GSK Webinar Provided Research Update on Drispersen

CureDuchenne hosted a webinar with GlaxoSmithKline on May 6 that provided a research update on Phase II clinical trial results for drispersen. More than 300 people including Duchenne family members and advocates participated in the webinar.

John E. Kraus, MD, PhD, DFAPA, is the Project Physician Leader for drisapersen, an oligonucleotide being developed for the treatment of Duchenne muscular dystrophy, presented the data. He is also the Head of Medical Governance in the Neurosciences Therapy Area Unit, GlaxoSmithKline (GSK) Research & Development.

See below for a summary of the data presented during the webinar:

DRISAPERSEN UPDATE: results from a randomized, double blind, placebo-controlled Phase II clinical trial Webinar with GlaxoSmithKline.

The webinar was to inform and update the Duchenne community on the Phase II clinical trial results for GSK2402968 (drisapersen) (DMD study 114117). Drisapersen is a 2'-O-methyl-phosphothioate oligo designed to skip exon 51 in the dystrophin pre-mRNA. The results of this exploratory, unpowered, double blind, placebo-controlled Phase II clinical study was presented by Dr. John E. Kraus, MD, PhD, DFAPA, the project physician leader at GSK.

DMD 114117 clinical study:

Subjects with Duchenne muscular dystrophy met the following inclusion criteria: ≥5 years old; ambulant; corticosteroid-treated; rise from the floor ≤7 sec; and a dystrophin mutation correctable by exon 51 skipping. 53 subjects were randomized to 2 drisapersen dosing regimens or matched placebo (2:1). Dosing regimens: continuous (6mg/kg/wk) and intermittent (10-week cycles of 9 doses at 6mg/kg over 6 wks, and 4 wks off drug). Treatment was administered subcutaneously for 48 weeks. The primary objective assessed the efficacy of 2 different drisapersen dosing regimens over 24 weeks. Secondary objectives included 6MWD at 48 weeks, various timed function tests, the North Star Ambulatory Assessment [NSAA], muscle strength and safety.

Results:

Dr. Kraus reported that the continuous treatment arm (n=18) showed a clinically meaningful and statistically significant difference from placebo (n=18) on 6MWD at 24 weeks (mean, 35.09m; [95%CI, 7.59-62.60m], p=0.014), with trends supportive of efficacy in other timed function tests and the NSAA. A clinically meaningful difference from placebo (35.84m [-0.11-

71.78m], p=0.051) was maintained at 48 weeks. The intermittent treatment arm (n=17) did not separate from placebo at week 24, though by week 48 there was a clinically meaningful difference from placebo on 6MWD (27.08m [-9.83-63.99m], p=0.147), supported by trends in timed function tests and the NSAA. The decline in the 6MWDT for the placebo group paralleled that expected from the natural history of the disease. There was little change in muscle strength at either time point for either treatment arm, and it was commented that this measure was not likely to be sensitive to significant change in this patient group over 48 weeks. Drisapersen was generally well tolerated, with the majority of adverse events related to injection site reactions and proteinuria. All patients completed the study. The analysis of dystrophin in muscle biopsy samples is ongoing and results are expected during the 4th quarter 2013.

The primary objective was achieved, the continuous treatment arm showed a clinically meaningful and statistically significant difference from placebo on 6MWD at week 24. At week 48, both treatment arms showed a clinically meaningful difference from placebo on 6MWD (supported by improvement in other secondary endpoints). Drisapersen may represent an important treatment option for boys with DMD having mutations correctable by exon 51 skipping.

An open label Phase 3 study of GSK2402968 is now in progress (http://www.clinicaltrials.gov/ct2/show/NCT01480245?term=drisapersen&rank=2), the purpose of which is to explore long-term safety, tolerability and efficacy of GSK2402968 in DMD subjects who previously participated in the DMD114117 study.

For information about additional exon skipping projects in the pipeline at Prosensa, please go to: http://prosensa.eu/

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Cure Duchenne™

"Together we can CureDuchenne"

May 2013

Ryan Getzlaf to Host 3rd Annual Getzlaf Golf Shootout to Benefit CureDuchenne

Ryan Getzlaf

Ryan Getzlaf, captain of the Anaheim Ducks, is hosting the third annual Getzlaf Golf Shootout on September 7 and 8 benefitting CureDuchenne. The Getzlaf Golf Shootout is an opportunity to interact with your favorite players, play golf and support a great cause. This two-day charity golf event includes a reception at Sutra Lounge in Costa Mesa, Calif. and a golf tournament held at the Monarch Beach Golf Links in Dana Point, Calif. Each foursome will be teamed up with a professional athlete or celebrity for a unique and exciting day on the green.

