Cure Duchenne™

"Together we can CureDuchenne"

November 2013

Message from the Founder

Paul, Hawken and Debra Miller.

Thanksgiving is a wonderful time to share our gratitude for everyone who is helping to make a difference in the lives of boys who have Duchenne. I want to give a heartfelt thank you to:

Boys and young men with Duchenne. You are a blessing in our lives and an inspiration to others. Thank you for having a positive attitude in the face of daily challenges of living with Duchenne. You give us joy and remind us how every moment of life is precious.

Duchenne parents and families. Our lives all changed when our sons' were diagnosed with Duchenne. It is a journey we didn't expect but we have embraced. Thanks to all parents, grandparents, aunts and uncles, brothers and sisters, and caretakers for all your support.

Boys who are participating in clinical trials. We are grateful for your contribution to helping to find a cure for Duchenne. Thank you for the sacrifices you and your family have made to help boys around the world. Each step moves us closer to a cure.

Pharmaceutical/biotech companies and academic institutions conducting Duchenne research. Thank you for focusing on rare diseases. The Duchenne community is grateful for every promising treatment. Please continue to pursue every avenue until there is a cure for all our sons with Duchenne.

CureDuchenne Scientific Advisory Board. We appreciate the time and talent of our Scientific Advisory Board. Thank you for sharing your expertise and dedication to help finding a cure for Duchenne.

CureDuchenne Board of Directors. Thank you for your contributions to CureDuchenne. We are grateful for your guidance and support.

Corporate sponsors. Thank you to all our sponsors. Your support has helped us create successful events across the country. Corporate dollars allows us to leverage the amount raised at each event to enable us to fund more research to find a cure for Duchenne.

CureDuchenne Champions. We are blessed to have top-notch celebrities and athletes who have supported CureDuchenne. Thank you for lending your name and star power to help increase awareness of Duchenne. A big shout out to Clay Matthews, Scott Niedermayer, Ryan Getzlaf, Mack and Sally Brown, Vince Young, Rocket Ismail, Fergie and Josh Duhamel.

Donors. Thank you to everyone who has made a donation to CureDuchenne. Every dollar makes a difference. With your support, we will continue to fund research until there is a cure for Duchenne. Thank you for giving us an opportunity to move research from proof of concept into clinical trials as quickly as possible.

All of us at CureDuchenne wish you and your family a happy Thanksgiving. Together, we can CureDuchenne.

Dr. Adeline Vulin-Chaffiol

Upcoming Events

Tinker Bell Half Marathon Weekend, January 17-19, 2014, Anaheim, Calif.

Bowling to CureDuchenne, February 16, 2014, Addison, Texas

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With gratitude,

Debra Miller
Founder and CEO

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Prosensa Enrolls 100th Patient to its Natural History Study of Duchenne Muscular Dystrophy

Prosensa Holding N.V. (NASDAQ: RNA), the Dutch biopharmaceutical company focusing on RNA-modulating therapeutics for rare diseases with high unmet need, announced November 7 the successful enrollment of the 100th patient into the Natural History Study of Duchenne muscular dystrophy (DMD).

The goal of this observational study is to characterize DMD at various stages of progression using the same measures used in ongoing clinical studies, such as the “six minute walk test.” No medication is being tested in this study.

“The enrollment of the 100th patient for the Natural History Study reinforces our commitment to developing innovative treatment options for DMD,” said Dr. Giles Campion, Prosensa's Chief Medical Officer. “This study will foster greater understanding of the progression of this debilitating disease and will help explore new endpoints that could be used to expedite drug development.”

Dr. Brenda Wong, Director of the Comprehensive Neuromuscular Center at Cincinnati Children's and one of the study's lead investigators, adds “this critical work on the natural history of DMD represents an impactful milestone for patients and their families. Our findings may enable more efficient trial design for follow-on programs and improved outcomes for patients.”

Patients in the study are assessed every six months for a period of three years to measure their muscle strength and function, in addition to how the disease affects their quality of life as the condition evolves over time. Investigators are observing the patients as they perform various physical tests, and assess their quality of life through survey questions. Furthermore, certain biomarkers are measured through blood and urine samples to investigate a possible correlation to disease progression.

Prosensa and GlaxoSmithKline (GSK), who are both funding the study, expect to enroll up to 250 DMD patients into the study with any type of mutation in the DMD gene between the ages of three and 18. The study is being conducted in 16 hospitals across 10 countries with 14 sites already open in the U.S. and Europe. Further information on the study can be found here: www.ClinicalTrials.gov - Study ID: NCT01753804.

