Sarepta Therapeutics Announces FDA Will Not Complete The Review Of The Eteplirsen New Drug Application By The PDUFA Date

Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, announced May 25 that the U.S. Food and Drug Administration (FDA) has notified the Company that they are continuing their review and internal discussions related to our pending NDA for eteplirsen and will not be able to complete their work by the Prescription Drug User Fee Act (PDUFA) goal date of May 26, 2016...

Read more.

Santhera Updates On Regulatory Filings For Raxone (Idebenone) In Duchenne Muscular Dystrophy (DMD)

Santhera Pharmaceuticals (SIX: SANN) announces that it has submitted comprehensive briefing material and a meeting request to the FDA to discuss the filing of a New Drug Application (NDA) for Raxone® (idebenone) for the treatment of DMD patients not taking concomitant glucocorticoids...

Read more.

WAVE Life Sciences To Advance Next-Generation Nucleic Acid Therapies To Address Unmet Need In Duchenne Muscular Dystrophy

WAVE Life Sciences Ltd. (NASDAQ:WVE), a genetic medicines company focused on developing stereopure nucleic acid therapies for patients impacted by rare diseases, reaffirmed on May 9 its commitment to advance next-generation nucleic acid therapies to address the significant unmet need of patients diagnosed with Duchenne Muscular Dystrophy (DMD)...

Read more.

DMDRP Program Announcements Released

The Duchenne Muscular Dystrophy Research Program announcements have been released by the Congressionally Directed Medical Research Programs. Debra Miller is on the review committee. We continue to be impressed with the program and are
thankful for the Duchenne funding...

Read more.

CUREDUCHENNE CARES

Two New Physical Therapists Join CureDuchenne Cares Therapy Team

Proper physical therapy designed particularly for those with Duchenne muscular dystrophy, a fatal genetic disease that causes muscle deterioration, can extend vital mobility to a young patient’s life...

Read more.

Biotech Capital Of The U.S. Welcomes CureDuchenne Cares With Open Arms

More than 45 Duchenne family members and caregivers came to the CureDuchenne Cares family workshop in Waltham, MA on May 14 to learn about Duchenne management, see live stretching demonstrations, listen to biotech representatives, and test out an assortment of mobility equipment...

Read more.

NEWS

A Charitable Goal.

Anaheim Ducks captain Ryan Getzlaf uses his celebrity status to support an expansive philanthropic portfolio. Check out and share this amazing article in Newport Beach Magazine about Ryan and Paige’s support to help cure Duchenne...

Read more.

Debra Miller Earns Rare Impact Award

Congratulations to Debra Miller for being honored with a Rare Impact Award from NORD, the National Organization for Rare Disorders. On May 17 Miller has honored at the Warner Theatre in Washington DC during NORD’s annual celebration for the year’s top achievements in rare diseases...

Read more.
Waiting For A Cure

Unlike most boys with his disease, 12-year-old Evan Procko can still walk. Usually, they’ve already lost that ability by his age, according to his father...

Read more.

The Wait For An Approved Drug For Duchenne Continues

The wait continues. Sarepta Therapeutics announced this morning that the FDA will not complete the review of the eteplirsen new drug application by the PDUFA date...

Read more.

RECENT SUCCESS

Third Annual Blingo Raises $200,000 For CureDuchenne

On Saturday, April 30\textsuperscript{th}, CureDuchenne, a national nonprofit that raises awareness and funds for research to find a cure for Duchenne Muscular Dystrophy, hosted a “blinged-out” event at The Logan Hotel—featuring special bingo callers including, FOX29 News General Assignment Reporter, Jennaphr Frederick, former Governor Edward G. Rendell, WMMR Preston and Steve show producer, Marisa Magnatta, local sensation John Bolaris, and comedian Joe Conklin in celebration for a cause to help save the lives of those with Duchenne...

Read more.

UPCOMING EVENTS

CureDuchenne Cares Workshop For Families, June 11, Long Island, NY

Please join CureDuchenne for a free, informative class on Duchenne muscular dystrophy on May 14 in Waltham, MA for family members and caregivers at 70 3rd Ave, Waltham, MA from 9 a.m. 2 p.m...

Read more.

CureDuchenne Cares Workshop For Families, July 9, Chicago, IL

Please join CureDuchenne for a free, informative class on Duchenne muscular dystrophy on July 9 in Chicago, IL for family members and caregivers at Vitals
Rehabilitation, 5820 W Irving Park Rd, Chicago from 9 a.m. 2 p.m... 

Read more.

