Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

Sarepta Therapeutics (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, announced on November 26 a collaboration for the development of an additional exon-skipping drug targeting exon 53, its fourth drug in development, in support of Sarepta's broad-based program for the treatment of Duchenne muscular dystrophy (DMD). Sarepta's collaboration is with University College London's (UCL) scientist, Professor Francesco Muntoni, MD, the Dubowitz Neuromuscular Centre, the Institute of Child Health and other scientists from the EU and US. The EU Health Innovation-1 2012 Collaborative research grant will support certain IND-enabling activities and clinical proof of concept studies for an exon 53-skipping therapeutic. Sarepta recently announced positive results from its extension study of its Phase IIb trial of eteplirsen, its exon 51-skipping therapeutic candidate for the treatment of DMD. Sarepta is also developing other PMO-based exon-skipping drug candidates for exons 45 and 50.

"The recent compelling clinical data on eteplirsen targeting exon 51, which started with our work on the Phase I study in the UK, provides a strong foundation for using Sarepta's technology against exon 53," said Francesco Muntoni, professor of pediatric neurology and head of the Dubowitz Neuromuscular Centre at the UCL Institute of Child Health, London. "We are pleased to be working with Sarepta to bring this exciting exon-skipping therapeutic approach to Europe and the broader DMD population."

This program will be based on Sarepta's advance proprietary RNA-based platform, Phosphorodiamidate Morpholino Oligomers (PMOs), which is a novel backbone chemistry that provides enhanced target tissue specificity, increased potency to allow for more efficient dosing, and greater uptake within a cell to further increase protein production. Targeting exon 53 with this technology will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping (deletion of exons 42-52, 45-52, 47-52, 48-52, 49-52, 50-52, or 52) by restoring the cellular machinery's ability to produce a functional dystrophin protein.

"The initiation of this program, along with our other collaborations for exons 45 and 50, continues to advance Sarepta's strategy in pursuing exon-skipping therapeutics for all of the DMD patients who may benefit from this drug technology," said Chris Garabedian, President and CEO of Sarepta Therapeutics. "Our goal of demonstrating that the success of eterplirsen can be reproduced across other exon-skipping targets is a critical step in being able to treat more boys and young men affected with this devastating disease."

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

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

Sarepta Therapeutics (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, announced on November 26 a collaboration for the development of an additional exon-skipping drug targeting exon 53, its fourth drug in development, in support of Sarepta's broad-based program for the treatment of Duchenne muscular dystrophy (DMD). Sarepta's collaboration is with University College London's (UCL) scientist, Professor Francesco Muntoni, MD, the Dubowitz Neuromuscular Centre, the Institute of Child Health and other scientists from the EU and US. The EU Health Innovation-1 2012 Collaborative research grant will support certain IND-enabling activities and clinical proof of concept studies for an exon 53-skipping therapeutic. Sarepta recently announced positive results from its extension study of its Phase IIb trial of eteplirsen, its exon 51-skipping therapeutic candidate for the treatment of DMD. Sarepta is also developing other PMO-based exon-skipping drug candidates for exons 45 and 50.

"The recent compelling clinical data on eteplirsen targeting exon 51, which started with our work on the Phase I study in the UK, provides a strong foundation for using Sarepta's technology against exon 53," said Francesco Muntoni, professor of pediatric neurology and head of the Dubowitz Neuromuscular Centre at the UCL Institute of Child Health, London. "We are pleased to be working with Sarepta to bring this exciting exon-skipping therapeutic approach to Europe and the broader DMD population."

This program will be based on Sarepta's advance proprietary RNA-based platform, Phosphorodiamidate Morpholino Oligomers (PMOs), which is a novel backbone chemistry that provides enhanced target tissue specificity, increased potency to allow for more efficient dosing, and greater uptake within a cell to further increase protein production. Targeting exon 53 with this technology will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping (deletion of exons 42-52, 45-52, 47-52, 48-52, 49-52, 50-52, or 52) by restoring the cellular machinery's ability to produce a functional dystrophin protein.

