CEO of CureDuchenne. “We need to continue to fund promising research to ensure all Duchenne boys have the chance for an extended lifespan and improved quality of life.”

Thank you to Cadillac our title sponsor of Bowling to CureDuchenne. Other sponsors included: Buchanan Technologies, Dicks Sporting Goods, DKH Academy, Fort Worth Cops for Kids, JJs Lawn Care, Masergy, Mobile Media Graphics, Parity IT Specialists, Pediatric Dental World, Remax, The Tutoring Center, WinKids, Youth Sports Denton County and Youth Sports Collin County.

Thank you to Jessica and Ted Rothe for all their hard work organizing the event. Also, a big shout out to Rocket Ismail for being a terrific spokesperson and embracing CureDuchenne.



Amber and Matt Martin.

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The Western Michigan University Broncos during Ice Duchenne weekend.

[Western Michigan University](#) hosted a weekend long "Ice Duchenne" fundraiser on February 22-23 to benefit [CureDuchenne](#). The Western Michigan University Bronco hockey team, a member of the [Central Collegiate Hockey Association](#) (CCHA), sold raffle tickets to raise money and help find a cure for Duchenne. To promote the cause, [Scott Niedermayer](#), one of the most decorated hockey players of all-time, is also featured in a public service announcement that debuted with the event.

All proceeds of Ice Duchenne will benefit CureDuchenne, the leading organization funding research through venture philanthropy to find a cure for Duchenne.

"Duchenne is a devastating disease," said Rob Facca, Western Michigan assistant hockey coach, whose five-year-old son Louie lives with Duchenne. "Right now there is no cure but the research looks promising. CureDuchenne is leading the effort to fund and accelerate research to help us get closer to finding a cure."

Niedermayer, former captain of the Anaheim Ducks and Stanley Cup champion, is a strong supporter of CureDuchenne and was recently honored by the nonprofit at the Champions to CureDuchenne gala. Niedermayer, who was recently named assistant coach of the Anaheim Ducks, is the only individual to win every major North American and international championship including the Memorial Cup, World Junior Championship, World Championship, World Cup, four Stanley Cups and two Olympic gold medals. The PSA will help raise awareness about Duchenne and encourage people to donate to CureDuchenne. To view the PSA click [here](#).

"We are grateful for the hockey community's support of CureDuchenne," said Debra Miller, founder and CEO of CureDuchenne. "Based on the science, Duchenne could become treatable for this generation of boys. We just need to continue to fund promising research to ensure all Duchenne boys have the chance for an extended lifespan and improved quality of life."

Thank you, Rob, for organizing Ice Duchenne.

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Family of the Month - The Sahagun Family

Mary, Berto and Kelsy Sahagun

One of the most impactful moments of this year's Champions to CureDuchenne Newport Beach Gala was when Mary Sahagun, a Duchenne parent, spoke at the event. Sahagun shared the story of her son, Kenny, while a photo montage was shown in the background.

Kenny was a delightful boy with a lot of friends. He hit all his developmental milestones as a child. However, Kenny had enormous calves and a funny run. He was diagnosed with Duchenne at age 5.

"We lived to make memories for our son," said Sahagun.

Kenny did well in school, loved hockey, was an Anaheim Duck's fan and had a great attitude. "Kenny had a huge heart both literally and figuratively," said Sahagun. Kenny passed away five years ago, at the age of 16, from cardiac arrest.

Sahagun encouraged guests to support Duchenne research.

"It is too late for my son, but it is not too late for other boys with Duchenne," said Sahagun.

Thank you to Mary, her husband, Berto, and daughter, Kelsy, for being at the gala and sharing your story.

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Scientist of the Month - Jeffery Molquentin, PhD

Jeffery Molquentin, PhD, is a professor at the UC Department of Pediatrics at Cincinnati Children's Hospital and a Howard Hughes Medical Institute investigator. Dr. Molquentin's laboratory focuses on understanding the molecular mechanisms behind heart and skeletal muscle disease, and in particular his research studies the processes that underlie skeletal muscle degeneration and cell death. His work has helped advance the understanding of molecular events behind muscular dystrophy.

In Duchenne muscular dystrophy, understanding the relationship between the loss of dystrophin, destabilization of the skeletal muscle cell membrane and subsequent cell death has been a challenge. The Molquentin laboratory is currently investigating the hypothesis that when the membrane becomes unstable, because of the lack of dystrophin, the cell loads up with calcium and leads to skeletal muscle fiber degeneration and muscular dystrophy. Intermittent tears in the cell membrane permit an influx of calcium that functions as a primary inducer of cellular degeneration and necrosis (injury that results in the premature death of cells) of whole muscle fibers.

"We are looking at the downstream events that render cell damage in order to understand why the cells die," said Dr. Molquentin. "If we know the 'how and why' of the underlying mechanism we can try to block the parts of the cell that respond to calcium." Inhibition of these cell death pathways could have a significant impact on the progression of Duchenne muscular dystrophy.

To learn more about Dr. Molquentin's research, please see the following papers:

- Mitigation of muscular dystrophy in mice by SERCA overexpression in skeletal muscle. Click [here](#) to review.
- Calcium influx is sufficient to induce muscular dystrophy through a TRPC-dependent mechanism. Click [here](#) to review.
- Genetic and pharmacologic inhibition of mitochondrial-dependent necrosis attenuates muscular dystrophy. Click [here](#) to review.

"I have always had a strong interest in skeletal muscle research," said Dr. Molquentin. "I have a passion for the disease as I have met Duchenne families and see the plight of the patient. Duchenne is a horrible disease and we need to continue to follow promising leads and get projects into clinical trials to help slow or reverse this disease."



Jeffery Molquentin, PhD



"Together we can CureDuchenne"

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Champions to CureDuchenne, Austin Gala, March 22, 2013, Austin, Texas

Save the date for the Champions to CureDuchenne Austin Gala on Friday, March 22, 2013 at the University of Texas Golf Club. This event is hosted by Vince Young and presented by RBC Wealth Management.

Click [here](#) to register.



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Dealing for Duchenne, May 11, 2013, Los Angeles, Calif.

Save the date for the 6th annual Dealing for Duchenne event on Saturday, May 11, 2013 at the Sony Pictures Studio.

Dealing for Duchenne, created by Duchenne parents Chris and Amy Martin, is an annual event at the historic Sony Studios, including catering by Wolfgang Puck, a silent auction, an exciting live auction of one-of-a-kind items, a classic Texas hold 'Em Tournament all capped off with live entertainment by a musical guest. Funds raised help support the Center for Duchenne Muscular Dystrophy at UCLA.

More details to come.



CureDuchenne was honored to host a webinar on February 20, 2013, in collaboration with PPMD and MDA, to discuss accelerated approval for drugs for rare diseases. Dr. Robert Temple, director of the Office of Medical Policy of FDA's Center for Drug Evaluation and Research, presented detailed information on the different options available to speed the approval of drugs. Dr. Temple also answered questions posed by the participants.

Please click [here](#) for the recording of the webinar.

You'll be prompted to enter your name and email address to view the recording.