Cure Duchenne™

"Together we can CureDuchenne"

April 2013

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Earlier this month at Cold Springs Harbor, John Kraus, GSK, gave more details about the news that drisapersen, did indeed show positive results in the Phase II trial. The GSK team has brought a new level of resources and experience to Duchenne research that only a large pharmaceutical company can bring. The GSK team has been diligent in pushing trials forward around the world, having big pharma pay attention to Duchenne is a positive force for this disease. Click here for the press release.

Hans Schikan, CEO of Prosensa, and team developed their lead compound, to the point that they were able to partner with GSK to take this drug, drisapersen into several clinical trials, which includes over 300 patients. The results from the pivotal Phase III should be available late this year. We have known the Prosensa team since 2004 when we became the first non-profit organization to contribute to their drug development program. They have two other exon skipping projects in clinical trials right now and we appreciate their vision and dedication. I'd also like to thank the CureDuchenne donors who contributed significant personal funds so that CureDuchenne could develop this partnership at a time when exon skipping was a big unknown.

Chris Garabedian and his team at Sarepta have done a phenomenal job in executing a strategy that has resulted in positive results in their Phase II trial for their lead drug, etiplersen. On April 11, Sarepta announced that they have licensed exon skipping molecules for mutations that are not as common as the current patients who are participating in the trial. CureDuchenne has been working with Dr. Kevin Flanigan at Nationwide Children's Hospital to screen and investigate the possibility of using exon skipping for rare Duchenne mutations such as duplications, so we are very pleased to see this collaboration between Dr. Steve Wilton, University of Western Australia and Sarepta. We can't stop until all Duchenne boys and young men have a treatment. Dr. Wilton has been working on antisense for many, many years. Click here for the press release.

CureDuchenne was an early contributor to both Sarepta (formerly AVI) and Prosensa, but there have been numerous donors and investors that have enabled these companies to get this far. Notably, Dr. Eric Hoffman at Children's National Medical Center and Foundation to Eradicate Duchenne were protagonists in funding Sarepta several years ago.

These and many more individuals have dedicated their careers, donors have given their personal wealth and investors have poured millions into these projects. Given how little government funding is allocated to Duchenne research, the contributions of these people and companies have and will continue to be important to the Duchenne community.

As parents, we all want a treatment and cure for our kids. I think it's important to take a moment and acknowledge these individuals and companies and many, many others who have

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Data to Be Presented at the Muscular Dystrophy Association Scientific Conference

Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, announced updated data from Study 202, a Phase IIb open-label extension study of eteplirsen in patients with Duchenne muscular dystrophy (DMD). Results at 74 weeks showed a continued stabilization of walking ability in eteplirsen-treated patients evaluable on the 6-minute walk test (6MWT). As previously reported, Study 202 met its primary endpoint of increased novel dystrophin as assessed by muscle biopsy at week 48 and is now in the long-term extension phase in which patients continue to be followed for safety and clinical outcomes. Eteplirsen is Sarepta's lead exon-skipping compound in development for the treatment of patients with DMD who have a genotype amenable to skipping of exon 51.

After 74 weeks, patients in the 30 mg/kg and 50 mg/kg dose cohorts who were able to perform the 6MWT (modified Intent-to-Treat or mITT population; n=6) showed a statistically significant treatment benefit of 65.2 meters ($p \leq 0.004$) when compared to the placebo/delayed-treatment cohort (n=4). The eteplirsen-treated patients in the mITT population demonstrated less than a 5 percent decline (13.4 meters) from baseline in walking ability. After experiencing a substantial decline earlier in the study, the placebo/delayed-treatment cohort also demonstrated stabilization in walking ability from week 36 through 74, the period in which meaningful levels of dystrophin were likely produced, with a less than 10 meter decline over this timeframe.

"We are encouraged to see a continued stabilization of walking ability in patients treated with eteplirsen for nearly one and a half years," said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "These data are particularly compelling when viewed in the context of published natural history studies, which showed substantial declines on the 6-minute walk test over this timeframe in a similar ambulatory DMD population. These results continue to support the potential of eteplirsen to be a major advance in the treatment of DMD in altering the course of this progressive and irreversible disease."

Through 74 weeks, eteplirsen was well tolerated and there were no clinically significant treatment-related adverse events, serious adverse events, hospitalizations or discontinuations. As previously reported at 62 weeks, one patient had a transient elevation of urine protein on a laboratory urine dipstick test, which resolved and resulted in no clinical symptoms. The patient continued treatment without interruption and remained free of proteinuria through week 74.
Across both the eteplirsen (mITT) and placebo/delayed-treatment cohorts, there is evidence of continued stabilization on clinical laboratory tests, echocardiogram, pulmonary function tests and muscle strength.