"The initiation of this program, along with our other collaborations for exons 45 and 50, continues to advance Sarepta's strategy in pursuing exon-skipping therapeutics for all of the DMD patients who may benefit from this drug technology," said Chris Garabedian, President and CEO of Sarepta Therapeutics. "Our goal of demonstrating that the success of eterplirsen can be reproduced across other exon-skipping targets is a critical step in being able to treat more boys and young men affected with this devastating disease."

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

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

CureDuchenne, Clinical Trials and the FDA

Following the long awaited news of Sarepta's phase IIB results for eteplirsen and continued Phase III progress of GSK/Prosensa's drisapersen, as well as the recent developments with PTC Therapeutics and Summit PLC among others, CureDuchenne took the initiative to reach out for patients and advocacy groups alike to begin a dialogue with the FDA and the broader community on issues related to future clinical trial design, expedited approval strategies, clinical endpoints and strategies for multiple exon skipping. It is hoped that these efforts will benefit those product opportunities currently in clinical development as well as future development programs.

It is encouraging to see so many product opportunities making their way into human clinical trials, with the most advanced getting closer to regulatory approval. However it is important to remember that the overall process is incredibly risky and while we remain very hopeful that the first drug will be approved soon, the drug development and approval process still suffers from high attrition rates and has the ability to disappoint at any moment. It's important to also remember that a lot of requirements must be met for an approved product to reach those who need it and not all of the hurdles are regulatory in nature. A product can be approved as safe and efficacious but could still present challenges to the patient and caregiver for example: issues such as production on a commercial scale, product availability, cost to patient and potential reimbursement issues can be stumbling blocks to patients getting access to the drug.

Click here for specific product development efforts.

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

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

Message from the Founder

As we draw near to the close of another year, we want to thank all of you who have supported CureDuchenne. It has been a tremendous year.

Research is at a tipping point. We can confidently say, for the first time, that we expect to see drugs and therapies approved in the coming years that will improve the quality of life and extend the lifespan of this generation of Duchenne boys.

CureDuchenne has funded four research projects in 2012: the MDX Duplication Mouse Model with duplicated exons; Spironolactone, a study with currently approved drugs that may help treat critical cardiac issues; Summit Corporation plc. which is testing the reformulation of the utrophin upregulator SMT C110; and Halo Therapeutics which is developing an anti-fibrotic drug.

CureDuchenne has also funded the Center for Duchenne Muscular Dystrophy at UCLA for the past five years. The CDMD at UCLA supports muscular dystrophy related translational research and clinical care at UCLA.

In 2012, CureDuchenne hosted three major, national events that raised a total of more than $1 million and had more than 1,400 attendees who gave generously to support our mission. In addition, countless parents and Duchenne advocates created local events and fundraisers across the country to help support CureDuchenne and its efforts to find a cure.

From national spokesperson Super Bowl Champion Clay Matthews of the Green Bay Packers to NHL All-Star Ryan Getzlaf, captain of the Anaheim Ducks, celebrities helped us increase awareness of Duchennne. CureDuchenne was literally shouted from mountain tops when 8-year-old Tyler Armstrong climbed Mt. Kilimanjaro and received national and local media coverage.

Very few health-related nonprofits have been as successful and as focused as CureDuchenne in helping bring potential life changing treatments into human clinical trials. To keep this momentum, we need to continue to raise awareness and funds to find a cure for Duchenne.

If you have donated to our year end campaign, we thank you. You are truly helping make medical history in the fight to find a cure for Duchenne. Thank you for giving hope to the 300,000 boys worldwide with Duchenne.

If you haven't donated yet we could still use your support. Your generosity can save a child from suffering pain, immobility and a short lifespan. Now is the time to invest in this life changing and lifesaving research. This will be the last generation of Duchenne boys we lose or the first generation of Duchenne boys who are saved.

Donate NOW

Click here to donate now!

Best wishes to you and your family for a joyous holiday season and a healthy, happy 2013.

With gratitude,

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

Debra Miller

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

Year-End Philanthropy Tax Implications

As we near the end of 2012, it is both the season for giving and the time of year when tax liabilities become a priority. As you calculate your earnings and estimated taxes, now is the perfect time to consider a charitable contribution to benefit the community while having a favorable effect on your taxes come April 15.