Ryan Getzlaf

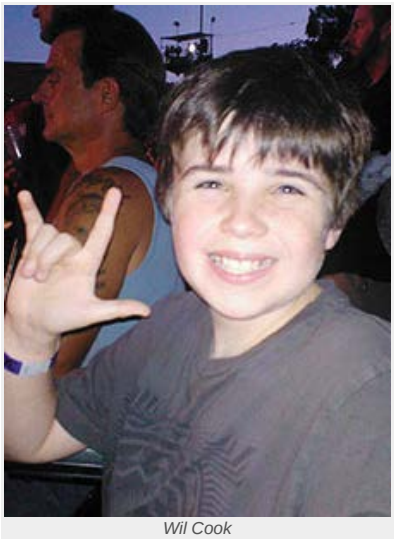
Join NHL All-Star and Olympic Gold Medalist Getzlaf and other athletes, celebrities and community leaders for an enjoyable weekend in support of CureDuchenne. The course will include a variety of fun activities including a hockey stick putting contest, longest drive contest and dunk tank as well as plenty of food and drink. Click here to watch a video of last year's event.

"We are delighted to have Ryan and Paige Getzlaf's continued support," said Debra Miller, founder of CureDuchenne. "Our CureDuchenne champions help us get closer to the cure. We invite you to join Ryan and his teammates and tee off to help us fund research to find a cure for the 300,000 boys worldwide living with Duchenne."

Foursome's cost $2,500 if paid by June 2 ($2,800 after). Click here to register. For sponsorship information, please contact Karen Harley, 949-872-2552 or karen@cureduchenne.org.

Local Bands Rockin' to CureDuchenne on June 2

Three local Orange County bands will perform a concert on June 2 to benefit CureDuchenne. The "Rockin' to CureDuchenne" concert features Breach the Summit, Johnny and the Gentlemen, and The Hager Boyz who are donating their time and talent. This family-friendly concert will be held at Stonegate Elementary School at 100 Honors in Irvine and begin at 2 p.m.



Wil Cook

The event is being organized by Irvine resident Tiffany Cook. Her son Wil, who will turn 13 the day of the concert, lives with Duchenne. Duchenne is a progressive muscle-wasting disease that impacts 1 in 3,500 boys. Boys are usually diagnosed by 5, in a wheelchair by 12 and most don't survive their mid-20s. There is currently no cure.

Cook and Harmony Hager, Wil's sixth grade teacher at Stonegate, created "Rockin' to CureDuchenne" as a fun way to generate more awareness about Duchenne and to raise money to fund research to find a cure for Duchenne.

"Most people have never heard of Duchenne until a family member is diagnosed and then it rocks your world," said Cook. "It is devastating to watch your child lose muscle strength and not be able to do activities that other boys his age are doing. Our family likes to say that 'if there is a Wil, there is a way.' We are doing everything we can to help fund research to find a cure for our son and the other 300,000 boys worldwide living with Duchenne."

Wil is featured in a CureDuchenne video that shows both the urgency and the hope of finding a cure. Wil's classmates at Stonegate and his friends are his biggest supporters.

"Wil is a joy to have in class and my students have rallied to support him and other boys with

Duchenne," said Hager. Hager's class recently had an assignment to write a persuasive essay. Collectively, they decided to write letters to the Ellen DeGeneres Show about Wil living with Duchenne to persuade the producers to have him on the show to help raise awareness about Duchenne.

"Real progress toward a cure is being made but we need the public's continued support to help us accelerate the discovery and development of new treatments for Duchenne," said Debra Miller, founder and CEO of CureDuchenne. "Fundraisers like Rockin' to CureDuchenne help get us closer to a cure."

Tickets cost $20 and all proceeds benefit CureDuchenne. To purchase tickets, click here. For more information, go to www.cureduchenne.org or call 949-872-2552.

"Rockin' to CureDuchenne" sponsors include ART Janitorial Services, McNeill Travel Plaza and KIP.

