Sarepta Therapeutics Announces Plans to Submit New Drug Application to FDA for Eteplirsen for the Treatment of Duchenne Muscular Dystrophy by Year End 2014

Sarepta Therapeutics, Inc., a developer of innovative RNA-based therapeutics, announced on April 21 it plans to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) by the end of 2014 for the approval of eteplirsen for the treatment of Duchenne muscular dystrophy (DMD). Eteplirsen is Sarepta’s lead exon-skipping drug candidate in development for the treatment of patients with DMD who have a genotype amenable to skipping of exon 51.

The plan to submit an NDA for eteplirsen by the end of 2014 is based on a guidance letter from the Agency that proposed a strategy regarding the submission of an NDA for eteplirsen under a potential Accelerated Approval pathway and served as the final meeting minutes for four meetings that took place between November, 2013 and March, 2014. The Agency stated that “with additional data to support the efficacy and safety of eteplirsen for the treatment of DMD, an NDA should be fileable,” and outlined examples of additional data and analysis that, if positive, will be important to enhance the acceptability of an NDA filing by addressing areas of ongoing concern in the existing dataset. Additionally, the Agency provided clear guidance on an open-label, historically controlled confirmatory study of eteplirsen, as well as initial guidance on a placebo-controlled study of one or more follow-on DMD drug candidates, which, like the open-label study, could also be considered an acceptable confirmatory study to verify the clinical benefit of eteplirsen in the event of an accelerated approval.

“As we announce our plan to submit an eteplirsen NDA by the end of 2014, we are very pleased with the detailed guidance that the FDA has provided us on a potential eteplirsen approval pathway and their support of a historically controlled eteplirsen confirmatory study,” said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. “We also appreciate that the FDA shares our urgency in dosing a broader base of eteplirsen patients and has encouraged us to begin the clinical program with our follow-on exon-skipping drugs as soon as possible.”

Click here to read the full Sarepta press release.

CureDuchenne collaborated with Children’s National Medical Center and the Foundation to Eradicate Duchenne in 2010 to provide funding and support that enabled Sarepta Therapeutics (then AVI Biopharma) to move forward with the clinical development of eteplirsen.

CureDuchenne is proud to have been an early supporter of Sarepta by providing funding that
enabled the company to move forward with their clinical trial of eteplirsen. We look forward to continued success for eteplirsen and all of their additional exon programs.
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Click [here](http://www.sarepta.com) to read the full Sarepta press release.

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Sarepta Therapeutics to Host Webcast and Conference Call for the Duchenne Muscular Dystrophy Community on May 13

Sarepta Therapeutics, Inc., a developer of innovative RNA-based therapeutics, will host a webcast and conference call for the Duchenne muscular dystrophy (DMD) community on Tuesday, May 13, 2014, beginning at 11:00 a.m. EDT (8:00 a.m. PDT / 5).

Chris Garabedian, president and chief executive officer, and Edward Kaye, M.D., chief medical officer, will provide information and answer questions about Sarepta’s DMD development program, including upcoming clinical trials. Sarepta has invited DMD community representatives from leading patient advocacy groups to participate in the question and answer portion of the webcast.

To access the webcast, visit the events and presentations section of Sarepta’s website at www.sarepta.com/events and follow the link for the webcast under “Upcoming Events.” Please connect several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary.

To access only the audio portion, dial in to 1-888-895-5479 (U.S. toll free) for U.S. domestic callers or 1-847-619-6250 for international callers. The passcode for the call is 37195657. Please specify to the operator that you would like to join the “Sarepta DMD Development Program Update.”

Following the event, the webcast will be archived in the events and presentations section of Sarepta’s website for 90 days.

Please submit questions you’d like to ask to Alicia@cureduchenne.org.
**Prosensa Actively Working on Re-dosing Drisapersen and Regulatory Path Forward**

CureDuchenne is pleased to share the following information from Prosensa.

Dear Patient Group Representative,

As we continue to receive all of the materials, data and know-how from GSK regarding the drisapersen clinical data set, we continue to actively work on re-dosing and engage with regulators to determine a path forward for drisapersen. We remain on track to re-dosing the first cohort of boys in the 3rd quarter of this year and to communicating around a regulatory path forward for drisapersen before the end of June.