With the uncertain future of the tax code, making a philanthropic gift in 2012, when tax deductions and tax rates are known, can have advantages. According to some tax experts, the changing tax code could result in a huge reduction in the tax deduction value (an estimated 80 percent drop according to some tax experts) of charitable contributions for the wealthiest individuals.

Ensuring your contributions are having an impact is critical when choosing a nonprofit to support. CureDuchenne is a worthy recipient of a year-end gift having demonstrated tremendous success in funding innovative and promising research for a cure for a deadly disease. CureDuchenne stands apart from other medical research nonprofits by having funded seven human clinical trials and is on the brink of a lifesaving treatment for Duchenne Muscular Dystrophy.

Check with your accountant or financial advisor to determine the best course of action for your financial benefit. CureDuchenne's tax ID number is 20-0299958 and information on making a stock, cash or other asset contribution is available by calling 949-872-2552.

Donate NOW

Click here to donate now!

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

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala.

The Niedermayer family.

CureDuchenne will honor Scott and Lisa Niedermayer at the Champions to CureDuchenne Newport Beach Gala on Saturday, February 9 for their generosity and commitment over the years to CureDuchenne.

Scott, former captain of the Anaheim Ducks and NHL champion, is the only player to win every major North American and international championship in his career. A true champion both on and off the ice, Scott holds two Olympic gold medals, four Stanley Cups and the World Cup. He has supported CureDuchenne by climbing Mt. Shasta and the Grant Tetons to raise awareness and funds and last year was a celebrity player in the Getzlaf Golf Shootout. Lisa has served on the Newport Beach Gala committee and inspired others to get involved. Lisa's cousin has Fascioscapulohumeral Muscular Dystrophy (FSHD), a form of muscular dystrophy, and she is also an advocate for that disease. The Niedermayers live in Newport Beach with their four sons.

The 2013 Champions to CureDuchenne Newport Beach Gala will be held at the Balboa Bay Club & Resort in Newport Beach, Calif. This prestigious annual charity event benefits CureDuchenne.

Community, business and sports elite will come together for a fun and memorable evening that includes a cocktail reception and a special menu developed by Executive Chef Josef Lageder of the Balboa Bay Club and our guest chef Stan Frazier drummer for the pop rock band Sugar Ray, co-owner of A Restaurant in Newport Beach and most recent winner of "Chef Race" on BBC America. The program with include the honoree presentation and a live auction. The program will be followed by live music.

Tickets are now available. Please click here to purchase a table. If you are interested in

sponsoring the Newport Beach Gala, please call 949-872-2552.

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

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

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

We are on the run of our lives to find a cure for Duchenne muscular dystrophy. The CureDuchenne Crusaders team is gearing up for the Tinker Bell Half Marathon on January 20, 2013 at the Disneyland Resort. Thirty runners will run the half marathon and five people will run the Never Land Family Fun Run 5K on January 19 to help raise awareness and funds to find a cure for Duchenne.

All our runner spots are sold out but you can help by supporting a participant. The goal is to raise $15,000. 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.

Click here to make a donation and a support member of the CureDuchenne Crusaders team.

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

Cure Duchenne™

"Together we can Cure Duchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast,

Family of the Month - The Crawford Family

The Crawford Family

When your son is diagnosed with Duchenne it changes your entire outlook on life.

"It hits you hard to think that your child may not live past their twenties," said Todd Crawford. This was Crawford's reaction when his son Mackenzie was diagnosed with Duchenne in 2004 at the age of 4½.

"As a father and a husband, it is devastating to be dealt a problem that you can't fix," said Crawford. "You learn to appreciate everything you have a lot more."

Mackenzie Crawford

Mackenzie, now 12, is very creative, artsy, and has a good sense of humor. He can turn anything into a craft project. The sixth grader enjoys video games and playing with a select group of friends in his neighborhood near Chattanooga, Tenn.

"Now the outlook is more hopeful than when Mackenzie was diagnosed," said Paula Crawford, Mackenzie's mom. "We are fortunate that Mackenzie was able to participate in a clinical trial."