Dealing for Duchenne Raises More Than $420,000

Madison Weiss, Nicole Weiss, Nathalie Martin, Marc Weiss, Amy Martin, Chris Martin, Stanley Nelson and Carrie Miceli

Celebrities and other Duchenne champions lit up the night on May 11 for the 6th Annual Dealing for Duchenne gala. Held under the stars at the legendary Sony Pictures Studios in Culver City, Calif., Dealing for Duchenne was a star-studded event that raised money for the Center for Duchenne Muscular Dystrophy (CDMD) at UCLA. Actor Joel Murray (The Artist, God Bless America, Mad Men, Two and a Half Men) hosted Dealing for Duchenne. The funds raised will support transformative research and provide quality care for children living with Duchenne.

More than 620 guests enjoyed a classic Texas Hold 'Em poker tournament, silent and live auction, dinner by Wolfgang Puck and live music. It was a beautiful evening with a fun Los Angeles vibe.

Celebrities including Hank Azaria (The Simpsons), Tim Olyphant (Justified, Hitman), Anna Belknap (CSI-NY), Brendan Hines (Scandal), Nicole Sullivan (Cougar Town, Family Guy), Sterling Brown (Army Wives), Josh Malina (Scandal), Kellie Martin (Army Wives), Rich Eisen (NFL Network), Phill Lewis (Suite Life of Zack and Cody), Kelli Williams (Army Wives), Willie Garson (Sex and the City) and Kevin Weisman (Alias) walked the red carpet and attended the event.

Dealing for Duchenne was co-chaired by Duchenne parents Amy and Chris Martin and Valerie and Jorge Llauro. Other local Duchenne parents were acknowledged during the program and the moms received a rose for Mother's Day.

CureDuchenne Founder Debra Miller spoke about how Duchenne research has progressed over the years. Miller explained how CureDuchenne funded several research projects early on that are currently in Phase II and Phase III clinical trials.

"We are grateful for all the support of our friends who have stood by and supported us since the beginning," said Amy Martin, Duchenne parent and co-chair of Dealing for Duchenne.

Dealing for Duchenne co-chairs Jorge and Valerie Llauro

“We created this event six years go to support research and so Duchenne families could have quality care locally in Southern California.”

The Martin’s presented the 2013 Champions for Life award to their good friends Marc and Nicole Weiss. The Weiss’ have served on the event committee every year, engaged friends and family to support CureDuchenne and CDMD at UCLA, and helped raise more than a million dollars in the last five years. The Weiss’ have emotionally supported the Martin’s since their son Will was diagnosed with Duchenne and have been an advocate ever since. Marc Weiss shared a story about a recent vacation they took with the Martins and during a game of Monopoly Will told Marc, “You are my best adult friend.” It is moments like those that inspire the Weiss’ to double their efforts and encourage others to find a cure for Will and other boys with Duchenne.

Valerie Llauro explained what it was like to hear her son, Alexander, was diagnosed with a rare disease with no cure by a doctor over the phone. After the shock of a diagnosis of Duchenne, the Llauro’s looked for support and found it at the Center for Duchenne Muscular Dystrophy at UCLA. They met Stan Nelson, M.D., and M. Carrie Miceli, Ph.D., directors for CDMD at UCLA, who were able to educate them on Duchenne as researchers as well as empathize with them as Duchenne parents. Nelson and Miceli’s son, Dylan, also has Duchenne.

The proceeds from Dealing for the Duchenne the past several years has helped Nelson and Miceli create the CDMD at UCLA, which allows Duchenne families to receive comprehensive care and participate in clinical trials locally. This is the only Duchenne center of its kind in the West. A new video showcased the great work being done at CDMD at UCLA.

Thank you to the Dealing for Duchenne presenting sponsors UCLA Health Systems and David Geffen School of Medicine. Additional sponsors included: Stella Artois, Grey Goose, Bacardi, Vorzimer Masserman, Ramland Construction, O’Melveny & Myers LLP, Gibson Dunn, Freedman + Taitelman LLP, The Meyer- Whalley Family, GGW Brands, Credit.com and Illumina. We appreciate your support.

Cure Duchenne™

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May 2013

Family of the Month - The Palm Family

Brothers Jack (left) and Ryan (right) Palm.

Eight-year-old Ryan and 6-year-old Jack Palm are brothers. They are just like any other typical brothers - they play together and they fight together. They love playing with Legos and other hands-on toys. They share dreams of growing up and being a fireman (Ryan) or a trash man (Jack). The one thing they also share is that they both live with Duchenne muscular dystrophy. The brothers were diagnosed when Ryan was 3 and Jack was 1.