As communicated during the UPPMD webinar on March 25th, re-dosing will likely take a staged approach, under a new treatment protocol or via an expanded access program, which is often country dependent. In a first wave, we plan to initiate dosing at sites in both North America and Europe and we have already started to work with the sites and investigators to execute these plans. As these plans have not yet finalized, we cannot provide more detail at this particular time. We realize that this is a very difficult period for the boys and families and we are working as quickly as possible to provide access to drisapersen and determine a path forward. As soon as we have concrete information on re-dosing we will communicate our plans in a timely manner. For your information, the recent webinar is accessible at [http://ir.prosensa.eu/events.cfm](http://ir.prosensa.eu/events.cfm) (under additional information on the event). Prosensa will also provide an update on drisapersen during the American Academy of Neurology (AAN) 2014 Annual Meeting on April 30th and will continue to finalize the transfer of all data and materials from GSK over the next few weeks.

We have been following the recent communication in the DMD community and are encouraged that there seems to be a certain regulatory flexibility with respect to potential pathways for investigational products for the treatment of DMD. Prosensa is in contact with regulators in both Europe and the US with the aim to obtain their input into the regulatory path forward for drisapersen and we expect to communicate on this before the end of June. We feel positive about the FDA’s commitment to DMD and their willingness to work closely with all key stakeholders and consider flexible approaches to expedite the availability of treatment options for DMD. Prosensa is dedicated to do what it takes to enable long term access to drisapersen and our follow-on products as novel treatments for DMD. We are determined to accomplish this as soon as possible.

We will keep you informed on all of the above. Please do not hesitate to contact us for questions!
With kind regards,

**Giles Campion, MD** — Chief Medical Officer and SVP Research & Development

**Claire Leyten, PharmD** — Manager Patient Group Relations and primary point of contact for enquiries from patients and their families ([patientinfo@prosensa.nl](mailto:patientinfo@prosensa.nl))
Bring on the Bling on May 3 at Blingo to CureDuchenne

The inaugural Blingo to CureDuchenne will be held on May 3 at the Hyatt Regency Philadelphia. Blingo is an elegant evening of bingo and bling with celebrity guests to raise funds to find a cure for Duchenne muscular dystrophy. Guests will wear their finest bling, play bingo with celebrity callers, win amazing prizes, enjoy delicious food and cocktails. Blingo to CureDuchenne is presented by Bernie Robbins Jewelers and Thrive Gaming LLC.

The event will honor local entrepreneurs and philanthropists Wayne and Kimberly Kimmel. CureDuchenne will honor the Kimmel’s for their commitment and dedication to the Philadelphia community.

Maria Papadakis of Comcast SportsNet and Philly.com will MC the evening. Appearances will include former Philadelphia Mayor and Pennsylvania Governor Ed Rendell; model Cory Wade Hindorff from cycle 20 of America’s Next Top Model; CNN on-air health expert Dr. Jennifer Caudle;
The Preston and Steve Show’s Marisa Magnatta; and The Monday Morning Quarterback’s Andrew Brandt. Providing the night’s entertainment will be DJ Zeke Thomas and singer-songwriter Sarah Burgess, a contestant on American Idol season 6, whose 7-year-old brother lives with Duchenne.

“We are delighted to host Blingo to CureDuchenne to raise awareness and help fund critical research to find a cure for Duchenne,” said Philadelphia resident Manu Gambhir, a Duchenne parent and the chair of Blingo to CureDuchenne. “Duchenne is a progressive muscle-wasting disease that impacts my son and over 300,000 other boys and young men. Funding now will accelerate critical drug development and help save future generations of these boys and hopefully the current generation.”

The event will include a raffle, live auction and pledge to benefit CureDuchenne. All proceeds from Blingo will support the most promising research projects aimed at treating and curing Duchenne.

We are raffling off two beautiful diamonds from Bernie Robbins Jewelers. Each raffle entry receives a $100 gift certificate good towards any purchase at Bernie Robbins. Raffle tickets are $100 and only 50 tickets will be sold for each item.

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**Bernie Robbins Jewelers**

**Beloved Diamond Earrings by Hearts On Fire**

1.00ct.

$4,950 Retail

**Beloved Diamond Pendant by Hearts On Fire**

1.00ct.

$5,500 Retail

The live auction includes:

**His and Her Package Night Out Collection**

For him a TAG Heuer Carrera Timepiece with silver dial and for her a Bernie Robbins Collection Diamond Heart Pendant. The package also includes a Parx Gill dinner for four and one-night stay at Hotel Monaco Philly.

**Every Girl’s Dream Package**

Bernie Robbins Collection Inside-Out Diamond Hoop Earrings. It also includes a Nicole Miller experience and Couture spa gift card.

**Mother’s Day Shopping Spree**
Bernie Robbins $5,000 shopping spree plus a one night stay at the Four Seasons Philly and Couture spa gift card.