Mackenzie has participated in a Glaxo Smith Kline Phase III exon skipping clinical trial. CureDuchenne was an early supporter of Prosensa, the biotech company that developed this drug before licensing it to GSK. Mackenzie finished the 48 week trial in November. The first 24 weeks Mackenzie received weekly injections. He and his dad travelled to Cincinnati once a week for eight weeks for treatments and then monthly visits after that. Mackenzie also had three

Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

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

muscle biopsies during the trial.

“It was a please to spend time getting to know the doctors, nurses, and staff at Cincinnati Children’s Hospital. It is more important to have doctors and nurses that really care about your son and know about Duchenne than the convenience of your local care facility,” said Todd. “It was also nice to meet other families in the trial.”

The Crawford’s hope to see success in the open label phase of the trial which is scheduled to begin in mid-January.

“We are amazed by what CureDuchenne is doing to fund Duchenne research,” said Todd. “CureDuchenne has had good success in helping to bring research into clinical trials. They inspire us to do more to help.”

This inspiration has led to action. In June, Todd participated in Climb to CureDuchenne to help raise awareness and funds to find a cure for Duchenne. Todd rallied three other dads, who work together in the Chattanooga information technology industry and have sons living with Duchenne, to climb Clingman’s Dome, the highest peak in the Smoky Mountains.

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

Scientist of the Month

Professor Steve Wilton

The following is a Q&A with Professor Steve Wilton from the University of Western Australia, Director of Translational Research, and the Australian Neuro-muscular Research Institute and Centre for Neuromuscular and Neurological Disorders. Professor Wilton is also the President of the Australian Gene Therapy Society.

Q: You are one of the pioneers in the use of exon skipping to treat Duchenne. How did you get involved in Duchenne research?

A: I moved to Western Australia to work for a small Biotech company in 1987 and worked there for four years. I left that company to undertake more applied research and joined the Australian Neuromuscular Research Institute (ANRI) in 1991, where I developed diagnostic tests for a series of neuromuscular diseases…. Duchenne muscular dystrophy (DMD) was one of the first diseases I studied.

I became very involved in mutation identification for the local DMD families. During this mutation screening, I found natural exon skipping was occurring in some DMD muscle tissue, albeit at very low levels. I started to wonder if it would be possible to specifically increase this natural exon skipping, and one thing has led to another.

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

A: There are clinical trials ongoing that show exon skipping has great potential. The numbers of treated boys, at least with the morpholino compounds, is very limited but the results are positive and indicate the disease progression has been halted based on the 6 minute walk data released to date.

While this is extremely positive, I am very conscious that this will only address a small proportion of the DMD population. One of the things my lab is doing to ensure we have compounds developed to treat as many different DMD mutations are possible. There are many challenges ahead, including production, regulatory approval, dosing and so on. If we can show there are conclusive benefits of exon 51 skipping, and the therapy is safe, then the aim is to gain regulatory approval of these compounds as a class so more compounds can be brought to the clinic.

Q: You developed the compound that was licensed to Sarepta for development. What are your thoughts of the most recent research results?

A: I'm very excited and relieved at the same time that there appears to be some benefit. The numbers of treated boys is small, but the data looks convincing. The 6 minute walk test data is compelling and the molecular testing (showing dystrophin expression and localization) is showing we are on the right track. There is obviously a lot of room for further improvement.

Q: This drug skips exon 51 and will only help about 15% of Duchenne boys. What is the best way to accelerate drugs for the other 85%?

A: It is going to be tough for many families, having to watch some boys being treated and waiting for their turn to come. Every dystrophin exon (except for the first and last) can be skipped, and while we were the first to publish a panel of oligos showing this, there is still

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

room to improve the potency of many exon skipping compounds. If class-specific approval can be obtained, then I would hope that other exon skipping compounds would be readily approved. Before this can happen though, it is essential to show that exon skipping is safe and beneficial. If we try and rush too many different compounds through, and a mistake is made, it could slow the entire field. The first couple of different compounds have to be trialed very thoroughly and carefully. While it seems slow, it is the only way we can bring other compounds to the clinic.

Q: Tell us about the Duchenne research you are currently working on.
A: Lots of different research being undertaken: We are looking at many different DMD mutations and developing exon skipping strategies. So far, every mutation can be by-passed/skipped, but in some cases we are not sure if the induced dystrophin will be functional. If a mutation occurs in an exon encoding an important dystrophin functional domain (i.e., beta-dystroglycan binding), we know we can by-pass the mutation but will the induced dystrophin retain any function. To address this question, we are making Becker-MD like mouse models with in-frame mutations in different parts of the dystrophin gene.