Summary of Additional 6MWT Analyses

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Patients performed two 6MWT evaluations on consecutive days at time points coinciding with a muscle biopsy procedure at baseline and weeks 12, 24 and 48. All other evaluations were a single 6MWT. The pre-specified primary analysis included the maximum distance walked at those clinic visits where repeated tests were taken. Other analyses of the repeated 6MWT results assessed mean, minimum, and day 1 (first measure) scores. Results from these additional 6MWT analyses confirm the robust treatment effect observed in the primary analysis.

Jerry R. Mendell, M.D., Director of the Centers for Gene Therapy and Muscular Dystrophy at Nationwide Children's Hospital and principal investigator of the Phase IIb study, presented this data in an oral presentation at the Muscular Dystrophy Association Scientific Conference on Tuesday, April 23 in Washington, D.C. Dr. Mendell's presentation will be posted on the Sarepta website in the "Events & Presentations" section after the session is completed.

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Drisapersen Update Webinar with GSK on May 6

You are invited to participate in a "Drisapersen Update" webinar with GSK on May 6 at 12 EST/9 a.m. PST. The purpose of the webinar is to update the Duchenne community about Phase II clinical trial results for GSK's drisapersen drug for the treatment of Duchenne muscular dystrophy. GSK investigator John Kraus will present and answer submitted questions. CureDuchenne, in collaboration with MDA and PPMD, is pleased to co-sponsor this webinar.

If you have questions you would like to submit, please email us at heather@cureduchenne.org with "Drisapersen Update" as the subject line. We will pass along all questions to GSK prior to the webinar. Please submit your questions no later than Thursday, May 2.

To register for the webinar, click here.

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Join Us for the Star-Studded Dealing for Duchenne Gala May 11

The 6th Annual Dealing for Duchenne event, benefiting the Center for Duchenne Muscular Dystrophy at UCLA, will be held on Saturday, May 11 at 6:00 p.m. under the stars at the legendary Sony Pictures Studios in Culver City, Calif. This star-studded event is co-chaired by Duchenne parents Amy and Chris Martin and Jorge & Valerie Llauro.

Enjoy a royal flush evening hosted by actor Joel Murray. Dealing for Duchenne includes a classic Texas Hold 'Em poker tournament, red carpet, live and silent auction, dinner by Wolfgang Puck and live music. Dealing for Duchenne is presented by UCLA Health Systems and David Geffen School of Medicine.

Join the following celebrities, who are scheduled to attend, for a fun-filled evening while raising money to find a cure for Duchenne: Hank Azaria, Tim Olyphant, Anna Belknap, Oliver Hudson, Anne Heche, James Tupper, Brendan Hines, Adam Arkin, Nicole Sullivan, Catherine Bell, Sterling Brown, Josh Malina, Kellie Martin, Rich Eisen, Ming-Na Wen, Phill Lewis, Mary McCormack, Camryn Manheim, Judy Greer, Kelli Williams, Willie Garson and Kevin Weisman. It will be a full house with more than 500 people expected to attend.

"Dealing for Duchenne allows us to celebrate our sons who live with Duchenne while raising awareness and money to find a cure for this deadly disease," said Amy Martin, Duchenne parent and co-chair of Dealing for Duchenne. "We thank those who have supported us in the past and welcome new champions in our urgent race to find a cure."

The 2013 Champions for Life honorees are Marc and Nicole Weiss. The Weiss' have served on the event committee every year, engaged friends and family to join us, and helped us raise over a million dollars in the last 5 years.

The silent auction includes hundreds of fabulous items to bid on including hard to get sporting and concert tickets, set visits and meet and greets, hotel getaways, signed memorabilia, cool

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electronics and much more! In addition, the live auction includes a four night stay for 20 guests at Casa Aramara, a private resort in Punta Mita Mexico. Click here to bid on this magnificent beachfront retreat on Charity Buzz. Joe Francis, Girls Gone Wild creator, generously donated this private home. Or you can bid on a trip for two to Florida to golf in the annual Caddyshack Tournament and hang with the Murray brothers for four days of golfing and partying. This item was generously donated by Joel Murray.

Proceeds of Dealing for Duchenne benefit the Center for Duchenne Muscular Dystrophy at UCLA which supports Duchenne related muscular dystrophy translational research and clinical care at UCLA. Key missions include building and supporting a state of the art multidisciplinary care program for children and adults with muscular dystrophy, building and supporting a clinical trials infrastructure to accelerate clinical investigation into new treatments, and the support of translational biomedical research relevant to Duchenne muscular dystrophy.