**CureDuchenne Cares Workshop For Families, July 30, Sacramento, CA**

Please join CureDuchenne for a free, informative class on muscular dystrophy on July 30 in Sacramento, CA for family members and caregivers at Shriners Hospital for Children, 2425 Stockton Blvd, Sacramento from 8:30 a.m. 2 p.m...

Read more.

**Getzlaf Golf Shootout, August 26 And 27, Costa Mesa And Dana Point, CA**

The 6th Annual Getzlaf Golf Shootout hosted by Ryan Getzlaf, captain of the Anaheim Ducks is a two-day charity golf event that brings together athletes, celebrities and community leaders, all teaming up in support of CureDuchenne...

Read more.

**All In For Duchenne, October 15, Sylvania, OH**

The annual All in for Duchenne event benefiting CureDuchenne is on October 15 from 6 p.m. to 11 pm. Tickets include admission to the event, dinner by Jeds Barbeque and Brew, drinks and chance to win in the reverse raffle. Proceeds of the event will help fund Duchenne muscular dystrophy research...

Read more.
Sarepta Therapeutics Announces FDA Will Not Complete The Review Of The Eteplirsen New Drug Application By The PDUFA Date

Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, announced May 25 that the U.S. Food and Drug Administration (FDA) has notified the Company that they are continuing their review and internal discussions related to our pending NDA for eteplirsen and will not be able to complete their work by the Prescription Drug User Fee Act (PDUFA) goal date of May 26, 2016. The FDA has communicated that they will continue to work past the PDUFA goal date and strive to complete their work in as timely a manner as possible.

Read more.
Santhera Updates On Regulatory Filings For Raxone (Idebenone) In Duchenne Muscular Dystrophy (DMD)

Santhera Pharmaceuticals (SIX: SANN) announces that it has submitted comprehensive briefing material and a meeting request to the FDA to discuss the filing of a New Drug Application (NDA) for Raxone® (idebenone) for the treatment of DMD patients not taking concomitant glucocorticoids. A Marketing Authorization Application for DMD in Europe is also expected to be submitted in coming weeks. The Company also reports that, as part of its strategy to expand the Raxone label, a new phase III study (SIDEROS) in DMD patients using glucocorticoids will start enrolling patients in coming weeks.

The FDA-meeting request included a very comprehensive data package intended to prepare for discussions with the Agency on an accelerated NDA approval (under Subpart H) for Raxone in patients with DMD not taking concomitant glucocorticoids. The intended indication is for patients in whom respiratory function has started to decline and would include patients who previously were treated with glucocorticoids or in whom glucocorticoid treatment is not desired, not tolerated or is contraindicated.

The data package provided to the FDA summarizes data from Santhera's phase II (DELPHI) program and the successful pivotal phase III (DELOS) study, which demonstrated a clinically relevant and statistically significant benefit of idebenone treatment in slowing the rate of respiratory function decline compared to placebo. Importantly, the package also includes data from the Cooperative International Neuromuscular Research Group's (CINRG) Duchenne natural history study (DNHS), which, in collaboration with CINRG, were used to conduct the first prospectively planned external control group study to compare outcomes for patients participating in DELOS with matched, contemporaneously-observed patients from the CINRG DNHS. The results demonstrate that the respiratory function decline observed in the placebo group of the DELOS study is consistent with the rate of decline observed in matched patients from the CINRG DNHS and therefore with the expected natural history of DMD. However, the slower rate of decline observed in idebenone-treated patients in DELOS was not observed in matched patients from the CINRG DNHS, indicating that the rate of respiratory function decline in idebenone-treated patients in DELOS differs from the expected natural history of DMD.

Read more.
WAVE Life Sciences To Advance Next-Generation Nucleic Acid Therapies To Address Unmet Need In Duchenne Muscular Dystrophy

WAVE Life Sciences Ltd. (NASDAQ:WVE), a genetic medicines company focused on developing stereopure nucleic acid therapies for patients impacted by rare diseases, reaffirmed on May 9 its commitment to advance next-generation nucleic acid therapies to address the significant unmet need of patients diagnosed with Duchenne Muscular Dystrophy (DMD). WAVE’s DMD program is based on preclinical data demonstrating an approximate 25-fold improvement in exon-skipping efficiency compared to drisapersen and eteplirsen, suggesting the potential for improved potency and an enhanced ability to restore the production of functional dystrophin. In addition, WAVE’s proprietary muscle targeting technology has demonstrated substantial improvement in distribution to critical tissues in animal models, including skeletal muscle, diaphragm, and heart.