Q: What are the goals and objectives of your research?
A: Simple, to make exon skipping available to as many different mutations as possible, hence we are taking great care to design the most efficient oligomers (genetic band aids) that we can (at least based on in vitro testing).
We are also looking at extending the splice switching therapy to other diseases, and there are many that could respond. If we can show proof-of-concept for other diseases then there will be even greater demand on large scale oligomer production and hopefully more industry interest in scale up.

Q: What will it look like if it is successful?
A: Hopefully we can arrest progression of DMD, but how well this may work will depend on where the gene mutation is, how much of the gene is missing etc. It must be remembered that exon skipping cannot be a cure. We hope to change the progression of the disease from DMD to Becker MD, with the aim of the mildest BMD possible, but this milder condition still shows symptoms.
Commencement of treatment is also important. We do not know if older non-ambulant DMD individuals would benefit from the treatment, and this is something that must, and will be, looked at.

Q: What are you excited about in terms of Duchenne research?
A: There is a lot going on at the moment and I am hoping things will get moving even faster, and when sufficient momentum is achieved it will become self-maintaining. It is no longer the case of one individual driving the research/trials, but rather large multi-disciplinary teams with industry (Sarepta or GSK) involvement.

Q: What are the next steps?
A: Patience and trusting the field is moving as fast as possible. Keep abreast of developments by checking reputable websites (we are currently discussing that aspect at an ENMC workshop). It is recognized that parents/patients should be kept informed of developments, or if there has been slow/no progress, then that should be stated and why. Individual reports or stories on the electronic media can be distorted or misinterpreted and this can be a cause of angst and must be corrected.

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

Sage Hill Student Alexander Sun ('14) will play a violin concert on January 12 at 2 p.m. at the Studio at Sage Hill School in Newport Coast, Calif., to benefit CureDuchenne. The violin concert will include a finale with the Sage Hill String Ensemble. A reception will be held following the concert at the Studio lobby. All proceeds of the concert go to CureDuchenne. Please R.S.V.P. by e-mailing info@cureduchenne.org or calling 949-872-2552.

Click here for more information.

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

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

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 support a participant and make a donation, click here. For more information call CureDuchenne at 949-872-2552.

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

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

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. The gala will feature a celebrity chef, live music, dinner and drinks and a live auction.

- **Individual Tickets -** $350
- **Table Sponsor (Table of 8) -** $2,500
- **VIP Table (Table of 8) -** $5,000

Click here to register.

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

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

Join Dallas Cowboys and Texas Rangers players for a Bowling to CureDuchenne event on Sunday, February 10 from 2 p.m. to 6 p.m. at Lewisville Lanes AMF in Lewisville, Texas. There will be a celebrity bowling on each lane. There will be games, food, raffles and a silent auction. All proceeds from the event will benefit CureDuchenne.

- **Bowler (child)** - $50
- **Bowler (adult)** - $100
- **Sponsor a team** - $400
- **Sponsor a lane** - $1,000

Click here to register.

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

Cure Duchenne™

"Together we can CureDuchenne"

December 2012

Research

Sarepta Therapeutics Enters Into Collaboration for the Development of Additional Exon-Skipping Product for Duchenne Muscular Dystrophy

CureDuchenne, Clinical Trials and the FDA

Message from the Founder

A Year-End Message from Debra Miller

News

Year-End Philanthropy Tax Implications

CureDuchenne to Honor Scott & Lisa Niedermayer at Champions to CureDuchenne Newport Beach Gala

CureDuchenne Crusaders Gearing Up for the Tinker Bell Half Marathon

Family of the Month

The Crawford Family

Scientist of the Month

Professor Steve Wilton

Upcoming Events

Alexander Sun and Friends for CureDuchenne Violin Concert, January 12, 2013, Newport Coast, Calif.

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

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

Bowling to CureDuchenne, February 10, 2013, Lewisville, Texas

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. More details to come.

Click here for more information.

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