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Summit Receives Regulatory Approval to Start Phase 1B Clinical Trial of SMT C1100 in DMD Patients

Summit (AIM: SUMM), a drug discovery and development company advancing therapies for Duchenne Muscular Dystrophy ('DMD') and C. difficile infection, announces that its Phase 1b Clinical Trial Application for SMT C1100 has received approval from the UK Medicines and Healthcare products Regulatory Agency ('MHRA') and the Ethics Review Committee. SMT C1100 is a small molecule utrophin modulator that has the potential to treat all patients with DMD, regardless of the underlying genetic fault.

“Securing regulatory approval for the first in patient Phase 1b clinical trial has achieved another important milestone in the development of utrophin modulator, SMT C1100, for DMD," commented Glyn Edwards, Chief Executive Officer. "We believe that utrophin modulation is a novel disease-modifying approach for all boys with DMD and this Phase 1b trial forms an integral part of our wider clinical plans towards establishing SMT C1100 as a viable treatment for this devastating condition.”

The Phase 1b trial will be a dose-escalating, open-label study and will be conducted in a total of 12 paediatric patients with DMD, aged between 5 and 11 years. It will evaluate the safety and tolerability of SMT C1100, and will measure blood concentration levels of the drug as Summit aims to confirm the dose to be used in a subsequent patient proof of concept efficacy trial. The Phase 1b trial will be conducted at up to four NHS hospitals located in the UK and patient recruitment is expected to start shortly.

The Chief Investigator for the trial, Professor Francesco Muntoni, Pediatric Neurologist at Great Ormond Street Hospital and Director of the Dubowitz Neuromuscular Centre added, "Utrophin is a promising approach for the treatment of all DMD patients, regardless of their genetic mutation. It also has the potential to be complementary to other therapeutics approaches in clinical development and the start of the first patient trial of SMT C1100 is an important moment for the whole DMD community."
Further details about the clinical trial will be made available via www.clinicaltrials.gov and www.clinicaltrialsregister.eu.

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Sarepta Therapeutics Announced FDA Considers NDA Filing for Eteplirsen Premature in Light of Recent Competitive Drug Failure and Recent DMD National History Data

SAREPTA THERAPEUTICS

On November 12, Sarepta Therapeutics, Inc. (NASDAQ: SRPT) provided an update on its discussions with the U.S. Food and Drug Administration (FDA) regarding its planned New Drug Application (NDA) submission and confirmatory clinical study with eteplirsen for the treatment of Duchenne muscular dystrophy (DMD). Citing recent developments since Sarepta's last meeting with the agency, including a failed study with a competitive product and recent natural history data in DMD, the FDA indicated the new data raise “considerable doubt” about both the dystrophin biomarker and the supportive clinical efficacy assessed on the 6-minute walk test (6MWT) in the Phase IIb clinical study of eteplirsen. As a result of these recent data, the FDA stated that they “currently consider an NDA filing for eteplirsen as premature.”

“We are very disappointed with the FDA's decision to reconsider their openness to a potential NDA filing based on our current data and the resultant impact this change may have on our efforts to achieve an earlier approval of eteplirsen,” said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. “We strongly believe in the potential of eteplirsen to address a serious unmet medical need in DMD and we are committed to its development. Our team at Sarepta recognizes the urgency of families who are seeking new treatments, and we will continue to work with the FDA on an acceptable confirmatory study design and, in parallel, seek to address their concerns regarding a potential NDA filing based on our current dataset.”

Click here to read more.