“We recognize the acute need of the Duchenne community for therapeutic options to address this devastating disease, and also appreciate regulators’ requirements for strong, well-validated, scientific evidence. Our goal is to fulfill both of these needs by bringing forward optimally designed drugs through robust clinical trials,” said Paul Bolno, M.D., MBA, President and CEO of WAVE Life Sciences. “Based on the strong preclinical data we’ve seen to date, we are highly encouraged that we are on track to develop exon-skipping medicines that maximize potency with a favorable safety profile. In addition, we plan to conduct rigorous, well-designed clinical trials that explore various predictive biomarkers and evaluate comprehensive endpoints.”

WAVE’s planned clinical trials will include both ambulatory and non-ambulatory patients in order to evaluate preservation of walking ability, heart and lung function.

Read more.
DMDRP Program Announcements Released
The Duchenne Muscular Dystrophy Research Program announcements have been released by the Congressionally Directed Medical Research Programs. Debra Miller is on the review committee. We continue to be impressed with the program and are thankful for the Duchenne funding.

Read more.
Two New Physical Therapists Join CureDuchenne Cares Therapy Team

Proper physical therapy designed particularly for those with Duchenne muscular dystrophy, a fatal genetic disease that causes muscle deterioration, can extend vital mobility to a young patient’s life. CureDuchenne Cares has developed a specialized physical therapy, education and outreach training program for caregivers and health professionals dedicated to helping those with Duchenne.

CureDuchenne Cares adds two new physical therapists, Celeste Graham and Doug Levine, to its leading edge physical therapy team.

Celeste Graham, PT, and Doug Levine, PT, join Jennifer Wallace, PT and Director of the CureDuchenne Cares program, who initially developed the workshops and training sessions. Together these physical therapists will
combine their years of experience with the common goal of creating a healthier future for Duchenne patients all over the county.

“Duchenne is such a rare disease, that it is often improperly diagnosed. This can lead to missed opportunities to begin care in the disease’s early stage,” said Debra Miller, Founder and CEO of CureDuchenne. “There is no cure for Duchenne and, as of now, physical therapy and steroids are the only known impactful treatments. That is why we truly encourage every Duchenne family to take advantage of the CureDuchenne Cares education and treatment programs.”

Duchenne is a devastating muscle disease that affects nearly 20,000 boys in the United States alone. Most children are diagnosed by the age of 5, lose their ability to walk about 12 and most do not survive their mid-20s. However, appropriate physical therapy has the potential to add years of mobility to lives of Duchenne patients.

The CureDuchenne Cares workshops feature two sessions. The first session is the continuing education course for healthcare professionals. The full-day course is designed for physical therapists, occupational therapists, adapted physical educators, speech and language pathologists, educators, physicians and nursing professionals. The session focuses on therapy treatment, orthopedic management, activity modification and equipment selection. The physical therapy workshops give healthcare professionals an in depth understanding of Duchenne including the typical progression of the disease, help them select appropriate physical therapy treatment options and develop thorough care plans for clients, and understand current research strategies and upcoming treatments. Upcoming physical therapist sessions are scheduled on June 10 in Long Island, New York; July 8 in Chicago, Illinois; and July 29 in Sacramento, California.

Professionals who complete the continuing education session are encouraged to become CureDuchenne Cares Certified Physical Therapists. Certified physical therapists received ongoing training on the most current standards of care for Duchenne and are recognized as qualified providers for physical therapy for Duchenne patients.

The second CureDuchenne Cares session is designed for families and caregivers. This free workshop focuses on proper education and basic exercises for primary and daily caregivers. Topics include stretching instruction and routines, equipment demos, activity and school modifications, and mobility management. The session also features updates on Duchenne research and clinical trials. Family and Caregiver Workshops are scheduled for this summer on June 11, in Long Island, New York; July 9, in Chicago, Illinois; and July 30 in Sacramento, California.

Both Graham and Levine have been licensed, practicing physical therapists since 1997. Graham’s experience includes in-home and clinical settings, establishing education and medical models of care. The majority of her pediatric experience was with San Diego County’s California Children Services program, where she served for some time as liaison to the Muscle Disease Clinic at Rady Children’s Hospital, where she coordinated care for patients with Duchenne. Her patient care currently focuses solely on boys and young adults with Duchenne.

Levine has worked for the Shorkey Center in Beaumont, Texas, Dell Children’s Hospital in Austin, Texas, and has provided physical therapy for over 10 school districts. Levine currently owns Growing Places Therapy Services in Austin, Texas, a pediatric therapy practice providing physical therapy, occupational therapy and speech therapy. He has been working with families affected by Duchenne from the beginning of his career and has chosen to focus much of his time and energy to help those who have been impacted by this disease. Levine has been the physical therapist for the Austin area Muscular Dystrophy clinic for the past seven years and has been an active volunteer for MDA Summer Camp for the past 10 years.