**Small Luxury Hotels 3 Night Getaway for 2**

Choose from over 500 locations in 70 countries for the vacation of a lifetime. The Small Luxury Hotels of the World™ (SLH) brand is an unrivalled portfolio of some of the world’s finest small independent hotels.

“We are grateful for all the Blingo to CureDuchenne sponsors,” said Debra Miller, founder and CEO of CureDuchenne. “Your support allows us to fund the most promising research projects aimed at treating and curing Duchenne.”

Thank you to all the Blingo CureDuchenne sponsors including:

**Presenting Sponsors** - Bernie Robbins Jewelers and Thrive Gaming

**Bingo Card Sponsor** - Zoomin and Skai Blue Media (in kind)

**Bingo Round Sponsors** - Thomas Jefferson University and TJUH System, Spalding Automotive, Philadelphia Style (in kind) and Community Sponsor The Children’s Hospital of Philadelphia

**Lounge Sponsor** - Moss Rehab and Parx Casino


**Benefactor Sponsor** - Ananth and Shikha Mudgil, AV International, Drexel University, Gala Cloths, Margie and Brian Wargon, Nancy Glass Productions, The Moyer Foundation and TD Wealth

**Patron Sponsor** - Land Services USA, Shreyas Gupta, Royer Cooper Braunfeld LLC

**In Kind and Prize Donors** - Dr. Gambhir/Couture, Nicole Miller, Four Seasons Hotel, Philies, Tashan Restaurant, Indeblue Restaurant, Lucky Strike Bowling, Comcast Spectator, Lacroix Restaurant, Small Luxury Hotels, Penn’s View Hotel, Hotel Monaco, Photobot 3000, Focused Studies and Luxe Hotel

Tickets for Blingo to CureDuchenne are still available for purchase.
Debra Miller to Speak at California Women's Conference

Debra Miller, founder and CEO of CureDuchenne, will be a speaker at the California Women's Conference on May 19-20 at the Long Beach Convention Center. This year’s conference includes amazing speakers like Arianna Huffington, Jane Pauley and Jack Canfield. Miller will participate in a breakout session called: Community Life A New Approach to Non-Profit Impact. Click out the list of speakers at this year’s conference.

The California Women's Conference offers its attendees inspiration, resources, and connections to take the next step in business, personal development, health and wellness, or philanthropic endeavors. Featuring widely respected thought leaders, talented entertainers, and a marketplace of ideas, exhibits, networking and panel discussions, the conference is designed to benefit women from all walks of life.

For nearly 30 years, women in California have enjoyed a conference that provided a forum for building successful businesses, strengthening careers, and positively influencing the world around them. The California Women's Conference will continue the tradition of empowering women and providing resources in every aspect of their lives, and embracing philanthropy by donating a portion of programming and proceeds to participating charitable organizations.

CureDuchenne will have a VIP table at the event. There are only 8 seats open at the table. If you are interested in attending, go ahead and purchase your spot now. It’s easy, just go to http://californiawomensconference.com/california-womens-conference-2014-arena-table/?seat_id=15752. Make sure you choose your seat number, then enter the PROMO Code “lib3rty” to receive your discounted ticket price. Miller is seated at table #164 in the Red section.
**Research**

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**Recent Success**

Champions to CureDuchenne Newport Beach Gala Raises More Than $360,000

Austin Gala Raises More Than $120,000

CureDuchenne Benefits from MJ&M

**Upcoming Events**


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**Give a Percent of Your Amazon Purchase to CureDuchenne**

Help support CureDuchenne while you shop on Amazon with Amazon Smile. Amazon Smile donates 0.5% of all qualifying purchases to the charity chosen by buyer. Millions of product qualify, and will be indicated on the item pages. There is no additional cost to you.

Here is what you need to do:

1. **Each time** you make a purchase from Amazon, you must start from smile.amazon.com.
   1. Log into your Amazon account.
   2. If you have not chosen a charity yet, you can search for CureDuchenne in the search bar at the bottom. Select CureDuchenne as your organization.
   3. CureDuchenne will show as your organization at the top of the page next to the Amazon Smile logo.

2. Make your purchase as usual. Amazon Smile gives you the same great benefits of Amazon, but you’re supporting CureDuchenne

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Champions to CureDuchenne Newport Beach Gala Raises More Than $360,000

CureDuchenne held its annual Champions to CureDuchenne Newport Beach Gala on March 29 at the Balboa Bay Club & Resort. More than 250 guests attended the gala that celebrated CureDuchenne’s 10 years of successfully advancing Duchenne research. The event raised more than $360,000 that will fund promising Duchenne research.