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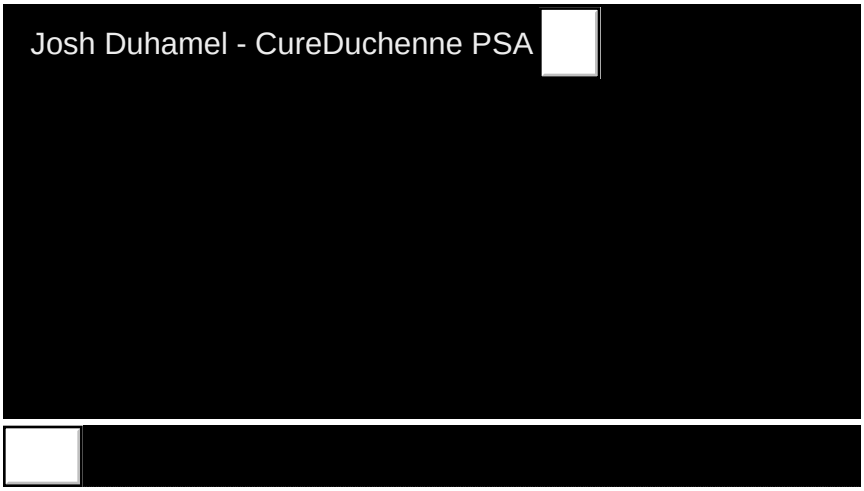
Actor Josh Duhamel Featured in New Public Service Announcement for CureDuchenne



CureDuchenne is pleased to announce that actor Josh Duhamel is featured in a new public service announcement (PSA) on behalf of the organization.

The PSA is part of CureDuchenne's continued efforts to raise awareness of Duchenne, a progressive muscle-wasting disease that impacts 1 in 3,500 boys. Boys with Duchenne are usually diagnosed by the age of 5, are in a wheelchair by 12 and most don't survive their mid-20s.

Duhamel has acted in a variety of movies and television series including Transformers, Las Vegas and All My Children. Duhamel is married to Fergie, a singer/songwriter best known as the female vocalist for The Black Eyed Peas. They recently became parents when their son Axl Jack was born. Fergie is also featured in a CureDuchenne PSA.

"We are delighted to have actor Josh Duhamel featured in a new CureDuchenne PSA," said Debra Miller, CEO and founder, CureDuchenne. "The more people know about Duchenne and its devastating effects, the more we can raise funds for promising research to find treatments for all boys who live with Duchenne."

The PSA can be viewed online on CureDuchenne's website and YouTube channel. Click here to view.

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Tyler Armstrong, 9, to Attempt Record Breaking Climb to Mt. Aconcagua to Raise Awareness and Funds to Benefit CureDuchenne

Tyler Armstrong

Nine-year-old Tyler Armstrong of Yorba Linda, Calif., will attempt a record breaking climb of Mt. Aconcagua in Argentina, the highest peak (22,837 ft.) in both the Western and Southern Hemispheres, to benefit CureDuchenne. CureDuchenne is a nonprofit that raises awareness and funds research to find a cure for Duchenne muscular dystrophy. Tyler will begin his climb on December 17 and anticipates summiting between December 26 and December 29 (weather dependent). If Tyler summits, he will be the youngest person to summit Mt. Aconcagua.

Tyler is an avid mountain climber who realized he could use his athletic abilities to help others. Mt. Aconcagua is one of the Seven Summits and it will be Tyler's second of the Seven Summits. Last year, Tyler climbed Mt. Kilimanjaro to raise awareness and money for Duchenne research and he will continue to support CureDuchenne again this year with his Mt. Aconcagua climb.

"I like hiking to the top of mountains for my friends with Duchenne," said Tyler. "Duchenne makes my friends muscles weaker and weaker. I want to use my muscles to let others know that we need to find a cure now to help all boys with Duchenne. Please donate and give Duchenne boys a chance."

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. The funds raised will support the most promising research projects aimed at treating and curing Duchenne.

"Tyler is an amazing and compassionate boy and a hero to the Duchenne community," said Debra Miller, founder and CEO of CureDuchenne. "Tyler thinks about other boys his age who are losing their ability to walk and is attempting a difficult challenge to raise awareness to help find a cure for this devastating disease. Our boys face difficult challenges everyday living with Duchenne. We

CureDuchenne Hosted a Webinar with Dr. Kevin Flanigan

Family of the Month

Ricky Tsang

Scientist of the Month

Dr. Adeline Vulin-Chaffiol

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hope Tyler will inspire people to donate so we can continue to fund research until there is a cure for Duchenne."

Visit www.cureduchenne.org/goto/Tyler to donate to Tyler's climb and raise money to fund Duchenne research. For more information on CureDuchenne, go to www.cureduchenne.org or call 949-872-2552.

More information on Tyler, visit: http://www.TopWithTyler.com or http://www.Facebook.com/TopWithTyler.

Former Dallas Cowboys Receiver Rocket Ismail to Host 2nd Annual Bowling to CureDuchenne

Rocket Ismail and guests at last year's Bowling to CureDuchenne event.