“Celeste and Doug will be valuable assets to our organization, Duchenne families and the entire Duchenne community,” said Miller. “Their experience will enable CureDuchenne Cares to expand our physical therapy training programs and workshops. Our aim is to provide the best therapy possible to thousands of children with Duchenne giving them a better chance of maintaining their mobility for as long as possible.”

For more information, go to www.CureDuchenneCares.org.
More than 45 Duchenne family members and caregivers came to the CureDuchenne Cares family workshop in Waltham, MA on May 14 to learn about Duchenne management, see live stretching demonstrations, listen to biotech representatives, and test out an assortment of mobility equipment.

As the biotech capital of the United States, Boston is home to many of the major players in the Duchenne research space. And with Waltham just 30 minutes away from Cambridge, it goes as no surprise that there were four pharmaceutical partners (Catabasis, FibroGen, PTC Therapeutics, and Sarepta Therapeutics) at the workshop, presenting information to the families on their clinical trials and research. There were also three pharmaceutical partners (Marathon, Pfizer, and WAVE Therapeutics) there as guests to answer questions and provide more information to the families one-on-one.
Also in attendance at the family workshop was CureDuchenne's newly formed CureDuchenne Cares physical therapist team. Jennifer Wallace, PT; Celeste Graham, PT; and Doug Levine, PT, presented and answered questions from family members on proper stretching techniques and mobility equipment. By training the parents and caregivers on best practices for Duchenne physical therapy, we are expanding the benefit they receive from their physical and occupational therapists.

Do you want CureDuchenne Cares to come to your city? Please contact Katie@cureduchenne.org or 949-872-2552 and let the team know. The upcoming educational sessions scheduled for 2016 include:

- June 10, Long Island, NY: Professional Continuing Education Course
- June 11, Long Island, NY: Family and Caregiver Workshop
- July 8, Chicago, IL: Professional Continuing Education Course
- July 9, Chicago, IL: Family and Caregiver Workshop
- July 29, Sacramento, CA: Professional Continuing Education Course
- July 30, Sacramento, CA: Family and Caregiver Workshop

CureDuchenne Cares would not have been possible without the support of our sponsors: BioMarin, Bristol-Myers Squibb, Catabasis, FibroGen, Idera Pharmaceuticals, Invitae, Marathon Pharmaceuticals, PhaseBio, PTC Therapeutics, Sarepta Therapeutics, and SITE SoCal.
Anaheim Ducks captain Ryan Getzlaf uses his celebrity status to support an expansive philanthropic portfolio. Check out and share this amazing article in Newport Beach Magazine about Ryan and Paige's support to help cure Duchenne. Join us for this year's Getzlaf Golf Shootout August 26 and 27.

Read more.
Congratulations to Debra Miller for being honored with a Rare Impact Award from NORD, the National Organization for Rare Disorders. On May 17 Miller has honored at the Warner Theatre in Washington DC during NORD’s annual celebration for the year’s top achievements in rare diseases. Thank you for your tireless effort to find a cure for Duchenne.

Read Miller’s story.

Read Miller’s remarks.

Watch the NORD Rare Impact Awards here.
Unlike most boys with his disease, 12-year-old Evan Procko can still walk. Usually, they’ve already lost that ability by his age, according to his father.

Evan and his parents Bill and Kimberly, from O’Brien were in Hyattsville, Md, for an FDA hearing on Monday to advocate for the approval of a drug to treat Duchenne muscular dystrophy. He’s one of 12 boys who have been taking t since 2011 as part of a clinical trials.

When the FDA advisory panel voted against recommending approval, Bill said, it was like reliving the grief he experienced when his son was first diagnosed.

Read more.
The Wait For An Approved Drug For Duchenne Continues

The wait continues. Sarepta Therapeutics announced this morning that the FDA will not complete the review of the eteplirsen new drug application by the PDUFA date. Considering the thousands of lives that could be affected by a decision on eteplirsen, we understand and appreciate the care the FDA is taking in its review of this innovative drug. We are hopeful that the delay is a positive sign. Today, the Duchenne community needs to remember that a delay is not a no.

CureDuchenne provided early funding to Sarepta for the development of eteplirsen, and we have worked together to reach this point in the years since. While we can certainly wait for the FDA’s decision, we cannot afford to lose another generation of boys to Duchenne because there weren’t treatment options. It is urgent that we get the right combination of approved treatments for our boys.

Every delay is taking precious time away from our boys. Time is not of the side of those with Duchenne. We must enjoy every moment with our children and every hug. Watch a video about the Procko family whose son Evan, 12, is on the Sarepta trial is confronting the disease and living in the moment.