"Right now they are very capable," said Lisa Palm, Ryan and Jack's mom. "They are still walking and running. Ryan has been on steroids for five years which has allowed him to keep up with his many cousins, friends and classmates. Jack is still climbing and running around like any other boy his age. Ryan and Jack are aware that their muscles are not as strong as their friends and they can't run as fast. The boys do not know the long term effects of Duchenne. "

Ryan and Jack's aunt, Kristen Mull, organized the Ryan and Jack Fight Back 5k walk/run to raise money to help find a cure for Ryan and Jack, and all boys inflicted with Duchenne. The first annual race was held on May 11 in Jenkintown, PA at Alverthorpe Park. Nearly 100 people participated in the walk/run and the event raised nearly $4,000.

Jack and Lisa Palm before the Ryan and Jack Fight Back 5K race.

"The race went really well," said Mull. "The forecast was calling for rain the night before and the entire day of the race. The morning of the race, the sun came out and the rain held off for three hours just enough time for us to finish the race/walk. It ended up being a beautiful morning, and as we were driving away, it started to rain again. Next year we will make an even bigger push within the community and the school where Ryan, Jack and their two sisters, Katie (who is also severely autistic) and Alexa, attend."

"We have a lot of support from our family," said Palm. "This is very important for us. It is a hard road to travel. We want Ryan and Jack to live long healthy lives and be with us as long as possible. Having a supportive family that understands our boys makes a difference. "

Scientist of the Month - Pier Lorenzo Puri, M.D., Ph.D.

Pier Lorenzo Puri, M.D., Ph.D.

Pier Lorenzo Puri, M.D., Ph.D., is a professor of pediatrics at the University of California in San Diego and associate professor of muscle development and regeneration at the Sanford Children's Health Research Center.

As a physician, Dr. Puri was frustrated when there were no therapies for some of his patients. Dr. Puri decided to focus on research and dedicate his time to help discover therapies and a cure for a pediatric disease. He has been researching Duchenne muscular dystrophy for the past 20 years.

Dr. Puri's lab investigates the mechanisms that control the reprogramming of cells and focuses on molecular pathways influencing gene expression particularly in muscle cells. His work helps to provide a better understanding of muscle regeneration in Duchenne. Dr. Puri has a lab in both San Diego as well as in Italy. The two labs work closely together and share data and resources.

"Our lab is extremely dynamic," said Dr. Puri. "It is like having a 24-hour lab on two continents all working on one effort to reach one goal."

Dr. Puri made the initial identification and molecular characterization of the first components of the epigenetic machinery that control muscles gene expression during skeletal myogeneis. This work led to the use of epigenetic drugs, such as the HDAC inhibitors in the treatment of muscular dystrophies, first in preclinical mouse models of diseases and recently in clinical trials with patients affected by Duchenne.

Debra Miller, CureDuchenne founder, contacted Dr. Puri in 2006 after reading a published paper on a mice model that provided proof of concept.

"As a scientist I think it is important to meet the parents and children living with Duchenne," said Dr. Puri. "It provides high motivation when we have a human face to our effort. It reminds us that all our efforts will help real people and not just mice."

Dr. Puri is also a member of the CureDuchenne Scientific Advisory Board. To learn more about Dr. Puri's research, please see below.

- Class I histone deacetylases sequentially interact with MyoD and pRb during skeletal myogenesis. Puri PL, Iezzi S, Stiegler P, Chen TT, Schiltz RL, Muscat GE, Giordano A, Kedes L, Wang JY, Sartorelli V. Mol Cell. 2001 Oct;8(4):885-97.PMID:11684023 [PubMed - indexed for MEDLINE] (www.ncbi.nlm.nih.gov/pubmed/11684023)
- Stage-specific modulation of skeletal myogenesis by inhibitors of nuclear deacetylases. Iezzi S, Cossu G, Nervi C, Sartorelli V, Puri PL.