The second annual Bowling to CureDuchenne event will be held on February 16, 2014. Once again this event will be hosted by Rocket Ismail, former Dallas Cowboys receiver, and sponsored by Cadillac. This fun afternoon includes bowling with Dallas Cowboys, Rangers and other sport celebrities. It also features food, drinks, raffle prizes and silent auction. The event will be held at Bowlmor Lanes in Addison, Texas, from 2:00 p.m. to 5:30 p.m. All proceeds to benefit CureDuchenne.

Duchenne parents Jessica and Ted Rothe are organizing Bowling to CureDuchenne. Their son Nathan, 11, lives with Duchenne. Nathan is currently participating in a clinical trial for a potential treatment for Duchenne.

Bowling to CureDuchenne event organizer Jessica Rothe and her son Nathan.

"We created Bowling to CureDuchenne to help raise awareness and fund research to find a cure for Duchenne," said Jessica Rothe. "It is a great opportunity for the community to get involved and help our sons who live with Duchenne."

Last year Bowling to CureDuchenne was a huge success. Click here to watch a highlights video that captures the excitement of the event.

"We would love for you to join the fun for this great cause," said Rothe. "The money raised will help fund research that could bring promising treatments to the 300,000 boys worldwide that live with Duchenne."

Click here for more information and to register. Tickets cost $100 for adults and $50 for children. You can also order bowling shirts or t-shirts online. For sponsorship information, contact Jessica Rothe at 214-538-9799.

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Spots Still Available for Tinker Bell Half Marathon and 10K

CureDuchenne Crusaders team member Alivia Williams wins 3rd place at last year's Tinker Bell Half Marathon.

There is still time to register for the Tinker Bell Half Marathon and 10K. Join the CureDuchenne Crusaders team on Saturday, January 18, 2014 at 6:00 a.m. for the Tinker Bell 10K or on Sunday, January 19, 2014 at 5:00 a.m. for the Tinker Bell Half Marathon.

The event is sold out so the only way to participate is through a charity group. Join the CureDuchenne Crusaders team and help us raise funds to find a cure for Duchenne muscular dystrophy. Your support will help fund research to find 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.

For more information, contact Karen Harley at karen@cureduchenne.org.

Click here to register for Tinker Bell Half Marathon.

Not a runner? Click here to support the CureDuchenne Crusaders team.

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Dr. Kevin Flanigan

CureDuchenne hosted a "New Duplication Exon 2 Mouse Model: A Tool for Studies of Duplication Skipping for Duchenne" webinar with Dr. Kevin Flanigan from Nationwide Children's Hospital on November 21.

The webinar was an opportunity for the Duchenne community to learn about the duplication mutation research and an exciting exon skipping method using AAV delivery with U7. CureDuchenne has provided the lead funding for Dr. Flanigan's duplication research.

Click here to watch the recorded webinar.

CureDuchenne has been an early and important funder of exon skipping research for Duchenne. As we gain visibility to an actual therapeutic with exon skipping, we believe it's important that all boys afflicted with this disease have access to a drug that will treat their specific mutation.

The detection and possible treatment of duplication mutations in Duchenne has long been a neglected issue. CureDuchenne has worked with Dr. Kevin Flanigan over the last few years and he is making progress to discern if exon skipping can help with duplication mutations.

Duplicated exons represent approximately 6% of the DMD patient population, with the duplication of exon 2 being the single most common one identified.

The mouse model of Duchenne harbors a mutation at exon 23 of the DMD gene making it the most established preclinical model in which to examine single exon skipping strategies with antisense drugs. Unfortunately, no preclinical animal model of DMD was known to harbor a duplicated exon.

To this end, CureDuchenne partnered with Dr. Kevin Flanigan and colleagues at Nationwide Children's Hospital at Columbus, to support the development of a mouse with a duplicated exon.

In the webinar, Dr. Flanigan updated the community on the latest development of this new mouse that has a duplicated exon 2 in its DMD gene. He reported on its phenotype and its utility as a critical tool for in vivo testing of new approaches to target exon skipping strategies for duplicated exons.

In addition, we also heard that his group have developed patient-derived fibroblast cell lines representative of a variety of duplicated exons and have recently used these new cells to demonstrate exon skipping with antisense oligonucleotides.

The focus of this research is now shifting towards clinical development using an AAV delivery of a U7 snRNA-mediated exon skipping, the first target of which will be directed towards patients harboring exon 2 duplications.