We continue to remain hopeful that we will have an approved treatment for all those with Duchenne soon.

Read some of the media coverage about the delay that include quotes from Debra Miller.

STAT

Reuters

Bloomberg

The Wall Street Journal
Third Annual Blingo Raises $200,000 For CureDuchenne

CureDuchenne Honors Sarepta Therapeutics for Their Commitment to Duchenne Community

On Saturday, April 30th, CureDuchenne, a national nonprofit that raises awareness and funds for research to find a cure for Duchenne Muscular Dystrophy, hosted a “blinged-out” event at The Logan Hotel—featuring special bingo callers including, FOX29 News General Assignment Reporter, Jennaphr Frederick, former Governor Edward G. Rendell, WMMR Preston and Steve show producer, Marisa Magnatta, local sensation John Bolaris, and comedian Joe Conklin in celebration for a cause to help save the lives of those with Duchenne.

The event brought together hundreds of Philadelphia VIP’s, celebrities and influencers, raising more than $200,000. The event included a four-round game of bingo, an auction with prizes ranging from a four-night
stay anywhere in the world to a luxury African safari and a pledge where guests at the event donated as much as they wanted to help find a cure for this fatal disease. Guests enjoyed music from DJ Nigel Richards along with some crafted cocktails and delicious small plates.

This year, BLINGO to CureDuchenne honored Sarepta Therapeutics, a biopharmaceutical company focused on developing innovative RNA-targeted therapeutics, as a supporter in fighting Duchenne muscular dystrophy. CureDuchenne pushes toward innovation and advancement of Duchenne research. CureDuchenne provided early funding to Sarepta which helped the company move into human clinical trials. The U.S. Food and Drug Administration (FDA) is reviewing Sarepta’s New Drug Application (NDA) for eteplirsen, an experimental drug currently in clinical trials. The FDA PDUFA date scheduled for completion of the review is May 26, 2016.
CureDuchenne Cares Workshop For Families, June 11, Long Island, NY

Please join CureDuchenne for a free, informative class on Duchenne muscular dystrophy on June 11 in Long Island, NY for family members and caregivers at the Long Island Marriott, 101 James Doolittle Blvd., Uniondale, NY from 9 a.m. 2 p.m.  Click here to register.  For more information, contact Katie Mastro at Katie@CureDuchenne.org or 949-872-2552.
CureDuchenne Cares Workshop For Families, July 9, Chicago, IL

Please join CureDuchenne for a free, informative class on Duchenne muscular dystrophy on July 9 in Chicago, IL for family members and caregivers at Vitals Rehabilitation, 5820 W Irving Park Rd, Chicago from 9 a.m. 2 p.m. Click here to register. For more information, contact Katie Mastro at Katie@CureDuchenne.org or 949-872-2552.
CureDuchenne Cares Workshop For Families, July 30, Sacramento, CA

Please join CureDuchenne for a free, informative class on muscular dystrophy on **July 30 in Sacramento, CA** for family members and caregivers at Shriner’s Hospital for Children, 2425 Stockton Blvd, Sacramento from 8:30 a.m. to 2 p.m. Click [here](#) to register. For more information, contact Katie Mastro at [Katie@CureDuchenne.org](mailto:Katie@CureDuchenne.org) or 949-872-2552.
Getzlaf Golf Shootout, August 26 And 27, Costa Mesa And Dana Point, CA

The 6th Annual Getzlaf Golf Shootout hosted by Ryan Getzlaf, captain of the Anaheim Ducks is a two-day charity golf event that brings together athletes, celebrities and community leaders, all teaming up in support of CureDuchenne.

The Shootout kicks off with our MVP Party at Sutra Lounge in Costa Mesa on Friday night. This private affair features cocktails, dinner, silent and live auctions, and complimentary admission to the nightclub when it opens to the public. Then, on Saturday morning, we’ll head over to the gorgeous Monarch Beach Golf Links in Dana Point for our golf tournament for a fun and exciting day on the green.

For more information contact Rachel Nelson at rachel@cureduchenne.org or go to www.getzlafgolf.org
All In For Duchenne, October 15, Sylvania, OH

The annual All in for Duchenne event benefiting CureDuchenne is on October 15 from 6 p.m. to 11 pm. Tickets include admission to the event, dinner by Jeds Barbeque and Brew, drinks and chance to win in the reverse raffle. Proceeds of the event will help fund Duchenne muscular dystrophy research. For more information contact Tammy Henegar at braedansbridge@live.com, www.braedansbridge.squarespace.com or 419-260-2687