The Champions to CureDuchenne gala highlighted the urgent need to find a cure for Duchenne, a progressive muscle-wasting disease that impacts 1 in 3,500 boys. Boys with Duchenne are usually diagnosed by 5, in a wheelchair by 12 and most don’t survive their mid-20s. Currently, there is no approved treatment for Duchenne.

CureDuchenne honored Doug Freeman, a nationally recognized former tax attorney and business executive, who played an integral role in establishing CureDuchenne’s successful philanthropy model. Freeman provides strategic planning and organizational management advice for business, nonprofit, foundation, and family clients and has consulted nearly 300 family foundations, support organizations and public charities throughout the United States. He serves on the board of directors at First Foundation Inc. and on several nonprofit organizations. He is also the co-founder of National Philanthropy Day.

“Doug has been a CureDuchenne Champion from the beginning,” said Debra Miller. “Ten years ago, when we talked about our vision for CureDuchenne, Doug took us under his wing and helped us get started. When we wanted to invest in a small biotech company, Doug embraced our vision and even had a name for what we were doing…venture philanthropy. Ten years later, CureDuchenne has successfully completed that venture philanthropy project and is able to apply the gain to future research.”
Guests were treated to a special menu designed by Chef Jamie Gwen, a celebrity chef, certified sommelier, lifestyle expert and four-time cookbook author who has been featured on television, on the radio and in print from coast-to-coast for more than a decade. The Champions to CureDuchenne menu included spring salad, grilled peaches, burrata cheese, prosciutto and vanilla bean vinaigrette; Mediterranean bouillabaisse with sea bass, sweet shellfish, saffron broth, vegetables of the season and crostini; iron seared filet mignon with roasted shallots, smashed potato gremolata, gorgonzola cream sauce and porcini mushroom sauce; and a flight of indulgences including vanilla bean cheesecake mousse, balsamic macerated strawberries with Chantilly and chocolate peanut butter caramel corn. Gwen hosts a weekly syndicated radio show that can be heard every Sunday on 70 radio stations across the country and in Southern California on KFWB News Talk 980. Gwen recently won the Food Network’s new show “Cutthroat Kitchen.”

CureDuchenne founders, Paul and Debra Miller, and their 17-year-old son Hawken, who lives with the Duchenne, welcomed guests. Hawken gave a moving speech where he thanked all CureDuchenne supporters, spokespeople and his parents for doing everything they can to find a cure to help him and all 300,000 boys who live with Duchenne. Hawken recognized Tyler Armstrong, 10, a CureDuchenne champion who has climbed Mt. Kilimanjaro and Mt. Aconcagua to help raise awareness and money to help boys with Duchenne.

Sarah Burgess, singer-songwriter and contestant of the sixth season of American Idol, shared her story about her 7-year-old brother Jacob who lives with Duchenne. Burgess also sang “Run Away,” a song she wrote for CureDuchenne and all
boys with Duchenne. All proceeds of “Run Away” are donated to CureDuchenne.

“Celebrating CureDuchenne’s 10-year anniversary is bittersweet: 10 is such a scary number for a child with Duchenne that is when they could start losing their ability to walk,” said Burgess. “Jacob loves life and the simple pleasures it allows, and since I can’t give him my healthy muscles, I have given him my stubborn determination of doing all I can to help aid in finding a cure for Duchenne.”

Debra Miller highlighted how Duchenne research has advanced during the past 10 years. CureDuchenne has funded seven research projects that have gone into human clinical trials. When her son was diagnosed there were no clinical trials for Duchenne. Now there are two promising drugs that can slow down, stop and in some cases, reverse the progression of this heartbreaking disease that are waiting FDA approval.

“Unfortunately, this disease is too small to cure itself,” said Miller. “The funding is not available within the Duchenne community alone to raise the money needed to develop the cocktail of drugs necessary to address all of the elements of Duchenne. We are collaborating with biotech and pharmaceutical companies to find and vet joint research projects to find a cure for Duchenne.”

The evening included a silent and live auction, live musical performance and after hours party.

Champions to CureDuchenne sponsors included Kelly Thornton Smith, Lisa Argyros/Argyros Family Foundation, Athletes First, Lilly, PIMCO Foundation, First Foundation, Illumina, Integrated Oncology Network, Small Luxury Hotels, Layer Cake Wine and Specialty Restaurant Corporation.

Photos of the event can be found on our Facebook page.

- Gallery 1
- Gallery 2
- Gallery 3
- Gallery 4
- Gallery 5
**Austin Gala Raises More Than $120,000**

More than 280 guests attended the fifth annual Champions to CureDuchenne Austin Gala on April 11 at the University of Texas Golf Club. The event was hosted by Vince Young, a NFL football player and former University of Texas quarterback. The Champions to CureDuchenne Austin Gala, presented by RBC Wealth Management, raised more than $120,000 to benefit CureDuchenne. Duchenne families from all over Texas came to Austin for the special night.