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CureDuchenne intends to continue its long-term support of this important work, and is asking the Duchenne community to rally around this exciting research opportunity and help CureDuchenne provide the funding to Dr. Flanigan to ensure that his work proceeds as quickly as possible. We need to raise $120,000 immediately for Dr. Flanigan and $500,000 over the next 18 months to enable him to get ready for human clinical trials.

Please click here to donate:

If you would like for your contribution to go directly to Dr. Flanigan's work, please call 949-872-2552 or email debra@cureduchenne.org.

Click here for the recorded webinar: https://cc.readytalk.com/cc/playback/Playback.do?id=2lyea5

Family of the Month - Ricky Tsang

Ricky Tsang and his parents following his television debut on Fairchild TV in an interview with a show called 'Leisure Talk.'

Ricky Tsang, 32, is a writer from Ajax, Ontario in Canada. Tsang was diagnosed with Duchenne muscular dystrophy at the age of seven and became wheelchair bound when he was nine. As a child Tsang wanted to be a cartoonist and a chef, but when he lost his ability to draw and paint with his hands he focused on writing to express himself.

Describe your personality:

Our bodies are worthless if we don't have a little character, and people who know me personally would agree that I'm a character, which isn't necessarily a good thing… for others. I blame it on sarcasm. I've learned an emotional independence that continues to strengthen my foundations as a person because physical limitations are simply external. I acknowledge without focusing on them.

It's funny because while I was blogging, a number of people accused me of playing the sympathy card. I have a disability and include it in my writings from time to time. Muscular dystrophy is a significant part of my life and not something I can get away from. Then again, I can't control how others react, and if some of them choose to feel sorry for me, it's their problem. I call it "reverse sympathy" because there's no hope for them either.

I'm glad and I'm sad and I'm mad and I'm bad. I'm stubborn and ridiculous and my humour is insane. I'm mysterious and eccentric and annoying and outspoken. I don't compromise with anything because I'm not a one-dimensional person. I'm me.

What are your interests?

Surprisingly, considering my age and the fact that I'm on a ventilator 24/7, I can still eat, swallow, and chew regular food, with the exception of nuts. Food is a great passion of mine as I live to eat, but I still must be careful to avoid aspiration pneumonia. I love going out to different restaurants. I enjoy watching movies and listening to music, particularly Taylor Swift. However, my greatest passion is writing. It used to be art until I lost the ability to draw/paint with my hands. I switched to literature because I needed to express myself, because of a girl, no doubt! In July of 2011, I self-

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published a book called 'Ridiculous: The Mindful Nonsense of Ricky's Brain', which sold over 400 copies. I edited, designed, and marketed it myself. I'm currently working on my second book.

Amazon: http://www.amazon.com/gp/product/B005CRRSP6

Tell me about your family:

My Dad is a real estate agent who works at home whenever possible to help attend my needs. He provides for my mother and myself. Mom gave up her career as a schoolteacher back in Hong Kong to take care of me before my diagnosis. She remains my main caregiver unto this day, despite her liver transplant due to cancer in September of 2007. My older sister is a family physician.

What is it like living with Duchenne?

It means I have a physical hindrance. Of course, to say that it hasn't influenced my character would be a lie, but I'm not defined by limitations. I'm defined by my person, according to thoughts and ideas, values and beliefs, and strengths and abilities. I write because of this.

How has Duchenne impacted your life?

Since my toddler days, I've been surrounded with limitations that have continued their progression throughout the years. As a result, I'm unable to write with my hands or feed myself anymore.

The weakness has also compromised my ability to expel the healthy amount of carbon dioxide, which is why I'm on a ventilator, a.k.a. my "giant mechanical ass." I'll be on it for the rest of my life, unless they find a cure. I've already outlived the statistics that I should have been dead in my late twenties, so who knows what the future might bring?

When it comes to being treated differently, it really depends on the person. Some treat me normally, some treat me like a child, and some disregard me altogether, while others probably think I'm contagious. It bothers me to a certain extent because no one should have to earn mutual respect, but there's no loss of sleep. I kindly correct them when the opportunity arises, but don't like to waste much time on the ignorant. Generally speaking, people are very courteous and helpful.

What challenges have you faced?