We would like to thank our generous sponsors: Stella Artois, Grey Goose, Bacardi, Vorzimer Masserman, Ramland Construction, O'Melveny & Myers LLP, Gibson Dunn, Freedman + Taitelman LLP, GGW Brands, Credit.com and Illumina.

We hope you'll join us on May 11 for this spectacular event. Register now to attend Dealing for Duchene at Dealing4Duchenne.org.

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Create a Climb to CureDuchenne Team

Join us for the fifth annual Climb to CureDuchenne: Pick Your Peak 2013. 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 research projects that are so close to treatments to save our sons' lives. With your help we can make it to the peak.

We invite you to form a team, set up a fundraising page, secure donations and Climb to CureDuchenne. Pick a date that works best for you. There are no geographical or athletic ability limitations. You pick where you want to walk, hike or climb. You can also join a team or donate without climbing.

Some of this year's planned events include:

- Mt. Aconcagua highest mountain in the Americas located in the Andes mountain range in Argentina (22,841 feet)
    - 9-year-old Tyler Armstrong to attempt to break record for youngest person to climb Mt. Aconcagua
- Mt. Mitchell the highest peak of the Appalachian Mountains located in North Carolina and the highest peak in the Eastern United States (6,684 feet)
    - Four Duchenne dads from the Southeast to climb

We would like to add your local climb to this list! It doesn't have to be a strenuous climb it can be a walk or hike. Plan an event that works for you and your area.

We hope you'll set your own personal climbing challenge, big or small, to help us conquer Duchenne. Organize a team and encourage your friends, family and colleagues to join and support 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.

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To get motivated, watch the Pick Your Peak video from last year.

For more information and resources to promote Climb to CureDuchenne in your community, contact Karen Harley at 949-872-2552 or karen@cureduchenne.org.

CureDuchenne Cajun-Parrot Run

Jim Ruetz is ready to hit the open road to help raise funds to benefit CureDuchenne.

Jim Ruetz is going on a 4,000+ mile, 12-day motorcycle trip across 12 states in the Cajun-Parrot Run to benefit CureDuchenne. He starts his journey on June 5 from Milwaukee, Wis. and is tentatively scheduled to arrive in Key West, Fla. (the Parrot part of the trip) on June 18. In route, he'll stop in New Orleans (the Cajun part of the trip).

"I'm definitely going to have to find an old football helmet without a face mask to do my best Jack Nicholson 'Easy Rider' pose," said Ruetz.

This is not the first time Ruetz has traveled on the open road to benefit CureDuchenne. In 2011, Ruetz took a "bucket list" ride took to Alaska. He covered more than 7,000 miles, 10 states, provinces, and territories in the U.S. and Canada. He helped raise $10,000 for CureDuchenne.

Ruetz hopes to double the funds raised for Duchenne in 2011 to $20,000 for this year's Cajun-Parrot Run. Please support the Cajun-Parrot run by donating to any of the following levels: Pirate level ($100), Rage'n Cajun ($200) and Parrot Head ($300). Everyone who contributes at the pirate level or above will receive a "Cajun-Parrot Run" t-shirt and will automatically be entered in a drawing for the Clay Matthews signed Packers' Jersey. Click here to donate.

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“I hope you’ll join me again this year as we raise funds and awareness for CureDuchenne,” said Ruetz. “I’ll be blogging during the trip and will definitely continue my search for great Irish pubs in the U.S. where the perfect pint of Guinness can be found.”

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Duchenne family members joined the CureDuchenne Scientific Advisory Board on March 29 for dinner following the Scientific Summit. The group enjoyed a cocktail reception and dinner at Bistango in Irvine, Calif., while mixing and mingling with scientists and other Duchenne parents.

The Scientific Advisory Board was in town for a Scientific Summit meeting to review and discuss CureDuchenne's research and development strategy.

Thank you to the following scientists for giving up their personal time to attend the Scientific Summit and meet with Duchenne family members: Barry Byrne, Kevin Campbell, Jeff Chamberlain, Eric Hoffman, Ed Kaye, Douglas Macdonald, M. Carrie Miceli, Kannebovina Nagaraju and Stanley Nelson.

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Family of the Month - Llauro Family

Jorge and Valerie Llauro and their children Elena and Alexander.

Empower yourself with knowledge, be involved and live in hope. Those are words of wisdom from Valerie Pappas Llauro of Woodland Hills, Calif., to other Duchenne parents.

"Attitude and action are key," said Llauro, whose son Alexander was diagnosed with Duchenne at age 5 ½. "Our faith and support network gives us the strength we need every day. This has been the cards we have been dealt and it can either destroy you or build you up. We choose to take control and get involved. We live each day and focus on the present."