Proc Natl Acad Sci U S A. 2002 May 28;99(11):7757-62. PMID: 12032356 [PubMed - indexed for MEDLINE] Free PMC Article (www.ncbi.nlm.nih.gov/pubmed/12032356)

- Deacetylase inhibitors increase muscle cell size by promoting myoblast recruitment and fusion through induction of follistatin. Iezzi S, Di Padova M, Serra C, Caretti G, Simone C, Maklan E, Minetti G, Zhao P, Hoffman EP, Puri PL, Sartorelli V. Dev Cell. 2004 May;6(5):673-84. PMID: 15130492 [PubMed - indexed for MEDLINE] (www.ncbi.nlm.nih.gov/pubmed/15130492)

- Functional and morphological recovery of dystrophic muscles in mice treated with deacetylase inhibitors. Minetti GC, Colussi C, Adami R, Serra C, Mozzetta C, Parente V, Fortuni S, Straino S, Sampaolesi M, Di Padova M, Illi B, Gallinari P, Steinkühler C, Capogrossi MC, Sartorelli V, Bottinelli R, Gaetano C, Puri PL. Nat Med. 2006 Oct;12(10):1147-50. Epub 2006 Sep 17. PMID: 16980968 [PubMed - indexed for MEDLINE] (www.ncbi.nlm.nih.gov/pubmed/16980968)

- HDAC2 blockade by nitric oxide and histone deacetylase inhibitors reveals a common target in Duchenne muscular dystrophy treatment. Colussi C, Mozzetta C, Gurtner A, Illi B, Rosati J, Straino S, Ragone G, Pescatori M, Zaccagnini G, Antonini A, Minetti G, Martelli F, Piaggio G, Gallinari P, Steinkuhler C, Clementi E, Dell'Aversana C, Altucci L, Mai A, Capogrossi MC, Puri PL, Gaetano C. Proc Natl Acad Sci U S A. 2008 Dec 9;105(49):19183-7. doi: 10.1073/pnas.0805514105. Epub 2008 Dec 1. (www.ncbi.nlm.nih.gov/pubmed/19047631)

- Histone deacetylase inhibitors in the treatment of muscular dystrophies: epigenetic drugs for genetic diseases. Consalvi S, Saccone V, Giordani L, Minetti G, Mozzetta C, Puri PL. Mol Med. 2011 May-Jun;17(5-6):457-65. doi: 10.2119/molmed.2011.00049. Epub 2011 Feb 7. Review. PMID: 21308150 [PubMed - indexed for MEDLINE] (www.ncbi.nlm.nih.gov/pubmed/21308150)

- Fibroadipogenic progenitors mediate the ability of HDAC inhibitors to promote regeneration in dystrophic muscles of young, but not old Mdx mice. Mozzetta C, Consalvi S, Saccone V, Tierney M, Diamantini A, Mitchell KJ, Marazzi G, Borsellino G, Battistini L, Sassoon D, Sacco A, Puri PL. EMBO Mol Med. 2013 Apr;5(4):626-39. doi: 10.1002/emmm.201202096. Epub 2013 Mar 18. PMID: 23505062 [PubMed - in process] (www.ncbi.nlm.nih.gov/pubmed/23505062)

- Preclinical studies in the mdx mouse model of Duchenne Muscular Dystrophy with the Histone Deacetylase inhibitor Givinostat. Consalvi S, Mozzetta C, Bettica P, Germani M, Fiorentini F, Del Bene F, Rocchetti M, Leoni F, Mascagni P, Puri PL, Saccone V. Mol Med. 2013 Mar 27. doi: 10.2119/molmed.2013.00011. [Epub ahead of print] PMID: 23552722 [PubMed - as supplied by publisher] (www.ncbi.nlm.nih.gov/pubmed/23552722)

Cure Duchenne™

"Together we can CureDuchenne"

May 2013

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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May 2013

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.

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

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.

Open Label Study of GSK2402968 in Subjects With Duchenne Muscular Dystrophy

ClinicalTrials.gov Identifier: NCT01480245

Recruitment Status : Terminated (The study was formally terminated given that GSK is not submitting an application for regulatory approval for drisapersen in Duchenne Muscular Dystrophy.)
First Posted : November 28, 2011
Last Update Posted : March 23, 2017

Sponsor:
GlaxoSmithKline

Information provided by (Responsible Party):
GlaxoSmithKline

Study Description

Go to

Brief Summary:
The purpose of this study is to explore long-term safety, tolerability and efficacy of GSK2402968 in DMD subjects who previously participated in either DMD114117 or DMD114044.