Such a loaded question! Where do I begin? From losing my ability to walk, feed myself, and breathe (without a machine)…I doubt I need to elaborate further. This year alone, I've had a record close encounters with death, including three visits to emergency that tested my physical endurance to the brim. They were some of my toughest obstacles that had me thinking it was the end. My chest was congested to a point where I could hardly breathe because of bronchitis. Continuing to clear my lungs through excessive physiotherapy and torture by means of inserting tubes down the lowest part of my lungs, I let go of all my worries and simply breathed. Sooner than later, I was home again and back to my ridiculous self. Sometimes you just have to go back to the basics and focus.

What would you like to say to other boys/young men with Duchenne?

Find yourself and build on your character without relying on external sources because there's not a person in the world who can give better advice than yourself. You already depend on others for all things physical. If you don't learn to have a sense of emotional independence, what's the point? Only you can hinder your ability to choose.

Any advice for other Duchenne families?

Don't think of Duchenne muscular dystrophy as a terminal illness. My age should be a testament that there is always hope, and in fact, I know someone who is 53 with DMD. Remember that possibility outweighs the impossible, regardless. Giving up is not an option to consider, while gratitude is everything. I'm grateful to the air that I breathe. And every moment of every second of

every minute of every hour of every day of every year is the right moment for Duchenne awareness!!!

What are your hopes?

If it were possible, I'd get married and start a family. While begetting children is an invaluable gift, spending the rest of my life with the girl who sweeps me off my wheels is and will remain to be my greatest hope and dream. I'm tired of being alone. I'm not just looking for someone to love, only a woman to fall madly in love with.

As far as Duchenne is concerned, I'm not looking to be a role model, neither a hero nor an inspiration. I'm simply a human being who is fragile, like everyone else. I hope that no one believes in me when I'll always remain to be an imperfect person. I will make mistakes and I will disappoint. I'm only here to encourage a message that is very important to me:

You are your own role models. You are your own heroes and heroines. You are your own inspiration because your capacity to be strong finds justification according to your own merit.

I want our community to thrive with a mindset that captures a sense of responsibility and accountability; a take-charge mentality. My mother taught me initiative, and we need to define ourselves according to who we are and the actions we take.

Tsang is active on Facebook, so please feel free to find him: https://www.facebook.com/DearRicky. His official website is: http://www.rickytsang.ca/

Cure Duchenne™

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November 2013

Scientist of the Month - Dr. Adeline Vulin-Chaffiol

Dr. Adeline Vulin-Chaffiol

Dr. Adeline Vulin-Chaffiol is a senior post-doctoral fellow at the Center for Gene Therapy at Nationwide Children's Hospital in Columbus, Ohio. She conducts research on animals, tissue and cells that is directed toward understanding mechanisms of gene and protein expression. Most recently, she has helped design a transgenic animal that is a new model of Duchenne muscular dystrophy.

Dr. Vulin was initially interested in becoming a veterinarian while studying in her native France. She met Dr. Stephane Blot, who was evaluating the potential of cellular and gene therapies in dogs as a model for human diseases. He became Dr. Vulin's mentor, in collaboration with Dr. Luis Garcia, and her career plans changed to focus on muscular dystrophy research. This led her to work on both mice and dog models for Duchenne muscular dystrophy, and to her joining Dr. Kevin Flanigan's lab at Nationwide Children's Hospital in 2010.

Dr. Vulin recently presented the latest data from her work on a new duplication mouse model at the World Muscle Society meeting, held in California in October 2013. The presentation was called "Efficient systemic exon-slipping and dystrophin restoration in a new mouse model of DMD: a tool for therapeutic development directed at exon duplications." As part of this work, Dr. Vulin and team created a mouse that carries a duplication of exon 2 (Dup2) in the dystrophin gene. Duplications account for approximately 6% of all Duchenne mutations, and duplications of exon 2 account for 10% of those mutations, making it the most common single exon duplication in Duchenne patients.

The goal of the current study is to skip only one copy of the duplicated single exon. This is challenging, but would be expected to restore an entirely normal DMD mRNA and thus result in the expression of a wild-type protein. Dr. Vulin and her colleagues set out to skip exon 2 via a viral approach, using an adeno-associated virus (AAV) vector that delivers a small nuclear RNA (U7snRNA) to interfere with exon splicing. After confirming the dystrophic features in the new mouse model, they were able to show that treatment with the AAV-U7snRNA restores a functional dystrophin protein at the cell membrane.