It was a beautiful Austin evening and guests enjoyed delicious food prepared by Austin area restaurants including Vince Young Steakhouse, Moonshine Patio Bar & Grill, The League Kitchen & Tavern, Cookwell & Company, Moviehouse Cinema & Eatery, Zack’s American Bistro, Steiner Ranch Steakhouse, UT Golf Club, Iron Cactus and Blackfinn Ameripub. Wine was provided by from Duchman Family Winery, beer from Thirsty Planet Brewing, Tito’s Handmade Vodka, and signature Bevo-ritas with Dulce Vida Organic Tequila.

Debra Miller, founder and CEO of CureDuchenne, and Dr. Mike Kelly, chief scientific officer of CureDuchenne, spoke about the current status of Duchenne research and the urgent need to find a cure for Duchenne.

Singer-songwriter and contestant on the sixth season of American Idol Sarah Burgess performed “Run Away,” a song she wrote for her 7-year-old brother and the 300,000 boys worldwide who live with Duchenne.

Guests bid on silent and live auction items such as signed guitars, autographed football memorabilia, a home in Angel Fire, and fine dining in Austin to help raise money for CureDuchenne.
Timothy Revell, 10, delighted the crowd by auctioning a cup of flowers for $350. He was the guest auctioneer and he stole the show with his impromptu talent.

“We want to thank our generous sponsors, supporters and friends in the Austin community who have helped us raise awareness and fund research to find a cure for Duchenne,” said Tim Revell, father of two boys with Duchenne who created this signature event for CureDuchenne. “We will continue to fund research until we find a cure for all boys with Duchenne.”

Champions to CureDuchenne sponsors included RBC Wealth Management, SoCo Missions, AT&T, Covert Cadillac, Carino’s Italian, HEB, PWC Barrett Shipman, FSG, Horns Illustrated, Summit Stoneworsk, Altura Healthshare, Lee Warth Designs, Garrison Brothers, Build A Sign and Soundcheck Austin.

Thank you to Tim and Laura Revell for organizing the Austin Gala.
Mack Brown, Jack Ingram and Matthew McConaughey made the world a better place for children with their Mack, Jack & McConaughey (MJ&M) event in Austin, Texas. CureDuchenne is proud to be one of the beneficiaries of the event that was help April 24 and 25.

This amazing, star-studded event kicked off on Thursday, April 24 with a Gala, Live Auction, and a special Jack Ingram performance followed by nine-time Grammy Award-winning artist Sheryl Crow headlining concert.

On Friday, April 25, MJ&M continued with the successful Celebrity Classic Golf Tournament at Barton Creek Resort and Fashion Show with A-list designer at Neiman Marcus. This was followed by the Jack Ingram & Friends concert presented by H-E-B at ACL Live at The Moody Theater. Special guests included: Lyle Lovett, Little Big Town, Allen Shamblin, Bobby Pinson, Buddy Miller, Butch Walker, Jon Randall, Jessi Alexander, Liz Rose, Lori McKenna, Scott Miller and more.

Every year, MJ&M supports select non-profit organizations that share the principals’ goal to empower kids. The funds raised through MJ&M will support The Rise School of Austin, just keep livin Foundation, HeartGift, CureDuchenne, and Grounded in Music.

Paul and Debra Miller, co-founders of CureDuchenne, and Tim and Laura Revell, Duchenne parents in Austin, attended the event.

“We are so thankful for Mack, Jack and Matthew for selecting CureDuchenne as a beneficiary of the MJ&M event again this year,” said Debra Miller. “It is urgent...


we find a cure for this generation of Duchenne boys. The MJ&M donation will help us to continue to fund promising research to find a cure for all boys who live with Duchenne.”
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Save the date for the 4th Annual Getzlaf Golf Shootout hosted by Ryan Getzlaf, captain of the Anaheim Ducks. This two-day charity golf outing brings together athletes, celebrities and community leaders, all teaming up in support of CureDuchenne.

The event kicks off with our MVP Reception at Sutra Lounge in Costa Mesa on Saturday night, September 6. This private affair features cocktails, gourmet appetizers, silent and live auctions, and complimentary admission to the nightclub when it opens to the public later in the evening.

On Sunday morning, September 7, we'll head over to the gorgeous Monarch Beach Golf Links in Dana Point for our Golf Tournament, where we'll team one of our professional athletes with each foursome for a fun and exciting day on the green.

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