Llauro first noticed Alexander was lagging behind his peers as a toddler. Alexander had a hard time walking long distances, he had trouble pedaling a bike and he wasn't a climber like other children at the park. His pediatrician said not to worry, he'll catch up.

"We really started to notice a difference when his younger sister was running circles around him," said Llauro.

Llauro took Alexander to the pediatrician again thinking that he needed a referral to an orthopedist since he was walking on his toes. The doctor put Alexander and his sister, Elena, on the floor together. Alexander had a hard time getting up and his calves were large.

"It was like going from A to Z all at once," said Llauro when Alexander was diagnosed with Duchenne. "We went in to the office thinking he might need support in his shoes and instead he needed a blood test and a referral to a neurologist. We were shocked and devastated."

Right now Alexander is a typical 7-year-old boy. He loves playing basketball and kickball with friends in front of his house. He is a sweet-natured, fun loving boy. Alexander is a happy child who smiles all the time. He wears night splints, his parents do extensive stretching each night and he is swimming

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with a therapist each week. He knows that all this helps his muscles. Alexander runs different but other than that he is not much different than other children his age.

"I cannot fathom his abilities being comprised. We pray Alexander grows up into a healthy, well-adjusted adult who lives a full life," said Llauro. "We are hopeful that the progression of the disease is halted so it can at least be managed as a chronic disease."

Alexander has duplication on exon 2. Llauro has been active fundraising for CureDuchenne's support of Dr. Kevin Flanigan research project that focus on exon duplication mutations. Dr. Flanigan and his team at Nationwide Children's Hospital have developed a new mouse model for Duchene that carries a duplication of exon 2. Llauro's family and friends have raised more than $10,000 to support this project. Alexander is treated at the Center for Duchenne Muscular Dystrophy at UCLA and Llauro also is involved supporting CDMD.

Llauro and her husband, Jorge, are co-chairing this year's Dealing for Duchenne event in Los Angeles. They are pulling together their film marketing and brand marketing background, respectively, to secure sponsors and auction items. Llauro also tapped into a friend that works at CNN to produce a video for the Center for Duchenne Muscular Dystrophy at UCLA. The Emmy award-winning cameraman and CNN editor are providing their services pro bono. The video will premiere at the event.

"Since Duchenne is a rare disease the funding it not where it needs to be," said Llauro. "We are using our resources and networks to get the word out. Awareness equals interest and interest leads to funding. Funding leads to action and better treatments and ultimately the cure - and that is what we need to help all our Duchenne boys."

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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.

Click here to register.

June 18 (tentative finish date) from Milwaukee, Wis. to Key West, Fla.

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

Ryan and Jack Fight Back 5K Run and 1 Mile Fun Run, May 11, 2013, Jenkintown, Penn.

Join the Ryan and Jack Fight Back 5K Run and 1 Mile Fun Walk benefitting CureDuchenne on May 11, 2013 to raise awareness and funds to find a cure for Duchenne. The event will be held at Alverthorpe Park located in Jenkintown, Penn.

Click here to register. The registration cost is $25 online. It is $30 in person the day of the race.

You must register by April 20th to receive a t-shirt. There will be a $50 prize for the first place female and first place male runner.

Registration and race bib pick up will start at 9:15 and race will start at 10:30 promptly. Please contact Kristen at kristen@ryanandjackfightback.com or 215-850-3067 for more information.

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Cure Duchenne™

"Together we can CureDuchenne"

April 2013

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CureDuchenne Cajun-Parrot Run, June 5 through

Rockin' to CureDuchenne, June 2, 2013, Irvine, Calif.

Come join us for music and fun as we rock to some local bands to raise awareness and fund research to find a cure for Duchenne. All proceeds to benefit CureDuchenne.

Bands include Breach the Summit, Johnny and the Gentleman and The Hager Boyz.

Tickets cost $20 and t-shirts are $25.

Click here for more information and to register.

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CureDuchenne Cajun-Parrot Run, June 5 through June 18 (tentative finish date) from Milwaukee, Wis. to Key West, Fla.

Jim Ruetz is riding his motorcycle from Milwaukee, Wis. to Key West, Fla. in the Cajun-Parrot Run to benefit CureDuchenne. This 4,000+ mile, 12-day motorcycle trip will cover 12 states.

Click here for more information and to donate.