In addition to providing a useful tool for the evaluation of potential exon duplication skipping strategies, the new mouse provides a tool to study a novel feature of dystrophin discovered in the Flanigan lab, called an internal ribosomal entry site (IRES). The IRES is found within exon 5 of the dystrophin gene, and when activated can lead to the creation of a partially truncated but highly functional protein, suggesting a new route of therapy for mutations in the first few exons of the gene.

CureDuchenne has worked with Kevin Flanigan's lab over the last two years to provide funding for this research.

"Without CureDuchenne's funding I wouldn't be able to work on this project and move it along so well and so fast," said Dr. Vulin. "I want Duchenne parents to have faith and hope. We are getting closer to a therapy. I believe moving forward with AAV-U7 mediated exon-skipping is

Dr. Adeline Vulin-Chaffiol

Upcoming Events

Tinker Bell Half Marathon Weekend, January 17-19, 2014, Anaheim, Calif.

Bowling to CureDuchenne, February 16, 2014, Addison, Texas

Champions to CureDuchenne Newport Beach Gala, March 29, 2014, Newport Beach, Calif.

Champions to CureDuchenne Austin Gala, April 11, 2014, Austin, Texas

Blingo to CureDuchenne, May 3, 2014, Philadelphia, Penn.

an efficient and very promising approach."

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Tinker Bell Half Marathon Weekend, January 17-19, 2014, Anaheim, Calif.

CureDuchenne is proud to be an official charity of the Tinker Bell Half Marathon. The Tinker Bell Half Marathon weekend is January 17-19, 2014. Runners are encouraged to join the CureDuchenne team and help raise funds to find a cure for Duchenne, a progressive-muscle wasting disease. Spots for the Tinker Bell Half Marathon are still available through CureDuchenne.

Disneyland® Resort becomes Never Land for a magical weekend. The events include the Tinker Bell 10 K (January 18) and the Tinker Bell Half Marathon (January 19) which is 13.1 mile run that weaves through Disneyland® Resort.

Click here, to register and run with the CureDuchenne Crusaders team. Runner packages include a one-day/one-park ticket to Disneyland.

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Bowling to CureDuchenne, February 16, 2014, Addison, Texas

Join us for the second annual Bowling to CureDuchenne event on February 16, 2014 hosted by Rocket Ismail, former Dallas Cowboy. Bowl with celebrities from the Dallas Cowboys and the Texas Rangers. The event will be held at Bowlmor Lanes in Addison, Texas, and will feature games, food, drinks, raffle prizes and silent auction.

Click here for more information and to register.

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Special

Message from the Founder

Research

Prosensa Enrolls 100th Patient to its Natural History Study of Duchenne Muscular Dystrophy

Summit Receives Regulatory Approval to Start Phase 1B Clinical Trial of SMT C1100 in DMD Patients

Sarepta Therapeutics Announced FDA Considers NDA Filing for Eteplirsen Premature in Light of Recent Competitive Drug Failure and Recent DMD National History Data

News

Actor Josh Duhamel Featured in New Public Service Announcement for CureDuchenne

Tyler Armstrong, 9, to Attempt Record Breaking Climb to Mt. Aconcagua to Raise Awareness and Funds to Benefit CureDuchenne

Former Dallas Cowboys Receiver Rocket Ismail to Host 2nd Annual Bowling to CureDuchenne

Spots Still Available for Tinker Bell Half Marathon and 10K

Recent Successes

CureDuchenne Hosted a Webinar with Dr. Kevin Flanigan

Family of the Month

Ricky Tsang

Scientist of the Month

Champions to CureDuchenne Newport Beach Gala, March 29, 2014, Newport Beach, Calif.

Save the Date. The Champions to CureDuchenne Newport Beach Gala will be held on March 29, 2014 at the Balboa Bay Club.

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Champions to CureDuchenne Austin Gala, April 11, 2014, Austin, Texas

Save the date for Champions to CureDuchenne Austin Gala on Friday, April 11 at the University of Texas Golf Club.

Click here for more information and to register.

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Blingo to CureDuchenne, May 3, 2014, Philadelphia, Penn.

Join us for a night of celebrity bingo and bling at Blingo to CureDuchenne on May 3 at the Hyatt Regency Philadelphia.

Click here for more information. If you are interested in sponsoring Blingo, please contact Drew Hoyer at drew@cureduchenne.org or 949-872-2552.

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