Condition or disease	Intervention/treatment	Phase
Muscular Dystrophies	Drug: GSK2402968	Phase 3

Study Design

Go to

Study Type : Interventional (Clinical Trial)
Actual Enrollment : 233 participants
Allocation: Non-Randomized
Intervention Model: Single Group Assignment
Masking: None (Open Label)
Primary Purpose: Treatment
Official Title: An Open-label Extension Study of the Long-term Safety, Tolerability and Efficacy of GSK2402968 in Subjects With Duchenne Muscular Dystrophy
Study Start Date : September 2011
Primary Completion Date : March 2014
Study Completion Date : March 2014

Resource links provided by the National Library of Medicine

Genetics Home Reference related topics:
Duchenne and Becker muscular dystrophy

MedlinePlus related topics: Muscular Dystrophy

Genetic and Rare Diseases Information Center resources:
Muscular Dystrophy Duchenne Muscular Dystrophy
Becker Muscular Dystrophy

U.S. FDA Resources

Arms and Interventions

Go to

Arm	Intervention/treatment
Experimental: Continuous Dosing GSK2402968 6mg/kg/week	Drug: GSK2402968 6mg/kg/week
Experimental: Intermittent Dosing GSK2402968 6mg/kg/week	Drug: GSK2402968 6mg/kg/week

No Intervention: Natural History Observation The objective of this arm will be to explore DMD disease progression in a naturalistic setting once discontinuing active treatment	

Outcome Measures

Go to

Primary Outcome Measures :

1. Differences between the 6MWD at baseline and Week 104 [Time Frame: 104 weeks]

Secondary Outcome Measures :

1. Timed Function tests [Time Frame: 104 weeks]
2. Muscle strength [Time Frame: 104 weeks]
3. North Star Ambulatory Assessment Scores [Time Frame: 104 weeks]
4. Creatine kinase Serum concentrations [Time Frame: 104 weeks]
5. Pulmonary Function [Time Frame: 104 weeks]
6. Pediatric Quality of Life Neuromuscular module [Time Frame: 104weeks]
7. Clinician Global Impression of Improvement [Time Frame: 104 weeks]
8. Health Utilities Index [Time Frame: 104 weeks]
9. Frequency of accidental falls during 6 Minute Walk Distance test [Time Frame: 104 weeks]
10. Functional Outcomes Assessment [Time Frame: 104 weeks]
11. Time to major disease milestones [Time Frame: 104 weeks]

Eligibility Criteria

Go to

Information from the National Library of Medicine

Choosing to participate in a study is an important personal decision. Talk with your doctor and family members or friends about deciding to join a study. To learn more about this study, you or your doctor may contact the study research staff using the contacts provided below. For general information, Learn About Clinical Studies.

Ages Eligible for Study: 5 Years and older (Child, Adult, Senior)

Sexes Eligible for Study: Male

Accepts Healthy Volunteers: No

Criteria

Inclusion Criteria:

- Previous participation in either DMD114117 or DMD114044
- Continued use of glucocorticoids
- Willing and able to comply with all protocol requirements
- Able to give informed consent
- French subjects: Eligible for inclusion only if either affiliated to or a beneficiary of a social security category.

Exclusion Criteria:

- Subject experienced a serious adverse event or who met safety stopping criteria that remains unresolved from DMD114117 or DMD114044, which in opinion of the investigator could have been attributable to study medication and is ongoing,
- Use of anticoagulants, antithrombotics or antiplatelet agents, previous treatment with investigational drugs,except GSK2402968, within 1 month of the first administration of study medication,
- Current or anticipated participation in any investigational clinical studies,
- History of significant medical disorder which may confound the interpretation of either efficacy or safety data e.g. current history of renal or liver disease/impairment, history of inflammatory disease.

Contacts and Locations

Go to

Information from the National Library of Medicine

To learn more about this study, you or your doctor may contact the study research staff using the contact information provided by the sponsor.

Please refer to this study by its ClinicalTrials.gov identifier (NCT number): ***NCT01480245***

Show 59 Study Locations

Sponsors and Collaborators

GlaxoSmithKline

Investigators

Study Director: GSK Clinical Trials GlaxoSmithKline

More Information

Go to

Responsible Party:	GlaxoSmithKline
ClinicalTrials.gov Identifier:	NCT01480245 History of Changes
Other Study ID Numbers:	114349
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Duchenne

DMD

drisapersen

Additional relevant MeSH terms:

Muscular Dystrophies

Muscular Dystrophy, Duchenne

Muscular Disorders, Atrophic

Muscular Diseases

Musculoskeletal Diseases

Neuromuscular Diseases

Nervous System Diseases

Genetic Diseases, Inborn

Genetic Diseases, X-Linked