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R&D

UPDATED: GlaxoSmithKline measures 36-meter leap over placebo in Duchenne walking test

by *John Carroll* | Apr 11, 2013 2:39pm

GlaxoSmithKline investigator John Kraus reviewed the long-awaited mid-stage results for its Duchenne muscular dystrophy drug drisapersen this afternoon, revealing that boys taking the experimental therapy benefited with a significant improvement in walking distance compared to a placebo arm. And they did it without any serious signs of the toxicity that has raised questions about how well this drug may compete in the future with a rival therapy from Sarepta ($SRPT).

Looking at the history of the effect of this disease, Kraus tells *FierceBiotech*, "there is a predictable decline (in patients); the rate accelerates. We would have been happy with results that showed stability in walking distance and further encouraged by any evidence of gain. So when you talk about magnitude of change, 30 meters change from placebo, that is considered clinically meaningful." In addition, he adds, there were no serious adverse events in the study that forced any of the boys to stop taking the medication, though investigators did track mild signs of proteinuria in volunteers.

GlaxoSmithKline ($GSK) randomized 53 subjects for their Phase II study of drisapersen. Of those, 18 were in the placebo arm. Kraus, the project physician leader, noted that in this group of younger boys, some could normally be expected to register an improvement in walking distance without treatment.

For boys in the continuous treatment group after 24 weeks, there was a mean improvement over baseline of 31.5 meters in the 6-minute walking test, says Kraus, who reviewed the data at a scientific meeting at Cold Spring Harbor Labs. In the placebo arm there was an average decline of 3.6 meters at 24 weeks. After 48 weeks of therapy there was an average improved walking distance of 11.2 meters compared to a 24.7 meter mean decline in the placebo arm--a swing of about 36 meters.

In the intermittent treatment group, after 24 weeks the drug arm declined 0.1 meters, reflecting little sign of change. After 48 weeks the intermittent drug arm reflected an improvement in walking distance of 2.4 meters.

GSK investigators also rated boys using the North Star Ambulatory Assessment scale, a 17-point system on which a score of 34 is normal.

After 48 weeks of continuous treatment, both treatment groups were approximately two points above the placebo arm (the actual mean values were 2.50 continuous, 2.29 intermittent). As a recent natural history study indicates that boys with Duchenne are expected to see a 1.8-point decline over a year, Kraus says they were encouraged to see a 1.6-1.7 point difference from placebo after week 24, though he noted that the study wasn't powered to assess a statistically significant improvement on this score.

Among the adverse events recorded, investigators noted an injection site reaction, which is not uncommon in drug trials, as well as mild signs of proteinuria, an excess level of protein in the urine. Close observers of drisapersen have paid particularly close attention to the proteinuria cases after GlaxoSmithKline discussed cases in Phase III that required hospitalization.

Kraus notes that of the 12 subjects who have taken drisapersen for more than three years, none have had to withdraw from the study. Now a total of about 300 boys have been randomized in all trials, where the potential safety and efficacy of the compound can be explored for a longer period with significantly increased numbers. It's only after the study was expanded that investigators could see more serious adverse events, "which was not necessarily unexpected," says Kraus.

About the proteinuria in the current study, Kraus noted that "it was not progressive, it didn't get worse over time. In our other trials, there have been two subjects with excessive proteinuria but this resolved after stopping treatment."

For the Wall Street crowd, these Phase II numbers will quickly be compared to the data Sarepta ($SRPT) has accumulated on eteplirsen, another exon-skipping therapy like drisapersen--which was initially developed by Prosensa. Sarepta's promising Phase II study was significantly smaller, involving 12 boys. At 48 weeks the once-weekly 50 mg treatment arm demonstrated a 21-meter improvement over baseline, which was higher than GSK's results. But the placebo/delayed-treatment group declined 68.4 meters; much, much worse than the placebo arm in GSK's study--offering a much bigger combined advantage of 89.4 meters. Sarepta has also kept track of the boy's ongoing results after 48 weeks, tracking a sustained effect.

A big question here is how regulators are likely to review both sets of data. There's been a frenzy of speculation that Sarepta will go straight to an NDA if they receive some encouragement from the agency, which might allow them to do the Phase III after marketing approval. But if regulators are left dubious about the size of the trial or the potential for detecting adverse events in Phase III, and Sarepta is forced to push ahead with Phase III ahead of an application, a number of analysts expect the stock price to plummet. A green light on the NDA now, though, could push its share price even higher.

A decision from Sarepta should come shortly. GlaxoSmithKline, meanwhile, says it will post Phase III data later this year.

CORRECTION: The original article noted a 27.4 meter decline for the placebo group at 48 weeks. On review, GSK corrected that number to 24.7 meters.

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Save the Date for the 3rd Annual Getzlaf Golf Shootout benefiting CureDuchenne. Join Ryan Getzalf 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.

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